# 2018 ANNUAL REPORT

KINIKSA

*Every Second Counts!™*

Dear Fellow Shareholders,

2018 was a transformational year for Kiniksa, marked by corporate execution and clinical progress. We completed a successful initial public offering, advanced our pipeline, and expanded the team with high-caliber and passionate professionals. These accomplishments leave us well-positioned to invest in our clinical and preclinical product candidates and continue our drive to develop and commercialize life-changing therapies for patients suffering from rare and specialty autoimmune and autoinflammatory conditions.

## CLINICAL-STAGE PRODUCT CANDIDATES

The initial indications for our clinical-stage product candidates (rilonacept, mavrilimumab and KPL-716) are all based on strong biologic rationale and/or validated mechanisms and target underserved autoimmune and autoinflammatory disorders.

- Rilonacept is an inhibitor of interleukin-1α (IL-1α) and interleukin-1β (IL-1β). Our first target indication for rilonacept is recurrent pericarditis, a debilitating autoinflammatory cardiovascular disease of the pericardium driven by interleukin-1 (IL-1). We estimate there to be approximately 40,000 prevalent patients with recurrent pericarditis in the U.S. seeking and receiving medical treatment.
- Mavrilimumab is a fully-human monoclonal antibody inhibitor targeting granulocyte macrophage colony stimulating factor receptor alpha (GM-CSFRα). Our first target indication for mavrilimumab is giant cell arteritis (GCA), a chronic inflammatory disease of medium-large blood vessels. We estimate there to be approximately 75,000-150,000 prevalent patients with GCA in the U.S.
- KPL-716 is a fully-human monoclonal antibody inhibitor of signaling through oncostatin M receptor beta (OSMRβ). We are initially evaluating KPL-716 in a variety of pruritic diseases, including prurigo nodularis, a chronic dermatologic condition characterized by severely pruritic skin nodules. We estimate there to be approximately 300,000 prevalent patients with prurigo nodularis in the U.S.

## 2018 CLINICAL-STAGE DEVELOPMENT EXECUTION

In 2018, key accomplishments from our clinical-stage product candidates included:

- Announcing interim data from our open-label Phase 2 clinical trial of rilonacept in subjects with symptomatic recurrent pericarditis. These data showed reductions in both inflammation and reported pain after the first dose which persisted throughout the treatment period.
- Receiving an active investigational new drug application (IND) with the U.S. Food and Drug Administration (FDA) for the clinical study of mavrilimumab in subjects with GCA.
- Initiating multiple clinical trials, including our pivotal Phase 3 clinical trial of rilonacept in subjects with recurrent pericarditis (RHAPSODY), our global Phase 2 proof-of-concept clinical trial of mavrilimumab in subjects with GCA and our repeated-single-dose Phase 1b clinical trial of KPL-716 in subjects with moderate-to-severe atopic dermatitis.
- Presenting single-dose Phase 1a/1b clinical data for KPL-716. Data from this first-in-human clinical trial provided evidence of safety, tolerability, target engagement and an early signal of reducing pruritus as well as inflammation and disease severity in subjects with moderate-to-severe atopic dermatitis.

## 2019 CLINICAL-STAGE DEVELOPMENT PLAN AND MILESTONES

Looking ahead, we remain focused on building momentum across our clinical-stage pipeline through targeted investment and continued execution.

- For rilonacept, we are enrolling our pivotal Phase 3 clinical trial in subjects with recurrent pericarditis (RHAPSODY). We are also initiating our pre-commercial activities: raising disease awareness, developing a go-to-market strategy and engaging with physicians, nurses and patients to help formalize patient advocacy in support of the recurrent pericarditis community.
- For mavrilimumab, we are enrolling our global Phase 2 clinical trial in GCA.
- For KPL-716, we plan to initiate two Phase 2 clinical trials in the first half of 2019. The first is an adaptive design Phase 2a/2b clinical trial in subjects with prurigo nodularis. The second is an exploratory, multi-indication Phase 2 pilot study in a number of diseases characterized by chronic pruritus.
- We expect to report top-line data from the KPL-716 repeated-single-dose Phase 1b clinical trial in subjects with moderate-to-severe atopic dermatitis in the second half of 2019. These data will provide important insights into the potential of KPL-716's mechanism of action (which inhibits two cytokine pathways) as well as further conviction in our KPL-716 development strategy and ability to provide differentiated efficacy in the treatment of a variety of pruritic, inflammatory and fibrotic conditions.

By the end of 2020, we expect these clinical-stage product candidates to produce multiple clinical data readouts, including pivotal top-line Phase 3 results for rilonacept, global top-line Phase 2 results for mavrilimumab and top-line Phase 2a results for KPL-716.

## PRECLINICAL DEVELOPMENT

We are also advancing our preclinical and development activities, including the exercise of our option to acquire KPL-404, a fully-human monoclonal antibody inhibitor of the CD40 co-stimulatory receptor. We expect to file an investigational new drug application for KPL-404 in the second half of 2019.

As we drive toward becoming a fully-integrated global biopharmaceutical company, we reflect on the completion of a transformational year and thank you for your ongoing support. In 2019, we are excited to build upon a remarkable year in 2018 by executing across our pipeline and helping patients suffering from devastating autoimmune and autoinflammatory diseases.

***Every Second Counts!™***

**Sanj K. Patel**
*CEO and Chairman of the Board*

# 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, 2018**

**OR**

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

**For the transition period from to**

**Commission file number: 001-38492**

# Kiniksa Pharmaceuticals, Ltd.

(Exact name of registrant as specified in its charter)

| **Bermuda** | **98-1327726** |
|---|---|
| (State or other jurisdiction of incorporation or organization) | (I.R.S. Employer Identification Number) |

**Kiniksa Pharmaceuticals, Ltd.**
**Clarendon House**
**2 Church Street**
**Hamilton HM11, Bermuda**
**+ (44) 808-189-6257**
(Address, zip code and telephone number, including area code of principal executive offices)

**Kiniksa Pharmaceuticals Corp.**
**100 Hayden Avenue**
**Lexington, MA, 02421**
**(781) 431-9100**
(Address, zip code and telephone number, including area code of agent for service)

**N/A**
(Former name, former address and former fiscal year, if changed since last report)

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

| Title of each class | Name of each exchange on which registered |
|---|---|
| Class A Common Shares $0.000273235 par value | The Nasdaq Global Select Market |

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

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

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

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

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

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

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

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the Registrant as of June 30, 2018, was approximately $327.3 million.

As of March 1, 2019, there were 54,332,160 common shares outstanding in aggregate, comprised of:

18,639,733 Class A common shares, par value $0.000273235 per share

4,638,855 Class B common shares, par value $0.000273235 per share

14,995,954 Class A1 common shares, par value $0.000273235 per share

16,057,618 Class B1 common shares, par value $0.000273235 per share

**DOCUMENTS INCORPORATED BY REFERENCE**

Portions of the registrant's definitive proxy statement for its 2019 Annual Meeting of Shareholders, which the registrant intends to file with the Securities and Exchange Commission pursuant to Regulation 14A within 120 days after the end of the registrant's fiscal year ended December 31, 2018, are incorporated by reference into Part III of this Annual Report on Form 10-K.

**Kiniksa Pharmaceuticals, Ltd.**

**FORM 10-K**

**FOR THE YEAR ENDED DECEMBER 31, 2018**

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, or Annual Report, contains forward-looking statements. All statements other than statements of historical facts contained in this Annual Report including statements regarding our future results of operations and financial position, business strategy, prospective products and product candidates, their expected properties, performance and impact on healthcare costs, the expected timeline for achievement of our clinical milestones, the timing of, and potential results from, clinical and other trials, marketing authorization from the FDA or regulatory authorities in other jurisdictions, coverage and reimbursement for procedures using our product candidates, if approved, research and development costs, timing of regulatory filings and feedback, timing and likelihood of success, plans and objectives of management for future operations and future results of anticipated products, are forward-looking statements.

These 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.

In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “goal,” “design,” “target,” “project,” “contemplate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions, although not all forward-looking statements contain these identifying words. The forward-looking statements in this Annual Report are only predictions. 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. These forward-looking statements speak only as of the date of this Annual Report and are subject to a number of risks, uncertainties and assumptions described under the sections in this Annual Report entitled “Risk factors” and “Management’s discussion and analysis of financial condition and results of operations” and elsewhere in this Annual Report. These forward-looking statements are subject to numerous risks, including, without limitation, the following:

- our status as a development-stage company and our expectation to incur losses in the future;
- our future capital needs and our need to raise additional funds;
- our limited operating history;
- the lengthy and expensive clinical development process with its uncertain outcome and potential for clinical failure or delay;
- the decision by any applicable regulatory authority whether to clear our product candidates for clinical development and, ultimately, whether to approve them for marketing and sale;
- our ability to anticipate and prevent adverse events caused by our product candidates;
- our ability to identify, in-license, acquire, discover or develop additional product candidates;
- our ability to have our product candidates manufactured;
- the market acceptance of our product candidates;
- our ability to timely and successfully develop and commercialize our existing and future product candidates, if approved;
- physician awareness and adoption of our product candidates;

- the size of the market for our product candidates;
- our ability to meet the quality expectations of physicians or patients;
- our ability to improve our product candidates;
- the decision of third-party payors not to cover our product candidates or to require extensive or independently performed clinical trials prior to covering or maintaining coverage of our product candidates;
- our ability to successfully manage our growth;
- our ability to avoid product liability claims and maintain adequate product liability insurance;
- our ability to obtain regulatory exclusivity;
- our ability to obtain, maintain, protect and enforce our intellectual property rights related to our product candidates;
- federal, state and foreign regulatory requirements applicable to our product candidates; and
- ownership concentration of our executive officers and certain members of senior management may prevent our shareholders from influencing significant corporate decisions.

Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, 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. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this Annual Report will prove to be accurate. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

You should read this Annual Report and the documents that we reference in this Annual Report completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

## INDUSTRY AND OTHER DATA

Unless otherwise indicated, certain industry data and market data included in this Annual Report were obtained from independent third-party surveys, market research, publicly available information, reports of governmental agencies and industry publications and surveys. All of the market data used in this Annual Report involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. We believe that the information from these industry publications and surveys included in this Annual Meeting is reliable. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the sections of this Annual Report entitled “Risk factors” and “Special note regarding forward-looking statements” and elsewhere in this Annual Report. These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

## PART I

### ITEM 1. BUSINESS.

#### Overview

We are a biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients suffering from debilitating diseases with significant unmet medical need. We have a pipeline of product candidates, across various stages of development, focused on autoinflammatory and autoimmune conditions. We have three clinical-stage product candidates and two pre-clinical-stage product candidates. We follow a disciplined and methodical approach to selectively identify, discover and acquire product candidates with strong biologic rationales or validated mechanisms of action. We believe that our product candidates have the potential to address multiple indications.

Our portfolio of product candidates offers multiple development opportunities. By modulating different parts of the innate and adaptive immune system, these product candidates together have the potential to provide a variety of mechanisms to address multiple devastating diseases.

***Rilonacept*** is a protein cytokine trap for inhibiting interleukin-1α, or IL-1α, and interleukin-1β, or IL-1β. Cytokines are small proteins that play a key role in cell signaling. Rilonacept is approved by the U.S. Food and Drug Administration, or the FDA, for the treatment of Cryopyrin-Associated Periodic Syndromes, or CAPS, and has been commercially sold as ARCALYST by Regeneron Pharmaceuticals, Inc., or Regeneron, for this indication since 2008. We licensed rilonacept from Regeneron in 2017. We are initially developing rilonacept for the treatment of recurrent pericarditis, a painful autoinflammatory cardiovascular disease with an estimated U.S. prevalent population of approximately 40,000 patients seeking and receiving medical treatment. We are not aware of any therapy currently approved by the FDA for the treatment of recurrent pericarditis. We are enrolling a single, pivotal, global, Phase 3 clinical trial in recurrent pericarditis, named RHAPSODY. We expect to complete enrollment in this trial in the second half of 2019 and report top-line data in the second half of 2020. RHAPSODY is a double-blind, placebo-controlled, randomized-withdrawal, or RW, design study with open-label extension which is designed to assess the efficacy and safety of rilonacept treatment in subjects with recurrent pericarditis. We also have an ongoing open-label Phase 2 proof-of-concept clinical trial in recurrent pericarditis, for which we have completed enrollment. In December 2018, we reported interim data from the Phase 2 trial. As of the November 1, 2018 data cutoff date, interim data from 12 symptomatic subjects participating in one portion of the Phase 2 trial showed a reduction in both c-reactive protein, or CRP, an inflammation biomarker, and reported pain. As of the cutoff date, 10 of the subjects had completed the 6-week base treatment period and entered into the optional 18-week extension period. Four of the 10 subjects had completed the optional 18-week extension period. All subjects showed a persistent clinical response as measured by c-reactive protein and pain levels at each measurement point during the study. Rilonacept has been generally well-tolerated in the trial, with adverse events, or AEs, consistent with the FDA-approved label for the treatment of CAPS. The most common AEs were gastrointestinal disorders and injection site reactions. There was one treatment-related serious AE which resulted in discontinuation: a skin abscess which responded to medical treatment. Infections are reported in the rilonacept label. We expect to present additional data from the Phase 2 trial at the American College of Cardiology 68th Annual Scientific Session & EXPO 2019, or ACC, in March 2019.

***Mavrilimumab*** is a monoclonal antibody that antagonizes the signaling of granulocyte macrophage colony stimulating factor, or GM-CSF. We are focusing our initial development efforts for mavrilimumab on giant cell arteritis, or GCA, a chronic inflammatory disease of medium-large blood vessels with an estimated U.S. prevalence of approximately 75,000 – 150,000 patients. MedImmune Limited, or MedImmune, initially developed mavrilimumab for the treatment of rheumatoid arthritis, or RA. MedImmune's Investigational New Drug application, or IND, for the clinical development of mavrilimumab for the treatment of RA was initially put on clinical hold in 2010 before human data had been generated due to certain effects that were observed in non-clinical studies, which coincides with a theoretical risk of developing pulmonary alveolar proteinosis, or PAP, possibly in the setting of GM-CSF inhibition. Since then, in 2014, the FDA acknowledged that clinical studies in refractory RA may be appropriate based on MedImmune's clinical studies in Europe in which it dosed over 550 RA patients with mavrilimumab with no evidence of PAP. Medimmune has since withdrawn the IND for Mavrilimumab for the treatment of RA, and we submitted a new

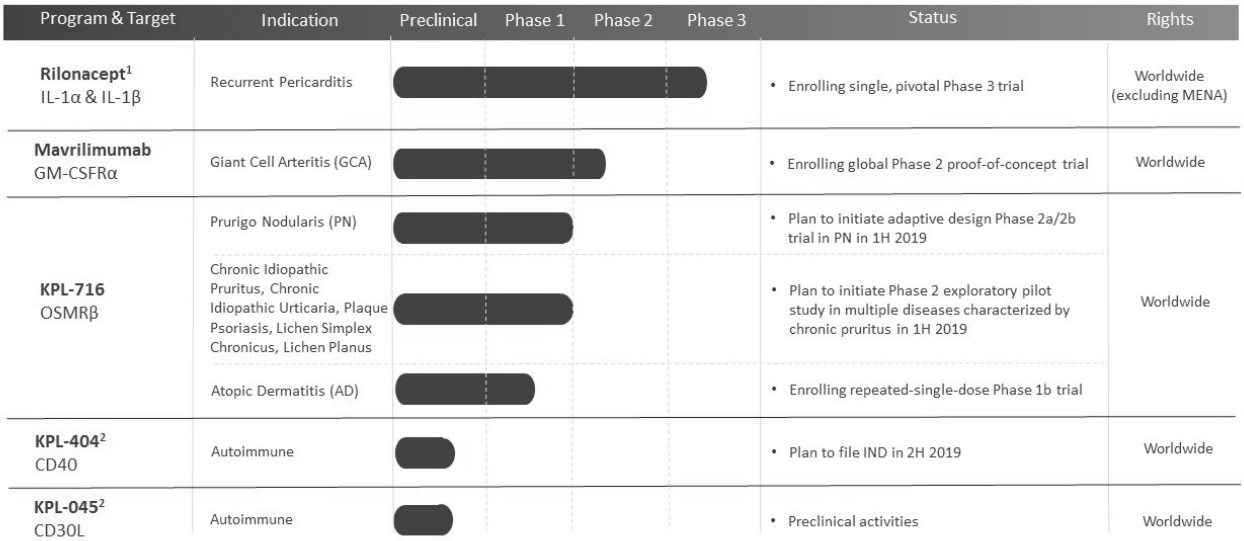
IND with the FDA for the study of mavrilimumab in GCA. The FDA initially placed our IND on clinical hold due to its request for additional information regarding the 510(k)-cleared delivery device to be used in our Phase 2 clinical trial. We have since provided the FDA with the requested information and our IND is now active. We plan for U.S. subjects to be included in our ongoing, randomized, double-blind, placebo-controlled, global Phase 2 proof-of-concept clinical trial, for which we have commenced dosing in multiple countries. We expect to complete enrollment in this trial in the second half of 2019 and report top-line data in the second half of 2020.

***KPL-716*** is a monoclonal antibody that simultaneously inhibits the signaling of the cytokines interleukin-31, or IL-31, and oncostatin M, or OSM, by targeting their common receptor subunit, oncostatin M receptor beta, or OSMRβ. We believe KPL-716 is the only monoclonal antibody in development that simultaneously targets both pathways. We plan to study KPL-716 in a variety of pruritic, inflammatory, and fibrotic indications driven by these cytokines, including prurigo nodularis, a chronic inflammatory skin condition with an estimated U.S. prevalence of approximately 300,000 patients. At the European Association of Dermatology and Venereology congress in September 2018, we presented results from the randomized, double-blind, placebo-controlled, single-ascending-dose, sequential-group portion of the Phase 1a/1b clinical trial in healthy volunteers and in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus. In this trial, single intravenous, or IV, and subcutaneous, or SC, doses were well tolerated. The results provided an early signal in efficacy in reducing pruritus (assessed by the Worst-Itch Numerical Rating Scale), as well as reducing inflammation and disease severity (assessed by Eczema Area Severity Index, or EASI) in atopic dermatitis subjects after a single dose of KPL-716 in a placebo-controlled, exploratory efficacy assessment (20 subjects were randomized 1:1 and received either 7.5 mg/kg IV of KPL-716 or placebo). We believe these single-dose results provide proof-of-principle for KPL-716's potential to treat a spectrum of IL-31-driven pruritic diseases, and support our plans to advance KPL-716 into multiple chronic pruritic diseases. We plan to initiate an adaptive design Phase 2a/2b clinical trial in prurigo nodularis in the first half of 2019 and expect to report top-line data from the first part of this trial in the first half of 2020. We also plan to initiate an exploratory, pilot Phase 2 clinical trial in the first half of 2019 designed to explore the role of IL-31 and OSM in a number of diseases characterized by chronic pruritus and to report top-line data from this trial in the second half of 2020. To help us to understand whether KPL-716 could be a competitive therapeutic in atopic dermatitis, if approved, and to provide long-term data on IL-31 driven pruritus as a proxy for other disease indications, we are enrolling a 12-week, repeated single-dose cohort as an additional part of the Phase 1b portion of the Phase 1a/1b clinical trial in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus. This cohort is designed to evaluate safety, tolerability, pharmacokinetics and immunogenicity, and it will allow us to conduct an exploratory efficacy analysis on both pruritus as well as disease severity response markers (assessed by EASI). We expect to report top-line data from this cohort in the second half of 2019.

***KPL-404*** is a monoclonal antibody inhibitor of the CD40/CD40L interaction, a central control node of T-cell-dependent, B-cell-mediated humoral adaptive immunity. We are continuing our pre-clinical activities in KPL-404 in T-cell dependent, B-cell mediated diseases, and expect to file an IND with the FDA for this program in the second half of 2019 and initiate a Phase 1 clinical trial in the first half of 2020. In January 2019, we exercised our exclusive option to acquire all of the outstanding capital stock of Primatope Therapeutics, Inc., or Primatope, the company that owned or controlled the intellectual property related to KPL-404, and closed this transaction in March 2019.

***KPL-045*** is a monoclonal antibody inhibitor of the CD30/CD30L interaction, a T-cell co-stimulatory receptor involved in activated T-memory cell function. We are currently evaluating the progression of KPL-045 pending preclinical data from the program in the context of our portfolio.

The following table summarizes our current pipeline of product candidates:




(1) Rilonacept (ARCALYST®) is approved and marketed for cryopyrin-associated periodic syndrome, in the United States by Regeneron. We will assume the rights to this indication upon receiving approval for rilonacept in the recurrent pericarditis indication.

(2) We are conducting pre-clinical activities for KPL-404 and KPL-045 and plan to review the potential for KPL-404 and KPL-045 in T-cell-dependent, B-cell-mediated diseases, such as pemphigus/pemphigoid, myasthenia gravis, or graft versus host disease.

In addition to the indications described above, we plan to review the potential for rilonacept, mavrilimumab and KPL-716 in other indications. We plan to be opportunistic in our business development activities to identify and potentially acquire the rights to additional programs or companies that could expand our portfolio or potentially add a platform capability. We have also initiated our own internal research efforts to discover and develop molecules to address areas of unmet medical need.

We currently plan to commercialize our product candidates, if approved, in the United States and select international markets. In parallel with our product development timelines, we plan to build our own commercial and operational organizations around the world as appropriate. We anticipate building targeted medical affairs and sales teams focused on specialist physicians who treat the patient populations addressed by our product candidates.

**Our Team**

We have assembled an experienced management team with a successful track record. Our team has expertise across the spectrum of global drug discovery, development, manufacturing and commercialization activities in diseases within both large and orphan indications. Our Chairman and Chief Executive Officer, Sanj K. Patel, has more than 25 years of scientific, clinical and commercial experience in the pharmaceutical and biotechnology industries. Our Chief Medical Officer, John F. Paolini, M.D., Ph.D., has more than 17 years of experience planning, operating and executing clinical development programs across a range of disease indications from orphan diseases to large cardiovascular diseases, and ten years as a practicing cardiologist. Other members of our senior management team have held key management positions at other companies that developed and commercialized therapies for underserved, rare and

specialty-focused patient populations. These companies include Synageva, Genzyme, Novo Nordisk, Shire, Sanofi, Pfizer, Bayer, Merck, Novartis and Vertex, among others.

**Our Strategy**

Our vision is to build a fully-integrated, global biopharmaceutical company by discovering, acquiring, developing and commercializing life-changing therapies for debilitating diseases. We are developing a pipeline of novel drug product candidates for the treatment of autoinflammatory and autoimmune diseases, and we aim to be an industry leader in these areas. We are pursuing multiple programs in parallel, with the goal of delivering safe and effective therapies to patients as efficiently as possible.

Critical components of our business strategy include the following:

- **Efficiently and Rapidly Advance Our Product Candidates Through the Development Process.** We believe that our product candidates have the potential to address significant unmet medical needs and intend to develop them as efficiently and rapidly as possible.

- **Commercialize Our Product Candidates to Bring New or Improved Therapies to Patients in Need.** We intend to market and commercialize our product candidates, if approved, in the United States and select international markets by developing our own sales, marketing, medical affairs and reimbursement organizations as appropriate. We anticipate creating a targeted sales organization that supports specialist physicians who treat these specific patient populations and plan to build out this organization as our product candidates approach potential regulatory approval. We believe this approach will allow us to effectively reach patients and prescribers that our product candidates target and leverage the commercial potential of our product candidates.

- **Maximize Our Existing Portfolio Opportunity by Expanding Use Across Multiple Indications.** A core component of our approach to product development is identifying assets that have the potential to treat multiple diseases. We aim to develop and commercialize our product candidates to produce meaningful impact for patients across relevant indications. Our assets are designed to modulate signaling pathways that are implicated across a spectrum of autoimmune and autoinflammatory conditions. We believe that our product candidates have potential in multiple indications.

- **Leverage Our Value-Driven Approach to Identify, Discover, Acquire and Develop New Therapies.** We follow a disciplined and methodical approach to our review of new opportunities. We focus on research-based and comprehensive indication mapping exercises to categorize and prioritize indications of interest. We evaluate a variety of factors for potential product candidates, technologies and discovery targets, including biologic rationale for addressing the disease, potential for regulatory approval, commercial viability, intellectual property position, prospects for favorable pricing and reimbursement and the impact of competition. We also look at assets that could potentially address multiple indications. In building our current pipeline, we evaluated a large number of opportunities and negotiated agreements with parties for the assets that met our criteria and have in-licensed or acquired the rights to develop and commercialize five separate biologics. Going forward, we intend to be opportunistic in our business development activities.

- **Build Our Core Capability in Autoimmune and Autoinflammatory Diseases to Establish a Leadership Position in the Field.** Our pipeline consists of protein therapeutic candidates across various stages of drug development, including a cytokine trap—rilonacept—and four monoclonal antibodies—mavrilimumab, KPL-716, KPL-404 and KPL-045. Both categories of therapeutics functionally inhibit signaling pathways that are implicated in autoinflammatory- or autoimmune-driven pathologies. We intend to leverage our internal discovery efforts and business development capabilities to complement our existing portfolio to build our core capability and establish a leadership position in the field.

## Our Product Candidates

### Rilonacept

#### *Overview*

Rilonacept was approved by the FDA for the treatment of cryopyrin-associated periodic syndromes, or CAPS, which includes cold auto-inflammatory syndrome and Muckle-Wells syndrome, and has been commercially sold as ARCALYST in the United States by Regeneron for this indication since 2008. We licensed rilonacept in 2017 from Regeneron. We believe that rilonacept has potential to treat certain diseases mediated by both IL-1α and IL-1β. Our lead indication for rilonacept is recurrent pericarditis, a painful autoinflammatory cardiovascular disease with an estimated U.S. prevalent population of approximately 40,000 patients seeking and receiving medical treatment. We are enrolling a single, pivotal, global, Phase 3 clinical trial in recurrent pericarditis, named RHAPSODY. We expect to complete enrollment in this trial in the second half of 2019 and report top-line data in the second half of 2020. We also have an ongoing open-label Phase 2 proof-of-concept clinical trial in order to gain further experience with rilonacept in different recurrent pericarditis populations. We completed enrollment for this trial and reported interim results of this trial in December 2018. We expect to present additional data from this trial at ACC in March 2019. We plan to review the potential for rilonacept in additional diseases mediated by IL-1α and IL-1β.

There is currently one other FDA-approved agent that blocks both IL-1α and IL-1β signaling, anakinra, and one that blocks only IL-1β, canakinumab. We believe both therapies have limitations, and neither is approved by the FDA for treatment of pericarditis. Anakinra requires once-daily injections, and canakinumab only blocks IL-1β, making it less effective or ineffective in diseases driven by IL-1α pathology. We believe that rilonacept with its more moderate, once-weekly dosing schedule and its ability to inhibit both IL-1α and IL-1β could provide an improved therapeutic option for a variety of IL-1α-mediated diseases.

#### *Mechanism of Action*

Rilonacept is an inhibitor of IL-1α and IL-1β. IL-1α and IL-1β have been demonstrated to play a key role in inflammatory diseases. IL-1α and IL-1β provoke potent, pro-inflammatory events by engaging the IL-1α and IL-1β receptor. Following tissue insult, the release of IL-1α acts as the primary initiating signal to coordinate the mobilization of immune cells to the damaged area, while IL-1β is secreted mostly by macrophages and is a prototypical cytokine of the canonical inflammasome. IL-1α and IL-1β signaling results in a dramatic increase in the production of cytokines that orchestrate the proliferation and recruitment of phagocytes to the site of damage, resulting in inflammation. Moreover, IL-1α and IL-1β signaling also affect other immune-system cells, such as T-cells and B-cells.

IL-1β's role in the inflammation process has been extensively studied, while in comparison, much is still unknown about the independent function of IL-1α in disease pathology. Despite driving similar immunological outcomes, IL-1α and IL-1β differ substantially in their expression and regulation, and non-redundant roles for IL-1α or IL-1β have been demonstrated in multiple inflammatory diseases. There are disease states in which IL-1β inhibition alone does not appear to be sufficient for disease remission in the absence of IL-1α inhibition. Published studies suggest certain autoinflammatory diseases may, in fact, be pathologically driven primarily by IL-1α.

An investigator-initiated study of anakinra successfully demonstrated mechanistic proof-of-concept for inhibiting both IL-1α and IL-1β in the treatment of recurrent pericarditis. In a published case study, a patient with a refractory form of recurrent pericarditis, who was well-controlled on anakinra, was switched from anakinra to canakinumab, which inhibits only IL-1β, for tolerability reasons. The patient's disease returned despite further dose escalation of canakinumab. When the patient was switched back to anakinra, which inhibits IL-α and IL-β, the disease promptly went back into remission. These data, together with clinical data from our ongoing open-label Phase 2 proof-of-concept study and confirmatory market research, may indicate that IL-1α and IL-1β play unique roles in recurrent pericarditis and other autoinflammatory diseases in which the pathology may be driven primarily by IL-1α.

***Background and Market Opportunity for Recurrent Pericarditis***

Pericarditis is the most common disorder involving the pericardium, the two-layered sac that surrounds the heart. Pericarditis is an inflammation of this sac and is typically characterized by significant chest pain, shortness of breath, coughing and fatigue and is often misconstrued by patients as a heart attack. In addition, typical signs of pericarditis include pericardial friction rub, electrocardiogram changes or pericardial effusion, which is a build-up of fluid around the heart. Pericarditis is described as recurrent if, following an initial occurrence of pericarditis, it recurs after a symptom-free period of about four to six weeks. Pericarditis is considered chronic if symptoms of any one episode last longer than three months, typically causing significant pain and frustration. If pericarditis is left untreated, patients can develop thickening and scarring of the pericardium, potentially requiring invasive surgical stripping. Pericardial effusion, if large enough, can compress the heart externally, requiring emergent drainage.

We intend to focus our development of rilonacept for the treatment of recurrent pericarditis initially in the United States, and we are exploring opportunities for potential expansion into other countries. Claims analysis, cross validated with published estimates, supports a prevalent population of patients with recurrent pericarditis seeking and receiving medical treatment to be approximately 40,000. Within this estimated diagnosed and treated recurrent pericarditis patient population, there are certain subgroups of patients totaling approximately 14,000 with particularly high unmet medical needs consisting of:

- patients who are refractory to conventional treatments (approximately 3,000);
- patients who are dependent on steroids or not well-controlled on their existing therapy (approximately 6,000); and
- patients who are steroid intolerant and refractory to NSAIDs and colchicine (approximately 5,000).

There may be other thoracic inflammatory syndromes where rilonacept may prove beneficial, such as pericarditis associated with post-pericardiotomy syndrome, an inflammatory reaction of the pericardium in patients who have undergone surgery that involves opening the pericardium. Post-pericardiotomy syndrome occurs in up to 30% of the 300,000 patients in the United States undergoing open heart surgery, and we believe rilonacept may be a therapeutic option for a subset of these patients.

***Current Treatment Landscape for Recurrent Pericarditis***

We are not aware of any current therapies approved by the FDA for the treatment of recurrent pericarditis. A patient's initial acute episode of pericarditis is typically treated with over-the-counter or prescription NSAIDs or colchicine, both of which are used off-label. Recurrent episodes are treated in a similar manner or by adding systemic corticosteroids which are also used off-label. Both colchicine and corticosteroids often have deleterious effects when used at high doses or for long periods of time, including, for colchicine, gastrointestinal distress and neutropenia and, for corticosteroids, glaucoma, fluid retention, hypertension, mood changes, memory changes, other psychological effects, weight gain and diabetes. Fourth-line treatment for these patients may include other immunosuppressants such as methotrexate and azathioprine, as well as anakinra.

***Our Solution***

Rilonacept is a weekly, subcutaneously-injected, recombinant fusion protein that blocks IL-1α and IL-1β signaling. Beyond recurrent pericarditis, we believe there is significant potential for rilonacept to address additional indications, including other pericarditis populations. More broadly, we believe diseases characterized by painful serosal inflammation may be driven by IL-1α, and we intend to consider development of rilonacept in these indications and in others where we believe IL-1α or IL-1β play a key role in disease pathophysiology.

*Clinical Development Plan for Recurrent Pericarditis*

We are enrolling a pivotal, global Phase 3 clinical trial, named RHAPSODY. The study is intended to evaluate the efficacy and safety of rilonacept treatment in subjects with recurrent pericarditis. RHAPSODY is a double-blind, placebo-controlled, randomized withdrawal, or RW study with an open-label extension period. We expect that up to approximately 50 subjects will be randomized into the RW period. Eligible subjects must present at screening with at least a third pericarditis episode, defined as at least 1 day with pericarditis pain ≥ 4 on the 11-point NRS and a C-reactive protein, or CRP, value ≥1 mg/dL within the 7-day period prior to first study drug administration. Subjects included in the study may be receiving concomitant nonsteroidal anti-inflammatory drugs (NSAIDs) or colchicine or oral corticosteroid treatment in any combination.

The clinical study is comprised of 5 periods:

- a screening period;
- a single-blind run-in period during which subjects receive a 320 mg loading dose of rilonacept subcutaneously followed by 160 mg SC weekly while background pericarditis medications are tapered and discontinued;
- a double-blind, placebo-controlled 24-week RW period where clinical responders to rilonacept are randomized 1:1 to 160 mg SC weekly rilonacept or placebo;
- a long-term extension treatment period for where all subjects completing the RW period have the option to receive up to 24-weeks of open-label rilonacept 160 mg SC weekly; and
- a long-term extension follow-up period during which all subjects in the long-term extension period will be followed for 24 weeks for safety and pericarditis recurrences.

The primary efficacy endpoint is time-to-first-pericarditis-recurrence in the RW period. The Clinical Endpoint Committee will adjudicate all suspected pericarditis recurrences for inclusion in the primary efficacy endpoint analysis. We expect to complete enrollment in this trial in the second half of 2019 and report top-line data in the second half of 2020.

We have an ongoing open-label Phase 2 proof-of-concept clinical trial for rilonacept in recurrent pericarditis in order to gain further experience with rilonacept in different pericarditis populations. This trial evaluates the treatment response to rilonacept in subjects with both symptomatic recurrent pericarditis as well as other patient subsets within pericarditis, including asymptomatic steroid-dependent subjects with recurrent pericarditis and subjects with post-pericardiotomy syndrome. The trial is divided into five parts, each enrolling subjects who are currently on any combination of co-administered NSAIDs, colchicine or corticosteroids. The subjects are dosed using the approved rilonacept dose for CAPS, which is a loading dose of two 160 mg subcutaneous doses (320 mg total), followed by single, self-administered 160 mg subcutaneous doses every seven days for a total of six weeks. This is followed by an 18-week extension period. During the extension period, the investigator may choose to wean concomitant NSAIDs, colchicine or corticosteroids according to standard-of-care paradigms. The assessed efficacy outcomes measures include an 11-point pain Numerical Rating Scale or, NRS, CRP, electrocardiogram, and size of pericardial effusion.

The five parts of the trial with different patient populations are:

- Part 1: Symptomatic subjects with recurrent pericarditis receiving NSAIDS +/− colchicine +/− steroids with high CRP, a marker of inflammation, measurements;
- Part 2: Symptomatic subjects with recurrent pericarditis receiving NSAIDS +/− colchicine +/− steroids without elevated CRP measurements but with evidence of pericardial inflammation by MRI;

- Part 3: Subjects with recurrent pericarditis who are dependent upon or unable to wean off of corticosteroids;

- Part 4: Symptomatic subjects with postpericardiotomy syndrome receiving NSAIDS +/− colchicine +/− steroids with high CRP measurements; and

- Part 5: Subjects with postpericardiotomy syndrome who are dependent upon or unable to wean off of corticosteroids.

In December 2018, we reported interim data from Part 1 of the open-label Phase 2 proof-of-concept clinical trial that showed a reduction in both inflammation and reported pain in the 6-week base treatment period and a persistent clinical response during the optional 18-week extension period.

As of the November 1, 2018 interim data analysis cutoff, 12 subjects, each with at least 3 episodes of pericarditis and elevated CRP (>1 mg/dL), enrolled in a 6-week base treatment period. Results showed a reduction in both a biomarker of inflammation (CRP) and reported pain (NRS) after the first dose and a persistent clinical response throughout the 6-week base treatment period:

- mean patient-reported pericardial pain on a 11-point NRS decreased from 4.6 at baseline (n=12) to 0.9 at 6 weeks (n=8);

- mean CRP decreased from 4.9 mg/dL at baseline (n=12) to 0.37 mg/dL at 6 weeks (n=4); median time to CRP normalization was 9 days (n=12); and

- pericardial signs resolved, including pericardial effusion (5/6 subjects), PR depression (3/4 subjects), widespread ST elevation (2/2 subjects), and pericardial rub (3/3 subjects).

As of November 1, 2018, 10 of the 12 enrolled subjects received at least 6 weeks of treatment with rilonacept, 6 continued into the optional 18-week extension period, and 4 completed 24 weeks of treatment. These subjects exhibited a continued clinical response to rilonacept, including as described below:

- mean patient-reported pericardial pain on a 11-point NRS was 0.3, and mean CRP was 0.44 mg/dL at 24 weeks (n=4);

- the pericardial effusion in the 1 remaining subject resolved during the extension period; and

- of the 4 subjects on corticosteroids at baseline, the 1 subject who had completed 24 weeks of treatment successfully tapered off corticosteroids.

Rilonacept has been generally well-tolerated in the study, with AEs consistent with the FDA-approved label for the treatment of CAPS, including Familial Cold Auto-inflammatory Syndrome and Muckle-Wells Syndrome. The most common AEs were gastrointestinal disorders and injection site reactions. Seven of 12 subjects experienced at least one treatment-related adverse event during the treatment period. There was one treatment-related serious AE which resulted in discontinuation: a skin abscess which responded to medical treatment. Infections are reported in the rilonacept label for CAPs.

We closed enrollment for the open-label Phase 2 proof-of-concept study in December 2018 and we expect to present additional data from this trial at ACC in March of 2019.

*Clinical History of Rilonacept*

Regeneron evaluated rilonacept in a total of 21 clinical trials, including two trials in over 100 patients for the treatment of CAPS, and six trials in over 1,800 patients for the treatment of gout flares.

- *CAPS:* Regeneron evaluated rilonacept for the treatment of CAPS in two trials. In these trials, 109 patients with CAPS, including eight pediatric patients, were treated with at least one dose of rilonacept. In the pivotal efficacy trial, which evaluated the long-term efficacy and safety of once-weekly dosing, 160 mg of rilonacept markedly decreased the clinical signs and symptoms of CAPS.

- *Gout:* Regeneron evaluated rilonacept for the treatment of gout flares in six trials. In the two pivotal efficacy trials in patients with gout, which evaluated the efficacy of once-weekly dosing for the prevention of gout flares during initiation of uric acid-lowering therapy, rilonacept at doses of 80 mg and 160 mg significantly decreased the number of gout flares. Regeneron abandoned active development for the treatment of gout flares after receiving a complete response letter from the FDA requesting additional clinical data, as well as additional CMC information related to a proposed new dosage form Regeneron was evaluating for gout, which was different than the dosage form approved in the CAPS indication and now being used for pericarditis.

- *Other Indications:* Regeneron conducted a total of 13 clinical trials of rilonacept for the treatment of rheumatoid arthritis, or RA, polymyalgia rheumatica, osteoarthritis, coronary artery disease, systemic juvenile idiopathic arthritis and end-stage renal disease.

In the 21 clinical studies conducted by Regeneron with rilonacept to date, the most common adverse events reported were injection site reactions and upper respiratory tract infections. Across these studies, there were a total of five serious adverse events, or SAEs, that were assessed by investigators as drug related. Among patients treated with rilonacept there were three SAEs, colitis, gastrointestinal hemorrhage, and drug eruption. One patient treated with placebo experienced cellulitis and another placebo-treated patient died. The largest clinical programs conducted by Regeneron with rilonacept were its Phase 2 and Phase 3 programs for gout flare prevention, which treated a total of 1,886 patients. The most common adverse events reported for the 160 mg dose, the dosage used for the treatment of CAPS, were injection site reactions (15.5% for rilonacept versus 2.6% for placebo) and upper respiratory tract infections (10.3% for rilonacept versus 10.1% for placebo).

**Mavrilimumab**

***Overview***

Mavrilimumab is a fully-human monoclonal antibody that antagonizes GM-CSF signaling by binding to the alpha subunit of the GM-CSF receptor. Our lead indication for mavrilimumab is GCA, a chronic inflammatory disease of medium-large blood vessels with an estimated U.S. prevalence of approximately 75,000 – 150,000 patients. We have commenced dosing in multiple countries in a randomized, double-blind, placebo-controlled, global Phase 2 proof-of-concept trial for the study of mavrilimumab in GCA. We expect to complete enrollment in this trial in the second half of 2019 and report top-line data in the second half of 2020.

Before we licensed mavrilimumab in 2017, MedImmune, Limited, or MedImmune, was developing mavrilimumab for the treatment of RA.

***Mechanism of Action***

Mavrilimumab is designed to inhibit the signaling of GM-CSF, a growth factor that stimulates the production of certain types of white blood cells. Studies have demonstrated that with GM-CSF overexpression, pathological changes almost always follow. Reported data suggest GM-CSF is a key player in autoinflammation and autoimmunity, as follows:

- GM-CSF enhanced trafficking of myeloid cells through activated endothelium of blood vessels and contributed to monocyte and macrophage accumulation in blood vessels during inflammation;

- GM-CSF promoted activation, differentiation, survival and proliferation of monocytes and macrophages, as well as resident tissue macrophages in inflamed tissues;

- GM-CSF production led to activation of the vasculature and bone marrow and also promoted the differentiation of effector T cells at inflamed sites and draining lymph nodes; and

- GM-CSF regulated the phenotype of antigen-presenting cells in inflamed tissues by promoting the differentiation of infiltrating monocytes into M1 macrophages and monocyte-derived dendritic cells, or MoDCs.

Additionally, GM-CSF has been shown to be a confirmed mediator in RA based on the results from the Phase 2b clinical trial in RA conducted by MedImmune. In this trial, mavrilimumab achieved the co-primary endpoints of change from baseline in disease activity score, or DAS, at week 12 and a response of 20% or greater improvement in the American College of Rheumatology criteria, at week 24. Patients with mavrilimumab showed a statistically significant reduction in DAS scores at all dosages compared to placebo, and significantly more mavrilimumab-treated patients achieved ACR20 at all dosages compared to placebo.

***Background and Market Opportunity for Giant Cell Arteritis***

GCA is an inflammatory disease of the blood vessels that strikes older adults and causes headaches, jaw and other muscle claudication, and possible ischemic visual loss. Many of the symptoms and signs of GCA result from involvement of the cranial branches of arteries that originate from the aortic arch, but the disease is systemic, and vascular involvement can be widespread. GCA is characterized by infiltration of monocytes, macrophages and the formation of giant cells (i.e., multinucleated fusions of macrophages). GCA generally occurs in adults over 50 years old with a 3:1 imbalance of women to men. We estimate there to be approximately 75,000 to 150,000 prevalent patients with GCA in the United States with similar prevalence rates for other major markets and believe that the incidence of GCA will increase over time as the population ages.

***Current Treatment Landscape for Giant Cell Arteritis***

Glucocorticoids, a type of corticosteroid, are the mainstay for the treatment of GCA because they normalize inflammatory markers and resolve patient symptoms. Many patients receive long courses of this therapy to prevent disease flare-up, which are associated with significant and serious side effects, including glaucoma, fluid retention, hypertension, mood changes, memory changes, other psychological effects, weight gain and diabetes. Up to 80% of patients suffer from glucocorticoid toxicity as a result of GCA treatment.

Despite being effective for some patients, many are unable to wean off of corticosteroids because they continue to experience disease flares as the dose is reduced. In one study cohort published in the literature that followed 106 patients with GCA for 4.5 to 10.1 years, 68 patients (64%) experienced at least one relapse during or after weaning, and 38 patients (36%) experienced two or more. Experimental evidence in mice suggests that corticosteroid treatment does not adequately suppress tissue-infiltrating macrophage function, a key cell type generated and maintained by GM-CSF signaling, and may explain why many patients require long-term chronic treatment and are unable to wean off corticosteroids. We believe by blocking GM-CSF signaling, mavrilimumab may provide additional benefit to these patients by reducing long-term sequelae that results from chronic vessel inflammation.

In addition, tocilizumab, an inhibitor of interleukin-6, or IL-6, is approved in the United States in GCA for use on top of a concomitant corticosteroid taper. However, nearly half of the patients studied in the Phase 3 clinical trial for tocilizumab experienced disease flares during the 52 weeks treatment period that included a 26-week corticosteroid taper. We believe this indicates a persistent unmet medical need.

***Our Solution***

We chose GCA as our first indication for mavrilimumab due to the mechanistic rationale of inhibiting GM-CSF. GM-CSF is a key growth factor for many of these key inflammatory cell types and is found in high

concentrations at the site of damage in the vessel wall. We believe these data provide a solid rationale for antagonizing this signaling with mavrilimumab.

*Phase 2 Clinical Trial for GCA*

We have commenced dosing in a randomized, double-blind, placebo-controlled, global Phase 2 proof-of-concept trial in multiple countries. In the United States, the FDA initially placed our IND on clinical hold due a request for additional information regarding the 510(k)-cleared delivery device to be used in our Phase 2 clinical trial. The device-related information request did not pertain to pre-clinical toxicology data nor the design of our trial. We have since provided the FDA with the requested information and the IND is now active. We plan for U.S. subjects to be included in the ongoing, global Phase 2 clinical trial, in which dosing has already commenced in multiple countries. We expect to complete enrollment in this trial in the second half of 2019 and report top-line data in the second half of 2020.

The Phase 2 clinical trial of mavrilimumab for the treatment of GCA is expected to enroll approximately 60 subjects with new-onset and refractory disease. Subjects will be randomized 3:2 to mavrilimumab 150 mg or placebo injected SC once every two weeks co-administered with a corticosteroid taper. Treatment duration is 26 weeks, and the primary efficacy endpoint is time to first flare.

We anticipate that to help inform the risk/benefit profile for the use of mavrilimumab in GCA, we will need to demonstrate the effectiveness and safety of mavrilimumab after 26 weeks, as well as evaluate its pharmacokinetic profile and the effectiveness of mavrilimumab at different doses. We also intend to initiate research and development activities of mavrilimumab's potential across other various disease states where cells of myeloid phenotype have been implicated by the literature, such as other vasculitides and cardiomyopathies, diseases characterized by barrier dysfunction, other arthropathies or oncologic indications.

*Clinical History in Rheumatoid Arthritis*

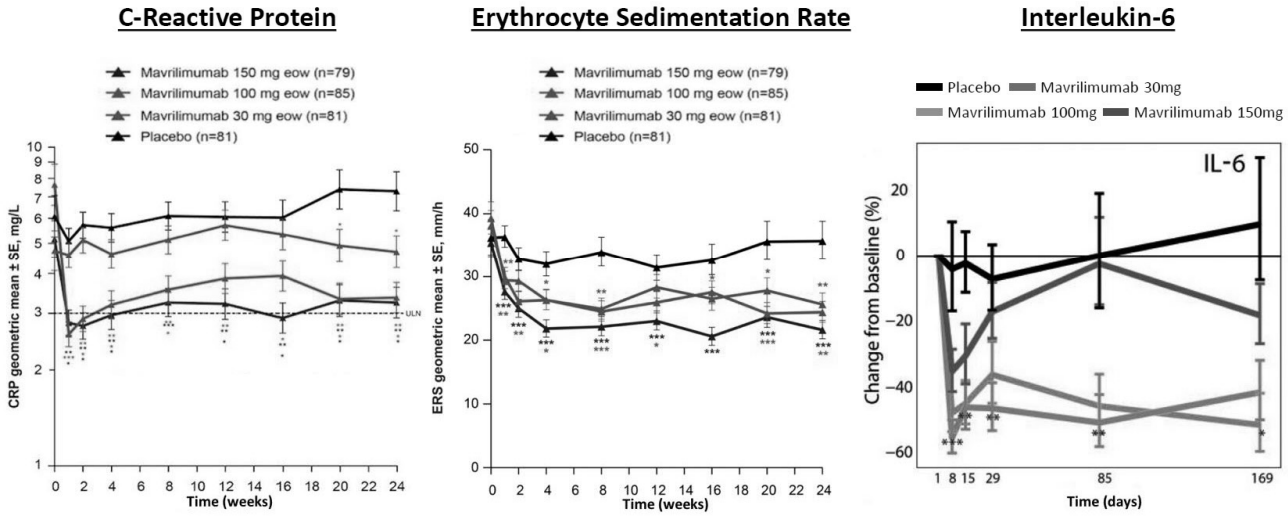
MedImmune had received authorization to conduct clinical trials for rheumatoid arthritis, or RA, in Europe and executed an extensive Phase 1 and Phase 2 clinical program where the company studied mavrilimumab in over 550 patients with RA through Phase 2b. All of MedImmune's European clinical trials achieved their prospectively defined primary endpoints of safety or efficacy.

MedImmune's IND for the clinical development of mavrilimumab for the treatment of RA was initially put on clinical hold in 2010 before human data had been generated due to certain effects that were observed in non-clinical studies, which coincides with a theoretical risk of developing PAP, possibly in the setting of GM-CSF inhibition. Since then, in 2014, the FDA acknowledged that clinical studies in refractory RA may be appropriate based on MedImmune's clinical studies in Europe in which it dosed over 550 RA patients with mavrilimumab with no evidence of PAP attributable to mavrilimumab following long-term administration. MedImmune did not engage in further dialogue with the FDA and withdrew the IND for mavrilimumab for the treatment of RA.

We believe that the trials conducted by MedImmune provide substantial support for the potential of mavrilimumab in autoimmune diseases. In these trials, mavrilimumab was observed to be well-tolerated. The most common adverse event was infection, with all dose groups (30 mg, 100 mg, 150 mg) in a Phase 2b clinical trial reporting similar rates of infection compared to the placebo group. We believe that these safety results provide an accurate early representation of the safety profile of mavrilimumab, which we believe to be at least competitive with and potentially better than existing systemically administered agents for autoimmune diseases.

Mavrilimumab's results from Phase 2b clinical trials in RA have provided important information about its safety and efficacy profile and helped solidify our choice for focusing our development efforts in GCA as a lead indication. In addition to the reductions to the primary endpoint demonstrated in the Phase 2b trials, other markers of inflammation, such as CRP, erythrocyte sedimentation rate, or ESR, and IL-6, were similarly reduced, as shown in the

graphs below. CRP, ESR and IL-6 are key markers of disease activity for GCA. We believe that these results may also provide evidence for mavrilimumab's utility across a broad range of indications with a similar biomarker profile.




**KPL-716**

***Overview***

KPL-716 is a fully-human monoclonal antibody that targets OSMRβ, which mediates signaling of IL-31 and OSM, two key cytokines implicated in inflammation, pruritus and fibrosis. We believe KPL-716 to be the only monoclonal antibody in development that targets both pathways simultaneously. We are initially evaluating KPL-716 for the treatment of a variety of pruritic diseases, including prurigo nodularis, a chronic inflammatory skin condition with an estimated U.S. prevalence of approximately 300,000 patients. In September 2018, we announced interim results from the randomized, double-blind, placebo-controlled, single-ascending-dose, sequential-group portion of the Phase 1a/1b clinical trial of KPL-716 in healthy volunteers and in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus. In this trial, single IV and SC doses were well-tolerated. Results also provided an early signal of efficacy in reducing pruritus (assessed by the Worst-Itch Numeric Rating Scale), as well as reducing inflammation and disease severity (assessed by EASI), indicative of target engagement. In addition, we observed a reduction of EASI scores in atopic dermatitis subjects after a single dose of KPL-716 in a placebo-controlled exploratory efficacy assessment in subjects who participated in the first portion of the Phase 1a/1b clinical study (20 subjects were randomized 1:1 and received either 7.5 mg/kg of KPL-716 or placebo). We believe these single-dose results provide proof-of-principle for KPL-716's potential to treat a spectrum of IL-31-driven pruritic diseases, and support our plans to advance KPL-716 into multiple chronic pruritic diseases. We plan to initiate an adaptive design Phase 2a/2b clinical trial in prurigo nodularis in the first half of 2019 and expect to report top-line data from the first part of this trial in the first half of 2020. We also plan to initiate an exploratory, pilot Phase 2 clinical trial in the first half of 2019 designed to explore the role of IL-31 and OSM in a number of diseases characterized by chronic pruritus. To help us to understand whether KPL-716 could be a competitive therapeutic in atopic dermatitis, if approved, and to provide long-term data on IL-31 driven pruritus as a proxy for other disease indications, we are enrolling a repeated-single-dose cohort as an additional part of the Phase 1b clinical trial in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus. We expect to report top-line data from this cohort in the second half of 2019.

We acquired the assets relating to KPL-716 from Biogen MA, Inc., or Biogen, in 2016.

***Mechanism of Action***

The OSMRβ subunit is an IL-6 type receptor which combines with one of two other subunits to form two distinct cytokine receptors used for the signaling of two different cytokines: IL-31, and OSM. IL-31 produced in the

setting of an inflammatory response binds to the IL-31 receptor on keratinocytes, epidermal cells, leading to a sensation of pruritus and further inflammatory responses in the skin. In addition to interacting with IL-31 receptors on keratinocytes, IL-31 also stimulates pruritus directly through IL-31 receptors expressed on unmyelinated C-fibers in the skin responsible for the sensation and transmission of pruritic signaling.

OSM is produced primarily under inflammatory conditions and stimulates dermal fibroblast proliferation and migration as well as synthesis of collagen and glycosaminoglycan in the skin, leading to fibrosis. In addition to these functions, OSM signaling through the type II OSM receptor upregulates interleukin-4, or IL-4, interleukin-13 receptor, or IL-13Rα1, and interleukin-4 receptor, or IL-4Rα, in human skin equivalent cultures, upregulates IL-4Rα in primary human keratinocytes and also impairs expression of filaggrin, loricrin and involucrin (classical "differentiation" markers of the epidermal differentiation complex cluster) in human skin equivalent cultures. These data implicate OSM signaling as important in many autoimmune diseases characterized by barrier dysfunction, fibrosis and inflammation.

KPL-716 inhibits both IL-31 and OSM activities at their respective receptors, potentially disrupting the pruritus, inflammation and fibrosis mediated by these cytokine pathways.

***Background and Market Opportunity for Prurigo Nodularis, Other Chronic Pruritic Diseases and Atopic Dermatitis***

*Prurigo Nodularis*

Prurigo nodularis is a chronic inflammatory skin condition that affects primarily older adults and is characterized by multiple firm and extremely pruritic nodules typically located on the arms and legs. The etiology of prurigo nodularis is largely unknown, however, human biopsy studies have shown that the cytokines IL-31 and OSM and the receptor chains IL-31Rα and OSMRβ are highly expressed in prurigo nodularis lesions. The pruritus is severe and distressing and can be sudden, sporadic or continuous, worsening with heat, sweating or irritation from clothing. The itching sensation in prurigo nodularis is extreme and often leads to scratching to the point of bleeding, infection or pain. Our market research to-date with physicians and patients highlights the severe and debilitating nature of this disease and the significant levels of unmet need. Multiple physicians have reported suicidal tendencies among their prurigo nodularis patients due to an overwhelming inability to control the unrelenting itch. The exact prevalence of prurigo nodularis is unknown, however, we estimate there to be approximately 300,000 prevalent cases in the United States.

*Other Chronic Pruritic Diseases*

In the exploratory, pilot Phase 2 clinical trial that we plan to initiate in the first half of 2019, we currently expect to evaluate study populations with chronic idiopathic urticaria, chronic idiopathic pruritus, plaque psoriasis, lichen planus, and lichen simplex chronicus.

- *Chronic Idiopathic Urticaria.* Chronic idiopathic urticaria is the chronic occurrence of hives without a known cause. We estimate that there are approximately two to three million patients in the United States with chronic idiopathic urticaria. Based on company survey data of over 100 treating physicians, or company survey data, approximately one out of three patients experience pruritus that is refractory to conventional therapies, and among patients treating their chronic idiopathic urticaria with Xolair (omalizumab), 15% to 20% continue to experience pruritus.

- *Chronic Idiopathic Pruritus.* Chronic idiopathic pruritus is chronic itching without a known cause. Based on company survey data, treating physicians report that there is approximately one patient with idiopathic pruritus for every three atopic dermatitis patients, and approximately 50% of these patients experience symptoms lasting for more than one year and one in three treated patients experience refractory pruritus.

- *Plaque Psoriasis.* Plaque psoriasis is the most common form of psoriasis and causes skin lesions with silvery scales. We estimate that there are approximately 12 million patients with plaque psoriasis in the United States with approximately two to three million patients experiencing moderate-to-severe pruritus.

- *Lichen Planus.* Lichen planus is a chronic inflammatory and immune-mediated disease that affects the skin, nails, hair, and mucous membranes. We estimate that there are approximately 500,000 patients in the United States with lichen planus. Based on company survey data, treating physicians report that among treated patients with this disease, approximately one in every three experience refractory pruritus.

- *Lichen Simplex Chronicus.* Lichen simplex chronicus results from chronic itching and scratching, which causes lichenified skin. Based on company survey data, treating physicians report approximately one lichen simplex chronicus patient for every prurigo nodularis patient, which equates to approximately 300,000 addressable patients in the United States, and among treated patients with lichen simplex chronicus, approximately 40% experience refractory pruritus.

*Atopic Dermatitis*

Atopic dermatitis is a chronic inflammatory skin disease that affects approximately 18 million adults in the United States. Human biopsy studies have shown that the cytokines IL-31 and OSM and the receptor chains IL-31Rα and OSMRβ OSMRβ are highly expressed in atopic dermatitis lesions. Based upon public data analyses and discussions with physicians and key opinion leaders in the field, we estimate that approximately 300,000 atopic dermatitis patients in the United States are diagnosed with a moderate-to-severe form of this disease that significantly impairs their professional and social life on a daily basis.

***Current Treatment Landscape for Prurigo Nodularis and Atopic Dermatitis***

*Prurigo Nodularis*

We are not aware of any current FDA-approved therapies for treating prurigo nodularis, and the treatment approach ranges from topical corticosteroids and occlusive steroid containing bandages for more mild patients to systemic corticosteroid, ultraviolet phototherapy and systemic therapies such as thalidomide, methotrexate and cyclosporine for those patients who fail initial treatments. Patients have reported using opioid pain medications to attempt to control the disease in its most severe form.

*Atopic Dermatitis*

Current therapies for atopic dermatitis are generally focused on the topical use of non-biologic small molecules, however, dupilumab (subcutaneously injected antibody directed to inhibiting signaling through IL-4Rα) has recently been approved by the FDA for the treatment of atopic dermatitis.

***Our Solution***

KPL-716 is a fully-human monoclonal antibody that targets two key pathways for the development of pruritus, inflammation and fibrosis through inhibition of OSMRβ. Chronic pruritic diseases are often characterized by a complex interplay among pruritus, inflammation and fibrosis. The pathogenesis of chronic pruritic diseases involves interlocking positive feedback loops in which pruritus causes scratch, and scratch causes reactive inflammation through mechanical disruption of the skin architecture. The decline in skin barrier function and resulting bacterial colonization or infection ultimately increase extracellular matrix formation and collagen deposition, leading to fibrosis. Fibrosis then begets more pruritus through disruption and dysregulation of sensory nerve fiber expression.

Current therapies target only one or two aspects of this complex pathophysiology and are inevitably limited in their effectiveness. Targeting only one pathway may address a single aspect of the symptomatology, e.g., pruritus, but not the full spectrum of the pathophysiologic components of the disease. This point is particularly relevant since OSM is upregulated in many chronic inflammatory skin diseases and synergistically interacts with pruritic and inflammatory pathways. Of particular relevance is the central role of OSM in inflammation and barrier function and its autocrine effects on type II OSM receptor in IL-31-dependent epidermal proliferation and remodeling as well as inflammation.

There is a relatively large body of literature linking inflammatory pruritic and inflammatory diseases to both IL-31 and OSM via signaling though OSMRβ. KPL-716 has been specifically designed to target both pathways simultaneously and thus KPL-716 may disrupt this pathologic cycle in patients afflicted by prurigo nodularis and atopic dermatitis.

*Pre-clinical Development*

In our pre-clinical development program we have observed favorable pharmacokinetics and toxicology characteristics to support clinical development of KPL-716. KPL-716 has shown signs of efficacy in two non-human primate models. In the first, KPL-716 abrogated the pharmacodynamic marker of pruritus in an IL-31 challenge model. A single three milligram per kilogram dose of KPL-716 substantially reduced scratch counts despite multiple repeated injections of IL-31 over several weeks at concentrations we believe to be supraphysiologic in a disease context. In the second non-human primate model, KPL-716 again abrogated the painful response to an injection to an inflammatory agent called carrageenan through the time period measured after a single infusion of KPL-716, implicating OSM in the inflammatory response. We have conducted pre-clinical toxicology studies for KPL-716 with a no adverse event level of 500 milligrams per kilogram with intravenous dosing.

*Phase 1a/1b Clinical Trial*

In early 2017, we filed an IND application and began clinical development with KPL-716 in a Phase 1a/1b clinical trial in healthy volunteers and in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus, respectively.

The first portion of the Phase 1a/1b clinical trial utilized a randomized, double-blind, placebo-controlled, single-ascending-dose, sequential-group design to evaluate the safety, tolerability, pharmacokinetics and immunogenicity of KPL-716 in healthy volunteers and subjects with atopic dermatitis following IV or SC administration. We used the pruritis in atopic dermatitis as a proxy for IL-31-driven pruritic diseases, including prurigo nodularis.

In total, 50 healthy volunteers and 32 subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus received a single dose of KPL-716 or placebo in the Phase 1a/1b clinical trial, with the top dose of 20 mg/kg IV in healthy volunteers and 7.5 mg/kg IV in subjects with atopic dermatitis. There was a seven-day wash out period of prior therapies for all subjects with atopic dermatitis before treatment, and topical corticosteroids, or TCS, were not allowed through day 28. All subjects were given TSC to use as needed after day 28, and rescue medication was provided for atopic dermatitis flares throughout the study. KPL-716 was well-tolerated, no dose-limiting toxicities were observed, and there were no serious adverse events. All drug-related or potentially drug-related treatment-emergent adverse events, or DR-TEAE, were mild, except for one patient who experienced a moderate DR-TEAE of dizziness.

KPL-716 showed dose-dependent elimination consistent with a target-mediated drug disposition profile and was still detectable at least eight weeks after the high dose of 7.5 mg/kg IV in subjects with atopic dermatitis. We believe the available pharmacokinetic and bioavailability data are supportive of testing once every other week or once monthly SC dosing regimens in subsequent studies of KPL-716.

An exploratory analysis of data in 10 subjects with moderate-to-severe atopic dermatitis receiving a single dose of KPL-716 7.5 mg/kg IV versus 10 pooled placebo IV recipients provided an early signal of efficacy for KPL-716 in reducing pruritus, indicative of target engagement. Among these groups, we observed:

- Mean percentage change in weekly-average Worst-Itch Numeric Rating Scale, or WI-NRS, decreased by 40.4% in KPL-716 recipients compared to a 17.6% decrease in placebo recipients at day 28 in the absence of concomitant TCS.

- Mean percentage change in pruritus Visual Analog Scale, or VAS, decreased by 55.4% in KPL-716 recipients compared to a 10.4% decrease in placebo recipients at day 28 in the absence of concomitant TCS.

- 50% of KPL-716 recipients showed a ≥ 4-point reduction in weekly-average WI-NRS, compared to 10% of placebo recipients at day 28 in the absence of concomitant TCS.

- The maximum decrease in WI-NRS at day 28 in the absence of concomitant TCS was ≥ 8 points (1 subject) in KPL-716 recipients compared to a maximum decrease of 4 points in placebo recipients.

- KPL-716 appeared to show a persistent effect on weekly-average WI-NRS in the period after day 28 through day 56, during which concomitant TCS use was permitted.

- KPL-716 recipients reported a 59.5% decrease in sleep-loss VAS compared to a 2.3% decrease in placebo recipients at day 28 in the absence of concomitant TCS.

- The mean percentage change in EASI (a standardized measure of atopic dermatitis disease severity) decreased by 42.3% in KPL-716 recipients compared to a 25% decrease in placebo recipients at Day 28 in absence of concomitant TCS.

We continue to enroll a repeated-single-dose cohort as an additional part of the Phase 1b clinical trial in the United States and Canada. In this randomized, double-blind, placebo-controlled study, subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus receive 360 mg or placebo via SC injection of KPL-716 once weekly for 12 weeks. The clinical study is designed to provide similar and longer-term exposures in order to evaluate safety and chronic efficacy data on both pruritus and inflammation and disease severity (assessed by EASI) response markers compared to the single-dose IV cohort in the Phase 1b clinical trial. We expect to report top-line data from this cohort in the second half of 2019.

*Adaptive Design Phase 2a/2b Clinical Trial in Prurigo Nodularis*

Based on the results from the single-dose IV Phase 1b results in atopic dermatitis subjects, we intend to initiate an adaptive design Phase 2a/2b randomized, double-blind, placebo-controlled clinical trial to investigate the efficacy, safety, tolerability, PK and immunogenicity of KPL-716 administered SC in subjects with moderate to severe prurigo nodularis experiencing moderate to severe pruritus. We expect that the Phase 2a portion of the study will enroll up to approximately 100 subjects with moderate to severe prurigo nodularis experiencing moderate to severe pruritus and will include two arms: one active arm and one placebo arm. A total of 16 doses of study drug, administered subcutaneously, once-weekly for 16 weeks, are planned during the treatment period to assess potential proof of concept in prurigo nodularis on the endpoints of pruritus, sleep, quality of life and disease severity, with dosing to achieve maximum or near-maximum steady-state exposures. The primary and key secondary endpoints, which focus on pruritus, are set at 8 weeks. Other secondary endpoints will explore the impact of KPL-716 versus placebo on pruritus, sleep, quality of life and disease severity over time (at each week of the study treatment period up to and including week 16).

The Phase 2b portion of the study, if enrolled, will include up to approximately 300 subjects with moderate to severe prurigo nodularis experiencing moderate to severe pruritus and will have five arms: four active arms, testing different dose levels or dosing regimens of KPL-716, and one placebo arm. The primary endpoint of the Phase 2b portion will also focus on pruritus at week 8, with other secondary endpoints evaluating pruritus, sleep, quality of life and disease severity over time.

*Exploratory Multi-Indication, Pilot Phase 2 Clinical Trial*

We believe there are multiple chronic pruritic diseases where IL-31 and OSM may play a role in disease pathology. In the first half of 2019, we plan to initiate an exploratory, multi-indication, randomized, double-blind, placebo-controlled, pilot clinical trial designed to (1) explore the role of IL-31 and OSM in a number of diseases

characterized by chronic pruritus seen by dermatologists or allergists and (2) investigate the efficacy, safety and tolerability of KPL-716 in reducing the moderate to severe pruritus experienced by these subjects. We currently expect the study populations to be chronic idiopathic urticaria, chronic idiopathic pruritus, plaque psoriasis, lichen planus, and lichen simplex chronicus.

The trial will be comprised of multiple cohorts, each for a different study population. Each cohort will be an independent sub-study assessing each study population for presence of an IL-31 or OSM protein or ribonucleic acid signature via biopsy and investigating the efficacy, safety and tolerability of KPL-716 administered SC in reducing pruritus in these populations. Investigators and subjects will remain blinded throughout the study. We expect each cohort to enroll up to approximately 26 subjects with each subject experiencing WI-NRS of seven or above at screening. A loading dose of KPL-716 (720 mg) or matching placebo will be administered on day 1, followed by single, weekly SC injections of KPL-716 (360 mg) or matching placebo for the next seven weeks, with the goal of achieving maximum or near maximum exposures at steady state. The goal of this exploratory study is to identify chronic pruritic conditions where IL-31 or OSM may be playing a role and assess the presence or absence of reduction in pruritus after KPL-716 treatment.

**Pre-clinical Development**

***KPL-404***

KPL-404 is a humanized monoclonal antibody that is designed to inhibit the CD40-CD40 ligand interaction, a key T-cell co-stimulatory signal critical for B-cell maturation and immunoglobulin class switching. Since September 2017, we have had a license to conduct research and development on KPL-404 from Primatope, the company that owned or controlled the intellectual property related to KPL-404 and, in March 2019, we acquired the company.

In pre-clinical development, KPL-404 has been observed to have a favorable pharmacokinetic and toxicology profile to support further pre-clinical development. KPL-404 has been effective in multiple non-human primate models of organ transplant rejection, as well as in multiple T-cell dependent antibody response models. We are continuing our pre-clinical activities in KPL-404 in T-cell dependent, B-cell mediated diseases, and expect to file an IND application with the FDA for this program in the second half of 2019 and to initiate a Phase 1 clinical trial in the first half of 2020.

***KPL-045***

KPL-045 is a fully-human monoclonal antibody that is designed to inhibit the CD30-CD30 ligand interaction, a co-stimulatory signal helpful in activating and sustaining memory T-cells. The majority of the therapeutics in development modulating the CD30-CD30 ligand interaction are depleting or conjugated to a toxin for the use in hematological malignancies. To our knowledge, KPL-045 is the only non-depleting antibody targeting primarily autoimmune disease in active clinical development. In August 2017, we licensed this antibody from Novo Nordisk.

In pre-clinical development, KPL-045 has been observed to have a pharmacokinetic profile that supported further pre-clinical development. KPL-045 has showed single-digit nanomolar potency against both human and cynomolgus non-human primate CD30L. We are currently evaluating the progression of KPL-045 pending preclinical data from the program in the context of our portfolio.

**Discovery Activities**

We have initiated internal discovery activities directed toward wholly owned molecules for the treatment of autoinflammatory and autoimmune disease targets where we believe there to be a strong mechanistic rationale and clear differentiation from existing approved agents or those in development.

**License and Acquisition Agreements**

***License Agreement with Regeneron***

In September 2017, we entered into a license agreement with Regeneron, or the Regeneron Agreement. Pursuant to the Regeneron Agreement, Regeneron granted us an exclusive license under certain intellectual property rights controlled by Regeneron to develop and commercialize rilonacept worldwide, aside from Israel, Egypt, Turkey and select countries in the Middle East and northern Africa, which we refer to collectively as the Excluded Territory. In the United States and Japan, our license is initially for all indications other than those involving local administration to the eye or ear, oncology, deficiency of the interleukin-1 receptor antagonist, or DIRA, and CAPS. If we are successful in receiving marketing approval for rilonacept in the United States for a new indication, the scope of the license granted to us will automatically expand to include DIRA and CAPS in the United States and Japan, and we will assume the sales and distribution of rilonacept in these additional indications. Outside the Unites States and Japan, our license is for all indications other than local application to the eye or ear, oncology, CAPS, DIRA and certain periodic fever syndromes set forth in the Regeneron Agreement, collectively the Excluded Indications. Under the Regeneron Agreement, we are obligated to use commercially reasonable efforts to develop and commercialize rilonacept outside of the Excluded Indications in our territory. Upon receiving positive data in a Phase 3 clinical trial, Regeneron will transfer the BLA for rilonacept to us.

We made an upfront payment of $5.0 million to Regeneron and are obligated to make regulatory milestone payments of up to $27.5 million in the aggregate. Thereafter, we have agreed to evenly split profits on our sales of rilonacept with Regeneron after deducting certain commercialization expenses subject to specified limits.

Regeneron has a right of first negotiation over our engagement of third parties to support our promotional activities in excess of a specified level and over the assignment or sale of our rights to any product we develop under the Regeneron Agreement to a third-party. Furthermore, under certain circumstances, we will need Regeneron's prior consent to assign our rights under the Regeneron Agreement.

The Regeneron Agreement will expire on the date on which we, our affiliates or sublicensees are no longer developing or commercializing any product containing rilonacept. We may terminate the agreement for convenience at any time after the date that is 18 months after the effective date of the agreement with 180 days' written notice or one year's written notice if we terminate the agreement following U.S. marketing approval of a rilonacept product developed by us. We may also terminate with three months' written notice if we reasonably determine that rilonacept is unsafe in the indications we are pursuing. Regeneron may terminate the agreement if there is a consecutive twelve (12) month period during which we do not conduct any material development or commercialization activities or we do not grant a sublicense to a third-party to do so, or if we challenge Regeneron's patent rights in any country in our territory. Either party may terminate the agreement in the event of a material breach by the other party that remains uncured for 90 days (or 30 days for payment-related breaches), or by either party due to the insolvency or bankruptcy of the other party.

We have also entered into a clinical supply agreement with Regeneron, or the Supply Agreement. Pursuant to the Supply Agreement, Regeneron has the exclusive right to manufacture and supply all of our requirements of rilonacept for clinical development. If Regeneron determines to discontinue the supply of rilonacept to us, it must use its reasonable efforts to transfer all relevant documentation, materials and technology necessary for the manufacture of rilonacept to us or our designee. The Supply Agreement terminates upon the termination of the Regeneron Agreement or the transfer of technology related to the bulk manufacture of rilonacept.

***License Agreement with MedImmune***

In December 2017, we entered into a license agreement with MedImmune, or the MedImmune Agreement. Pursuant to the MedImmune Agreement, MedImmune granted us an exclusive, worldwide license under certain intellectual property rights controlled by MedImmune to make, use, develop and commercialize mavrilimumab and any other product containing an antibody to the GM-CSF receptor alpha that is covered by certain MedImmune patent rights for all indications. We also acquired non-exclusive licenses to other MedImmune technology for use in exploiting licensed products. We may sublicense these rights subject to consent of MedImmune and any applicable licensors of

rights under which we are licensed. We also acquired reference rights to relevant manufacturing and regulatory documents, and existing inventory of mavrilimumab drug substance. We must use commercially reasonable efforts to develop and commercialize the licensed products.

We made an upfront payment of $8.0 million to MedImmune and are obligated to make future clinical, regulatory and initial sales milestone payments of up to $72.5 million in the aggregate for the first two indications, including a milestone payment of $10.0 million which we paid upon the occurrence of a specified regulatory milestone, and clinical and regulatory milestone payments of up to $15.0 million in the aggregate for each subsequent indication. We are also obligated to make milestone payments to MedImmune of up to $85.0 million upon the achievement of annual net sales thresholds up to, but excluding, $1.0 billion in annual net sales as well as additional milestone payments aggregating up to $1.1 billion upon the achievement of additional annual net sales thresholds starting at $1.0 billion and higher. Commencing on the first commercial sale of licensed products, we are obligated to pay tiered royalties on escalating tiers of annual net sales of licensed products starting in the low double-digit percentages and ending at twenty percent. We must pay such royalties on a product-by-product and country-by-country basis until the latest to occur of the expiration of licensed patents, the expiration of regulatory exclusivity or the tenth anniversary of first commercial sale of such product in such country.

In countries where licensed patents have issued, the statutory expiration date is 2027, not including any patent term extensions or adjustments. While the current expected patent expiration dates are known in countries where licensed patents have issued, these expiration dates are subject to significant uncertainty. For example, the patents may be challenged, and accordingly, the relevant expiration dates could be shortened. In addition, as we continue to file and prosecute patent applications related to mavrilimumab, the granting of pending applications or future patent applications could extend the relevant statutory expiration dates beyond 2027. The expiration date of regulatory exclusivity is determined on a country-by country-basis if the applicable product is approved in such country and if any applicable regulatory exclusivity applies and is granted. The actual expiration date of any such regulatory exclusivity, however, is subject to significant uncertainty. For instance, the applicable regulatory exclusivity period is often triggered by the date a product candidate obtains regulatory approval, and we cannot predict with any certainty whether and if so, when, the applicable product would receive regulatory approval in any given jurisdiction. Furthermore, the type, scope and duration of such exclusivities will vary on a country-by-country basis depending on the jurisdictions in which a product candidate is approved and the particular regulatory exclusivity for which the product is eligible as of the time of approval. For example, in the United States, a reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, which means that the FDA cannot make effective the approval of a biosimilar product that references the biologic product until 12 years from the date on which the reference product was first licensed. In the European Union, new products authorized for marketing may qualify for eight years of data exclusivity and an additional two years of market exclusivity upon marketing authorization. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. Furthermore, if a product candidate that has received orphan designation is subsequently approved for the disease or condition for which it has such designation, the product may be entitled to orphan drug exclusivity, which generally grants seven years of market exclusivity in the United States and up to 10 years of market exclusivity in the European Union, and such period may run contemporaneously with the other exclusivities that may apply. In the European Union, an orphan product can also obtain an additional two years of market exclusivity for pediatric studies. In the United States, an additional six-month period of pediatric exclusivity may be available as an extension to any existing non-patent regulatory exclusivity period if the sponsor has conducted and submitted pediatric studies in response to a written request from the FDA. Additionally, our eligibility for regulatory exclusivity may depend in part on the indications for which we seek regulatory approval of our product candidates, which may depend on the data we receive from our clinical studies, and accordingly, may change over time, and the laws and regulations governing regulatory exclusivity may change in various jurisdictions as the political focus on drug exclusivity increases. For risk related to regulatory exclusivity matters, see “Risk factors—Risks related to product development and regulatory approval.”

The MedImmune Agreement will remain in effect until the expiration of the royalty term in all countries for all licensed products. The MedImmune Agreement may be terminated earlier at any time by us with at least 90 days’ prior notice, by either party in the event of material breach by the other party that remains uncured for 90 days, by either party for insolvency or bankruptcy of the other party, or immediately by MedImmune if we challenge the licensed patents.

***Biogen Asset Purchase Agreement***

In September 2016, we completed the acquisition of certain assets of Biogen pursuant to an asset purchase agreement, or the Biogen Agreement. Pursuant to the Biogen Agreement, we acquired all of Biogen's right, title and interest in and to certain assets used in or relating to KPL-716 and other antibodies covered by certain patent rights, together the Acquired Assets, including patents and other intellectual property rights, clinical data, certain contracts, know-how and inventory. In addition, Biogen granted us a non-exclusive, sublicensable, worldwide license to certain background patent rights related the KPL-716 program. Under the Biogen Agreement, we are obligated to use commercially reasonable efforts to develop and commercialize the Acquired Assets.

Under the Biogen Agreement, we made an upfront payment of $11.5 million and a technology transfer payment of $0.5 million to Biogen. In addition, we made a milestone payment of $4.0 million during the year ended December 31, 2017 associated with the achievement of a specified clinical milestone event. We are also obligated to make future milestone payments for each antibody product that includes the Acquired Assets, or an Antibody Product, of up to $325.0 million in the aggregate upon the achievement of specified milestones. These milestone payments relate to multiple indications for an Antibody Product, and are comprised of up to $175.0 million in the aggregate upon achievement of specified clinical and regulatory milestone events and $150.0 million in the aggregate upon the achievement of specified annual net sales thresholds. Commencing on the first commercial sale of an Antibody Product, we are obligated to pay tiered royalties on escalating tiers of annual net sales of licensed products starting in the high single-digit percentages and ending below the teens. We must pay such royalties on a product-by-product and country-by-country basis until the latest to occur of the expiration of patents that cover an Antibody Product, the expiration of regulatory exclusivity or the tenth anniversary of first commercial sale of such product in such country. We have also agreed to pay certain obligations under third-party contracts retained by Biogen that relate to KPL-716.

In countries where patents covering Antibody Products have issued, the statutory expiration date is 2034, not including any patent term extensions or adjustments. While the current expected patent expiration dates are known in countries where licensed patents have issued, these expiration dates are subject to significant uncertainty. For example, the patents may be challenged, and accordingly, the relevant expiration dates could be shortened. In addition, as we continue to file and prosecute patent applications related to Antibody Products, the granting of pending applications or future patent applications could extend the relevant statutory expiration dates beyond 2034. The expiration date of regulatory exclusivity is determined on a country-by country-basis if the applicable product is approved in such country and if any applicable regulatory exclusivity applies and is granted. The actual expiration date of any such regulatory exclusivity, however, is subject to significant uncertainty. For instance, the applicable regulatory exclusivity period is often triggered by the date a product candidate obtains regulatory approval, and we cannot predict with any certainty whether and if so, when, the applicable product would receive regulatory approval in any given jurisdiction. Furthermore, the type, scope and duration of such exclusivities will vary on a country-by-country basis depending on the jurisdictions in which a product candidate is approved and the particular regulatory exclusivity for which the product is eligible as of the time of approval. For example, in the United States, a reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, which means that the FDA cannot make effective the approval of a biosimilar product that references the biologic product until 12 years from the date on which the reference product was first licensed. In the European Union, new products authorized for marketing may qualify for eight years of data exclusivity and an additional two years of market exclusivity upon marketing authorization. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. Furthermore, if a product candidate that has received orphan designation is subsequently approved for the disease or condition for which it has such designation, the product may be entitled to orphan drug exclusivity, which generally grants seven years of market exclusivity in the United States and up to 10 years of market exclusivity in the European Union, and such period may run contemporaneously with the other exclusivities that may apply. In the European Union, an orphan product can also obtain an additional two years of market exclusivity for pediatric studies. In the United States, an additional six-month period of pediatric exclusivity may be available as an extension to any existing non-patent regulatory exclusivity period if the sponsor has conducted and submitted pediatric studies in response to a written request from the FDA. Additionally, our eligibility for regulatory exclusivity may depend in part on the indications for which we seek regulatory approval of our product candidates, which may depend on the data we receive from our clinical studies, and accordingly, may change over time, and the laws and regulations governing regulatory exclusivity may change in various jurisdictions as the political focus on drug exclusivity increases.

For risk related to regulatory exclusivity matters, see "Risk factors—Risks related to product development and regulatory approval."

Under the Biogen Agreement, Biogen has a time-limited right of first negotiation to purchase the assets we acquired from Biogen or obtain a license to exploit Antibody Products, in each case, in the event we decide to sell the acquired assets, including through the sale of our company, or out-license the rights to the Antibody Products.

The Biogen Agreement will remain in effect until expiration of all payment obligations in all countries related to the last antibody product subject to the Biogen Agreement. The Biogen Agreement may be terminated by us with 90 days' prior notice, by either party in the event of a material breach by the other party that remains uncured for 90 days (or 30 days for payment-related breaches) or by both parties upon mutual consent. In the event of a termination, the Acquired Assets, including certain licenses and rights related thereto, will revert to Biogen, and, upon written request by Biogen, we are required to grant to Biogen an exclusive, worldwide, sub-licensable license to certain of our intellectual property related to the Acquired Assets, including know-how and patent rights.

**Manufacturing**

We do not currently own or operate any large-scale manufacturing facilities. Although we built small-scale manufacturing facilities to produce drug substance to support certain of our pre-clinical and clinical studies, we rely on third parties to manufacture all of our product candidates. We have entered into a clinical supply agreement with Regeneron to manufacture and supply rilonacept for our clinical trials. Regeneron has also agreed to provide commercial drug material until at least the later of four years after U.S. marketing approval or seven years after the effective date of the agreement.

We believe that we have sufficient quantities of drug substance to supply our Phase 2 clinical trial of mavrilimumab for the treatment of GCA. We also acquired a certain amount of finished mavrilimumab drug product that we plan to use in this clinical trial, and have entered into a fill/finish supply agreement with a contract manufacturing organization, or CMO, to produce additional finished mavrilimumab drug product from our current inventory of drug substance. In addition, we transferred the manufacturing process of mavrilimumab to, and entered into an agreement with, the same CMO to produce mavrilimumab drug substance beyond our existing inventory for other clinical trials, including any Phase 3 clinical trial, and eventual commercialization of mavrilimumab, if approved. There are certain components, for example, media and feed, used to produce our current mavrilimumab inventory that the CMO is not able to use in our future manufacturing process. We and this CMO or any other CMO that we enter into agreement with to manufacture mavrilimumab will need to find alternative components to replace the media and feed that had been used by MedImmune to date in the manufacture of mavrilimumab.

We acquired a certain amount of KPL-716 drug substance from Biogen from which we produced KPL-716 drug product using a CMO. In addition, we have engaged CMOs to manufacture KPL-716 drug substance and product for further clinical development activities. We intend to use CMOs for development and scale-up work for any future clinical trials and eventual commercialization of KPL-716, if approved.

We engaged CMOs to produce our pre-clinical product candidates for certain pre-clinical studies, but we intend to produce our pre-clinical product candidates for Phase 1 studies in our own small-scale manufacturing facilities. Longer-term, we expect to use CMOs to produce these product candidates for later-phase clinical studies and eventual commercialization, if approved.

We require our CMOs to conduct manufacturing activities in compliance with current good manufacturing practice, or cGMP, requirements. We have assembled a team of experienced employees and consultants to provide the necessary technical, quality and regulatory oversight of our CMOs. We currently rely solely on these third-party manufacturers for scale-up and process development work and to produce sufficient quantities of product candidate for use in pre-clinical studies and clinical trials. Although we have established our own small-scale manufacturing capabilities to support certain pre-clinical and early clinical-stage production of product candidates, we intend to continue to rely on third-party manufacturers for clinical and commercial supply of our product candidates. We anticipate that these CMOs will have the capacity to support both clinical supply and commercial-scale production, but we do not have any formal agreements at this time with any of these CMOs to cover commercial production. We also may elect to pursue additional CMOs for manufacturing supplies of drug substance and finished drug product in the future.

**Commercial Operations**

Our team is experienced in commercial leadership and we intend to expand our capabilities in parallel with the development path of our product candidates. If the FDA approves rilonacept for recurrent pericarditis, we intend to market and commercialize rilonacept in the United States by developing our own sales, marketing and medical affairs organizations targeting a subset of cardiologists and rheumatologists currently treating pericarditis. For our other product candidates, we intend to establish commercialization strategies for each as we approach potential marketing approval and, due to the specialization among physicians treating the indications we are targeting, we expect to be able leverage our then-existing sales, marketing and medical affairs organizations.

**Competition**

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face potential competition from many different sources, including pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize, including rilonacept, mavrilimumab and KPL-716, may compete with existing products and new products that may become available in the future.

Our competitors may have significantly greater financial resources, established presence in the market, expertise in research and development, manufacturing, pre-clinical and clinical testing, obtaining regulatory approvals and reimbursement and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific, sales, marketing and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of rilonacept, mavrilimumab and KPL-716, and any other product candidates that we develop, if approved, are likely to be their efficacy, safety, convenience, price, the level of generic competition and the availability of reimbursement from government and other third-party payors. Our commercial opportunity for any of our product candidates could be reduced or eliminated if our competitors develop and commercialize products that are more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products.

We are aware of the following products currently marketed or in clinical development for the treatment of the diseases that we are initially targeting:

*Rilonacept*

We are not aware of any therapies currently approved by the FDA for the treatment of recurrent pericarditis, our lead indication for rilonacept. Anakinra (KINERET), produced by Sobi, Inc., is an FDA-approved agent that inhibits IL-1α and IL-1β signaling and is approved for RA and CAPS. Canakinumab (ILARIS), produced by Novartis Pharmaceuticals Corporation, is a monoclonal antibody which inhibits IL-1β signaling and is approved for use in CAPS, tumor necrosis factor receptor associated period syndrome, hyperimmunoglobulin D syndrome, familiar Mediterranean fever and active systemic juvenile idiopathic arthritis. There are also other therapies modulating IL-1α or IL-1β which are in various stages of clinical development for diseases other than recurrent pericarditis from AbbVie, Inc., or AbbVie, XBiotech Inc. and Handok Inc.

*Mavrilimumab*

GCA: Tocilizumab (ACTEMRA), produced by Hoffmann—La Roche AG, or Roche, and Chugai Pharmaceutical Co., Ltd., is an IL-6 inhibitor that is approved by the FDA for the treatment of GCA on top of a concomitant corticosteroid taper. In addition, Eli Lilly and AbbVie are conducting clinical trials for oral janus kinase inhibitors. Sanofi S.A. and Regeneron are recruiting a Phase 3 clinical trial with their anti-IL-6 program, Novartis International AG, is recruiting a trial with their IL-17 antagonist secukinumab (Cosentyx) and Janssen's ustekinumab (STELARA) is being trialed in two small studies for GCA.

GM-CSF antagonists: There are also four other programs in clinical development in various indications that modulate GM-CSF signaling from GlaxoSmithKline plc, or GSK, Izana Bioscience Ltd., I-MAB Biopharma and Humanigen, Inc.

*KPL-716*

We are not aware of any therapies currently approved by the FDA for the treatment of prurigo nodularis. Menlo Therapeutics Inc., Trevi Therapeutics, Inc. and Galderma SA, or Galderma, have programs in various stages of clinical development for the treatment of prurigo nodularis.

The FDA recently approved Regeneron's dupilumab, an antibody that inhibits signaling through the interleukin 4 receptor, to treat atopic dermatitis. Other companies currently developing systemic therapies for atopic dermatitis include Roche, Dermira, Inc., Galderma, Asana BioSciences, LLC, Eli Lilly and Co., Pfizer Inc., AbbVie, Glenmark Pharmaceuticals Ltd., GSK, LEO Pharma Inc., Incyte Corporation, Dermavant Sciences, Inc., Novartis International AG, MedImmune, and AnaptsysBio, Inc.

**Intellectual Property**

Our success depends in part on our ability to obtain and maintain proprietary protection for our drug candidates, manufacturing and process discoveries, and other know-how, to operate without infringing the proprietary rights of others, and to prevent others from infringing our proprietary rights. We plan to protect our proprietary position using a variety of methods, which include pursuing U.S. and foreign patent applications related to our proprietary technology, inventions and improvements, including compositions of matter, drug product formulations, and methods-of-use, that are important to the development and implementation of our business. For example, we or our licensors have or are pursuing patents covering the composition of matter for each of our product candidates and we generally pursue patent protection covering methods-of-use for each clinical program. We also rely on trade secrets, know-how, continuing technological innovation and potential in-licensing opportunities to develop and maintain our proprietary position.

We have a field-specific exclusive license under the Regeneron Agreement to granted patents and pending applications in the United States and numerous foreign jurisdictions relating to rilonacept. As of December 31, 2018, the patent rights in-licensed under the Regeneron Agreement relating to our program include one granted patent in the United States and 54 patents granted in foreign jurisdictions, including Canada, Australia, Brazil and selected countries in Europe and Asia. In addition, the patent rights in-licensed under the Regeneron Agreement relating to our program include patent applications that are pending in the United States. A U.S. patent covering rilonacept as a composition of

matter has a statutory expiration date in 2019, not including patent term adjustment, and relevant foreign counterparts are expected to expire between 2019 and 2023, in each case, not including any patent term extensions. If we are successful in obtaining regulatory approval of rilonacept for the treatment of recurrent pericarditis and receive orphan designation, we would rely on orphan exclusivity, which generally grants seven years of marketing exclusivity in the United States and 10 years of marketing exclusivity in Europe. See "—License agreement with Regeneron" above for additional information on our rights under the Regeneron Agreement.

We have an exclusive license under the MedImmune Agreement to granted patents and pending patent applications in the United States and numerous foreign jurisdictions relating to mavrilimumab. These patents and patent applications cover mavrilimumab as a composition of matter and its use. As of December 31, 2018, the patent rights in-licensed under the MedImmune Agreement relating to our program include three granted patents in the United States and 106 patents granted in foreign jurisdictions, including Canada, Australia and selected countries in Europe and Asia. In addition, the patent rights in-licensed under the MedImmune Agreement relating to our program include patent applications that are pending in the United States and selected countries in Asia and Latin America. The composition of matter patents for mavrilimumab generally have statutory expiration dates in 2027, although the term of some U.S. patents may be longer due to patent term adjustment to compensate for delays during the patent prosecution process. Patent term extension could extend the expiration date of one patent in the United States and patents in certain other jurisdictions, each in accordance with applicable law. There can be no assurances that patents will issue from any pending patent applications. See "—License agreement with MedImmune" above for additional information on our rights under the MedImmune Agreement.

We own, via our acquisition of certain assets from Biogen, granted patents and pending patent applications in the United States and numerous foreign jurisdictions relating to KPL-716. These patents and patent applications cover KPL-716 as a composition of matter and its use. As of December 31, 2018, the patent rights acquired from Biogen include three patents granted in the United States and 30 patents granted in foreign jurisdictions, including Australia, Mexico and selected countries in Europe and Asia. In addition, the patent rights acquired from Biogen include patent applications pending in the United States, Europe, Canada, and selected countries in Asia. The issued composition of matter patents for KPL-716 have statutory expiration dates in 2034. Patent term extension could extend the expiration date of one patent in the United States and patents in certain other jurisdictions, each in accordance with applicable law. There can be no assurance that patents will issue from any of our pending patent applications. See "—Biogen asset purchase agreement" above for additional information on our rights under the Biogen Agreement.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO, in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier expiring patent. In certain countries, the term of a patent that covers a drug product may also be eligible for patent term extension when regulatory approval is granted, provided the legal requirements are met. In the future, if and when our drug candidates receive approval by the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents covering those drugs, depending upon the length of the clinical trials for each drug and other factors. There can be no assurance that any of our pending patent applications will issue or that we will benefit from any patent term extension or favorable adjustment to the term of any of our patents.

**Government Regulation**

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

***U.S. Government Regulation of Biological Products***

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

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

- Completion of extensive pre-clinical studies and tests in accordance with applicable regulations, including Good Laboratory Practice, or GLP, regulations and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- Submission to FDA of an IND which must become effective before human clinical trials may begin;
- Approval by an independent institutional review board, or IRB, or ethics committee at each clinical trial site before each trial may be initiated;
- Performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, good clinical practices, or GCPs, and other clinical-trial related regulations to evaluate the safety and efficacy of the investigational product for each proposed indication;
- Submission to FDA of a BLA for marketing approval that includes substantive evidence of safety, purity, and potency from results of pre-clinical testing and clinical trials;
- A determination by FDA within 60 days of its receipt of a BLA to accept the filing for review;
- Satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the biologic will be produced to assess compliance with cGMPs to assure that the facilities, methods and controls used in product manufacture are adequate to preserve the biologic's identity, strength, quality and purity;
- Potential FDA audit of the pre-clinical or clinical trial sites that generated the data in support of the BLA;
- Payment of user fees for FDA review of the BLA; and
- FDA review and approval of the BLA, including satisfactory completion of an FDA advisory committee review, if applicable, prior to any commercial marketing or sale of the product in the United States.

***Pre-clinical Studies***

Before testing any biological product candidate, including our product candidates, in humans, the product candidate must undergo rigorous pre-clinical testing. The pre-clinical development stage generally involves laboratory evaluations of the chemistry, formulation and stability of the product candidate, as well as trials to evaluate toxicity in animals, which support subsequent clinical testing. The conduct of the pre-clinical tests must comply with federal regulations and requirements, including GLP regulations. The sponsor must submit the results of the pre-clinical studies,

together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. An IND is a request for authorization from the FDA to administer an investigational product to humans, and must become effective before human clinical trials may begin. Some pre-clinical testing may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

***Clinical Trials***

The clinical stage of development involves the administration of the investigational product to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by, or under control of, the trial sponsor, in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative, and must monitor the clinical trial until completed. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Information about most clinical trials must be submitted within specific timeframes for publication on the *www.clinicaltrials.gov* website. Information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed in some cases for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

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

- Phase 1 clinical trials generally involve a small number of healthy volunteers or disease-affected patients who are initially exposed to a single dose and then multiple doses of the product candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacologic action, side effect tolerability and safety of the drug.

- Phase 2 clinical trials involve studies in disease-affected patients to determine the dose required to produce the desired benefits. At the same time, safety and further pharmacokinetic and pharmacodynamic information is collected, possible adverse effects and safety risks are identified and a preliminary evaluation of efficacy is conducted.

- Phase 3 clinical trials generally involve a larger number of patients at multiple sites and are designed to provide the data necessary to demonstrate the effectiveness of the product for its intended use, its safety in use and to establish the overall benefit/risk relationship of the product and provide an adequate basis for product approval. These trials may include comparisons with placebo or other comparator treatments. The duration of treatment is often extended to mimic the actual use of a product during marketing.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow up. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of a BLA.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. The FDA or the sponsor may suspend or terminate a clinical trial at any time or the FDA may impose other sanctions on various grounds, including a finding that the research patients are being exposed to an unacceptable health risk. Similarly, an IRB can refuse, suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRBs requirements or if the drug has been associated with unexpected serious harm to patients.

Concurrently with clinical trials, companies usually complete additional pre-clinical studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

***BLA Review and Approval***

Assuming successful completion of the required clinical testing, the results of the pre-clinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must contain proof of safety, purity, potency and efficacy and may include both negative and ambiguous results of pre-clinical studies and clinical trials as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA approval of a BLA must be obtained before a biologic may be marketed in the United States.

In most cases, the submission of a BLA is subject to a substantial application user fee. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, for original BLAs, the FDA has ten months from the filing date in which to complete its initial review of a standard application and respond to the applicant, and six months from the filing date for an application with priority review. The FDA does not always meet its PDUFA goal dates, and the review process is often significantly extended by FDA requests for additional information or clarification. This review typically takes twelve months from the date the BLA is submitted to the FDA because the FDA has approximately two months to make a "filing" decision.

Before approving a BLA, the FDA will typically conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether the manufacturing processes and facilities comply with cGMPs. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. The FDA also may inspect the sponsor and one or more clinical trial sites to assure compliance with GCP requirements and the integrity of the clinical data submitted to the FDA.

Additionally, the FDA may refer applications for novel biologic candidates which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions, if any. The FDA is not bound by recommendations of an advisory committee, but it considers such recommendations when making decisions on approval. The FDA likely will re-analyze the clinical trial data, which could result in extensive discussions between the FDA and the applicant during the review process. The FDA also may require submission of a REMS plan if it determines that a REMS is necessary to ensure that the benefits of the drug outweigh its risks and to assure the safe use of the biological product. The REMS plan could include medication guides, physician communication plans, assessment plans or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools. The FDA determines the requirement for a REMS, as well as the specific

REMS provisions, on a case-by-case basis. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS. The FDA will not approve a BLA without a REMS, if required.

Under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data that are adequate to assess the safety and efficacy of the product candidate for the claimed indications in all relevant pediatric populations and to support dosing and administration for each pediatric population for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. The Food and Drug Administration Safety and Innovation Act, or FDASIA, amended the FDCA to require that a sponsor who is planning to submit a marketing application for a 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, or PSP, within sixty days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 clinical trial. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including trial 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 and the sponsor must reach an 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 pre-clinical studies, early phase clinical trials or other clinical development programs.

After the FDA evaluates a BLA, it will issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the BLA identified by the FDA. The Complete Response Letter may require additional clinical or other data, additional pivotal Phase 3 clinical trial(s) or other significant and time-consuming requirements related to clinical trials, pre-clinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information are submitted, the FDA may decide that the re-submitted BLA does not satisfy the criteria for approval.

If a product receives regulatory approval, the approval is limited to the conditions of use (e.g., patient population, indication) described in the application. Further, depending on the specific risk(s) to be addressed, the FDA may require that contraindications, warnings or precautions be included in the product labeling, require that post-approval trials, including Phase 4 clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing trials or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

***Orphan Drug Designation***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States for this type of disease or condition will be recovered from sales of the product in the United States. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent 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. Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years from the date of such approval, except in

limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity by means of greater effectiveness, greater safety, by providing a major contribution to patient care or in instances of drug supply issues. Competitors, however, may receive approval of either a different product for the same indication or the same product for a different indication that could be used "off-label" by physicians in the orphan indication, even though the competitor's product is not approved in the orphan indication. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval before we do of the same product, as defined by the FDA, for the same indication we are seeking, or if our product candidate is determined to be contained within the scope of the competitor's product for the same indication or disease. If one of our products designated as an orphan drug receives marketing approval for an indication broader than that which is designated, it may not be entitled to orphan drug exclusivity. Orphan drug status in the European Union has similar, but not identical, requirements and benefits.

***Expedited Review and Approval***

The FDA has various programs, including fast track designation, breakthrough therapy designation, accelerated approval and priority review, which are intended to expedite or simplify the process for the development and FDA review of drugs and biologics that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs and biologics to patients earlier than under standard FDA review procedures.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need by providing a therapy where none exists or a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. Fast track designation provides opportunities for more frequent interactions with the FDA review team to expedite development and review of the product. The FDA may also review sections of the BLA for a fast track product on a rolling basis before the complete application is submitted, if the sponsor and the FDA agree on a schedule for the submission of the application sections, and the sponsor pays any required user fees upon submission of the first section of the BLA.

In addition, under the provisions of the or FDASIA, passed in July 2012, a sponsor can request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biologic may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs or biologics designated as breakthrough therapies are also eligible for accelerated approval. The FDA must take certain actions with respect to breakthrough therapies, such as holding timely meetings with and providing advice to the product sponsor, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require a sponsor of a product receiving accelerated approval to perform post-marketing trials to verify and describe the predicted effect on IMM or other clinical endpoint, and the product may be subject to expedited withdrawal procedures.

Once a BLA is submitted for a product intended to treat a serious condition, the FDA may assign a priority review designation if the FDA determines that the product, if approved, would provide a significant improvement in safety or effectiveness. Under priority review, the FDA must review an application in six months, compared to ten months for a standard review. Most products that are eligible for fast track or breakthrough therapy designation are also likely to be considered appropriate to receive a priority review.

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

***Post-approval Requirements***

Following approval of a new product, the manufacturer and the approved product are subject to pervasive and continuing regulation by the FDA, including, among other things, monitoring and recordkeeping activities, reporting of adverse experiences with the product, product sampling and distribution restrictions, complying with promotion and advertising requirements, which include restrictions on promoting drugs for unapproved uses or patient populations (i.e., "off-label use") and limitations on industry-sponsored scientific and educational activities. Although physicians may prescribe legally available products for off-label uses, manufacturers may not market or promote such uses. 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. If there are any modifications to the product, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new BLA or BLA supplement, which may require the applicant to develop additional data or conduct additional pre-clinical studies and clinical trials. The FDA may also place other conditions on approvals including the requirement for a REMS to assure the safe use of the product. A REMS could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Product approvals may be withdrawn for non-compliance with regulatory standards or if problems occur following initial marketing.

FDA regulations require that products be manufactured in specific approved facilities and in accordance with cGMPs. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. These manufacturers must comply with cGMP regulations that require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved drugs or biologics 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. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. The discovery of conditions that violate these rules, including failure to conform to cGMPs, could result in enforcement actions, and the discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved BLA, including voluntary recall.

Once an approval or clearance of a drug is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or other enforcement-related letters or clinical holds on post-approval clinical trials;
- refusal of the FDA to approve pending BLAs or supplements to approved BLAs, or suspension or revocation of product approvals;

- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- consent decrees, corporate integrity agreements, debarment, or exclusion from federal healthcare programs; or mandated modification of promotional materials and labeling and the issuance of corrective information.

***Biosimilars and Exclusivity***

An abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product was created by the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act. This amendment to the PHSA, in part, attempts to minimize duplicative testing. A recent federal district court ruling struck down the Affordable Care Act in its entirety. This decision means numerous reforms enacted as part of the Affordable Care Act, but not specifically related to health insurance, such as the BPCIA, are invalid as well. While the presidential administration and CMS have both stated that the ruling will have no immediate effect, it is unclear how this decision, subsequent appeals, if any, and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business. To date, the FDA has approved a number of biosimilars, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining its approach to reviewing and approving biosimilars.

Biosimilarity, which requires that the biological product be highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there be no clinically meaningful differences between the product and the reference product in terms of safety, purity and potency, must be shown through analytical studies, animal studies and a clinical trial or trials. Interchangeability requires that a biological product be biosimilar to the reference product and that the product can be expected to produce the same clinical results as the reference product in any given patient and, for products administered multiple times to an individual, that the product and the reference product 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 biological product without such alternation or switch. Complexities associated with the larger, and often more complex, structure of biological products as compared to small molecule drugs, as well as the processes by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product. “First licensure” typically means the initial date the particular product at issue was licensed in the United States. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a supplement for the reference product for a subsequent application filed by the same sponsor or manufacturer of the reference product (or licensor, predecessor in interest or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength or for a modification to the structure of the biological product that does not result in a change in safety, purity or potency. Therefore, one must determine whether a new product includes a modification to the structure of a previously licensed product that results in a change in safety, purity or potency to assess whether the licensure of the new product is a first licensure that triggers its own period of exclusivity. Whether a subsequent application, if approved, warrants exclusivity as the “first licensure” of a biological product is determined on a case-by-case basis with data submitted by the sponsor.

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.

***U.S. Patent Term Restoration***

Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension must be based on the first approval for the product, and the extension cannot extend the total patent term beyond fourteen years from approval. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner.

***European Union Drug Development, Review and Approval***

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

Similar to the United States, the various phases of pre-clinical and clinical research in the European Union are subject to significant regulatory controls.

The Clinical Trials Directive 2001/20/EC, the Directive 2005/28/EC on GCP, and the related national implementing provisions of the individual EU Member States govern the system for the approval of clinical trials in the European Union. Under this system, an applicant must obtain prior approval from the competent national authority of the EU Member States in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial at a specific study site after the competent ethics committee has issued a favorable opinion. The clinical trial application must be accompanied by, among other documents, an IMPD (the Common Technical Document) with supporting information prescribed by Directive 2001/20/EC, Directive 2005/28/EC, where relevant the implementing national provisions of the individual EU Member States and further detailed in applicable guidance documents. All suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial have to be reported to the competent national authority and the Ethics Committee of the Member State where they occurred.

In April 2014, the new Clinical Trials Regulation, (EU) No 536/2014 (Clinical Trials Regulation) was adopted. The Regulation is anticipated to come into application in 2019. The Clinical Trials Regulation will be directly applicable in all the EU Member States, repealing the current Clinical Trials Directive 2001/20/EC. Conduct of all clinical trials performed in the European Union will continue to be bound by currently applicable provisions until the new Clinical Trials Regulation becomes applicable. The extent to which ongoing clinical trials will be governed by the Clinical Trials Regulation will depend on when the Clinical Trials Regulation becomes applicable and on the duration of the individual clinical trial. If a clinical trial continues for more than three years from the day on which the Clinical Trials Regulation becomes applicable the Clinical Trials Regulation will at that time begin to apply to the clinical trial.

The new Clinical Trials Regulation aims to simplify and streamline the approval of clinical trials in the European Union. The main characteristics of the regulation include: a streamlined application procedure via a single entry point, the “EU portal”; a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (Member States concerned). Part II is assessed separately by each Member State concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the Clinical Trials Regulation.

To obtain a marketing authorization of a drug in the European Union, we may submit marketing authorization applications, or MAA, either under the so-called centralized or national authorization procedures.

*Centralized Procedure*

The centralized procedure provides for the grant of a single marketing authorization following a favorable opinion by the European Medicines Agency, or EMA, that is valid in all EU member states, as well as Iceland, Liechtenstein and Norway. The centralized procedure is compulsory for medicines produced by specified biotechnological processes, products designated as orphan medicinal products, advanced-therapy medicines (such as gene-therapy, somatic cell-therapy or tissue-engineered medicines) and products with a new active substance indicated for the treatment of specified diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions and viral diseases. The centralized procedure is optional for products that represent a significant therapeutic, scientific or technical innovation, or whose authorization would be in the interest of public health. Under the centralized procedure the maximum timeframe for the evaluation of an MAA by the EMA is 210 days, excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the Committee of Medicinal Products for Human Use, or the CHMP. Accelerated assessment might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation. The timeframe for the evaluation of an MAA under the accelerated assessment procedure is of 150 days, excluding stop-clocks.

*National Authorization Procedures*

There are also two other possible routes to authorize medicinal products in several EU countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure:

- Decentralized procedure. Using the decentralized procedure, an applicant may apply for simultaneous authorization in more than one EU country of medicinal products that have not yet been authorized in any EU country and that do not fall within the mandatory scope of the centralized procedure.

- Mutual recognition procedure. In the mutual recognition procedure, a medicine is first authorized in one EU Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other EU countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

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

***European Union Regulatory Exclusivity***

In the European Union, new products authorized for marketing (i.e., reference products) qualify for eight years of data exclusivity and an additional two years of market exclusivity upon marketing authorization. The data exclusivity period prevents generic applicants from relying on the pre-clinical and clinical trial data contained in the dossier of the reference product when applying for a generic marketing authorization in the European Union during a period of eight years from the date on which the reference product was first authorized in the European Union. The market exclusivity period prevents a successful generic applicant from commercializing its product in the EU until ten years have elapsed from the initial authorization of the reference product in the EU. The ten-year market exclusivity period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

***European Union Orphan Designation and Exclusivity***

The criteria for designating an orphan medicinal product in the European Union, are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. The application for orphan designation must be submitted before the application for marketing authorization. The applicant will receive a fee reduction for the marketing authorization application if the orphan designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The ten-year market exclusivity in the European Union may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if:

- the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior;
- the applicant consents to a second orphan medicinal product application; or
- the applicant cannot supply enough orphan medicinal product.

***Rest of the World Regulation***

For other countries outside of the European Union and the United States, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from jurisdiction to jurisdiction. Additionally, the clinical trials must be conducted in accordance with cGCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

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

***Other Healthcare Laws***

In addition to FDA restrictions on the marketing of pharmaceutical products, other U.S., federal and state healthcare regulatory laws restrict business practices in the pharmaceutical industry. These laws include, but are not limited to, federal and state anti-kickback, false claims, data privacy and security and physician payment and drug pricing transparency laws.

The U.S. federal Anti-Kickback Statute prohibits, among other things, any person or entity from knowingly and willfully offering, paying, soliciting, receiving or providing any remuneration, directly or indirectly, overtly or covertly, to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any good, facility, item or service reimbursable, in whole or in part, under Medicare, Medicaid or other federal healthcare programs. The term “remuneration” has been broadly interpreted to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical and medical device

manufacturers on the one hand and prescribers, purchasers, formulary managers and beneficiaries on the other hand. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not meet the requirements of a statutory or regulatory exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the U.S. federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the statute has been violated. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Moreover, a claim including items or services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act. The majority of states also have anti-kickback laws, which establish similar prohibitions and in some cases may apply to items or services reimbursed by any third-party payor, including commercial insurers, or to self-pay patients.

The federal false claims and civil monetary penalties laws, including the civil False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false, fictitious or fraudulent claim for payment to, or approval by, the federal government, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. A claim includes "any request or demand" for money or property presented to the U.S. government. Actions under the civil False Claims Act may be brought by the Attorney General or as a *qui tam* action by a private individual in the name of the government. Violations of the civil False Claims Act can result in very significant monetary penalties and treble damages. Several biopharmaceutical, medical device and other healthcare companies have been prosecuted under these laws for, among other things, allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of products for unapproved (e.g., off-label), and thus non-covered, uses. In addition, the civil monetary penalties statute imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Many states also have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Violations of fraud and abuse laws, including federal and state anti-kickback and false claims laws, may be punishable by criminal and civil sanctions, including fines and civil monetary penalties, the possibility of exclusion from federal healthcare programs (including Medicare and Medicaid), disgorgement of profits and corporate integrity agreements, which impose, among other things, rigorous operational and monitoring requirements on companies. Similar sanctions and penalties, as well as imprisonment, also can be imposed upon executive officers and employees of such companies. Given the significant size of actual and potential settlements, it is expected that the government authorities will continue to devote substantial resources to investigating healthcare providers' and manufacturers' compliance with applicable fraud and abuse laws.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, created additional federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

In addition, there has been a recent trend of increased federal and state regulation of payments made to physicians and certain other healthcare providers. The Affordable Care Act imposed, among other things, new annual

reporting requirements through the Physician Payments Sunshine Act for covered manufacturers for certain payments and "transfers of value" provided to physicians and teaching hospitals (and additional categories of health care practitioners beginning with reports due on or after January 1, 2022), as well as ownership and investment interests held by physicians and their immediate family members. Failure to submit timely, accurately and completely the required information for all payments, transfers of value and ownership or investment interests may result in civil monetary penalties. Covered manufacturers must submit reports by the 90th day of each subsequent calendar year and the reported information is publicly made available on a searchable website. In addition, certain states require implementation of compliance programs and compliance with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, impose restrictions on marketing practices or require the tracking and reporting of marketing expenditures and pricing information as well as gifts, compensation and other remuneration or items of value provided to physicians and other healthcare professionals and entities.

We may also be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their respective implementing regulations, impose specified requirements relating to the privacy, security and transmission of individually identifiable health information held by covered entities and their business associates. Among other things, HITECH made HIPAAs security standards directly applicable to "business associates," defined as independent contractors or agents of covered entities that create, receive, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity, although it is unclear that we would be considered a "business associate" in the normal course of our business. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same requirements, thus complicating compliance efforts.

Similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, anti-fraud and abuse laws and implementation of corporate compliance programs and reporting of payments or other transfers of value to healthcare professionals, may apply to us to the extent that any of our product candidates, once approved, are sold in a country other than the United States.

Because of the breadth of these laws and the narrowness of their exceptions and safe harbors, it is possible that business activities can be subject to challenge under one or more of such laws. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry.

Ensuring that business arrangements with third parties comply with applicable healthcare laws and regulations is costly and time consuming. If business operations are found to be in violation of any of the laws described above or any other applicable governmental regulations a pharmaceutical manufacturer may be subject to penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement of profits, individual imprisonment, exclusion from governmental funded healthcare programs, such as Medicare and Medicaid, contractual damages, reputational harm, diminished profits and future earnings, additional reporting obligations and oversight if subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and curtailment or restructuring of operations, any of which could adversely affect a pharmaceutical manufacturer's ability to operate its business and the results of its operations.

***Coverage, Pricing and Reimbursement***

Significant uncertainty exists as to the coverage and reimbursement status of any biological products for which we obtain regulatory approval. The United States government, state legislatures and foreign governments 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 requirements for substitution of generic products for branded

drug and biologic products. In the United States and markets in other countries, patients who are prescribed products generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Providers and patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products. If approved, sales of our product candidates will depend, in part, on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities, managed care plans, private health insurers and other organizations.

In the United States, the process for determining whether a third-party payor will provide coverage for a biological product typically is separate from the process for setting the price of such product or for establishing the reimbursement rate that the payor will pay for the product once coverage is approved. With respect to biologics, third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication, or place products at certain formulary levels that result in lower reimbursement levels and higher cost-sharing obligation imposed on patients. A decision by a third-party payor not to cover our product candidates could reduce physician utilization of a product. Moreover, a third-party payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable a manufacturer to maintain price levels sufficient to realize an appropriate return on its investment in product development. Additionally, coverage and reimbursement for products can differ significantly from payor to payor. One third-party payor's decision to cover a particular medical product does not ensure that other payors will also provide coverage for the medical product, or will provide coverage at an adequate reimbursement rate. As a result, the coverage determination process usually requires manufacturers to provide scientific and clinical support for the use of their products to each payor separately and is a time-consuming process.

As noted above, the marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide coverage and adequate reimbursement. An increasing emphasis on cost containment measures in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. 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. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of pharmaceutical products, in addition to questioning safety and efficacy. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover that product after FDA approval or, if they do, the level of payment may not be sufficient to allow a manufacturer to sell its product at a profit.

In addition, in many foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. In the European Union, governments influence the price of products through their pricing and reimbursement rules and control of national healthcare systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed to by the government. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. The downward pressure on healthcare costs in general, particularly prescription products, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross border imports from low-priced markets exert a commercial pressure on pricing within a country.

***Healthcare Reform and Potential Changes to Healthcare Laws***

The FDAs and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, in December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to

modernize the regulation of drugs and devices and to spur innovation, but its ultimate implementation is uncertain. In addition, in August 2017, the FDA Reauthorization Act was signed into law, which reauthorized the FDAs user fee programs and included additional drug and device provisions that build on the Cures Act. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we otherwise may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products and services, implementing reductions in Medicare and other healthcare funding and applying new payment methodologies. For example, in March 2010, the Affordable Care Act was enacted, which, among other things, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care plans; imposed mandatory discounts for certain Medicare Part D beneficiaries as a condition for manufacturers' outpatient drugs coverage under Medicare Part D; subjected drug manufacturers to new annual fees based on pharmaceutical companies' share of sales to federal healthcare programs; imposed a new federal excise tax on the sale of certain medical devices; created a new Patient Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; created the Independent Payment Advisory Board, which, once empaneled, will have authority to recommend certain changes to the Medicare program that could result in reduced payments for prescription drugs; and established a Center for Medicare Innovation at the CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

Since its enactment, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to the Affordable Care Act in the future. The current Presidential administration and members of the U.S. Congress have indicated that they may continue to seek to modify, repeal or otherwise invalidate all, or certain provisions of, the Affordable Care Act. Most recently, the Tax Cuts and Jobs Acts was enacted, which, among other things, removes penalties for not complying with the individual mandate to carry health insurance. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the Affordable Care Act, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the Affordable Care Act, such as the BPCIA, are invalid as well. While the presidential administration and CMS have both stated that the ruling will have no immediate effect, it is unclear how this decision, subsequent appeals, if any, and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business.

In addition, other legislative changes have been proposed and adopted in the United States since the Affordable Care Act to reduce healthcare expenditures. These changes include the Budget Control Act of 2011, which, among other things, included aggregate reductions of Medicare payments to providers of 2% per fiscal year and that will remain in effect through 2027 unless additional action is taken by Congress; and the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. More recently, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for pharmaceutical and biologic products.

Individual states in the United States have become increasingly active in passing legislation and implementing regulations designed to control biotechnology and pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services.

**Employees**

As of December 31, 2018, we had 111 employees.

**Our Corporate Information**

We are an exempted company incorporated under the laws of Bermuda in July 2015. Our registered office is located in Bermuda at Clarendon House, 2 Church Street, Hamilton HM11, Bermuda. The telephone number for our registered office is +44 808-189-6257. Our website address is www.kiniksa.com. The information contained on our website is not incorporated by reference into this Annual Report, and you should not consider any information contained on, or that can be accessed through, our website as part of this Annual Report.

**Where You Can Find More Information**

We are subject to the information requirements of the Securities Exchange Act of 1934, as amended. The SEC maintains an internet site that contains reports, proxy and information statements, and other information regarding issuers that file electronically, such as ourselves, with the SEC at *http://www.sec.gov*.

We make available free of charge on our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably possible after we electronically file such material with, or furnish it to, the SEC. Our website is located at *www.kiniksa.com*. The reference to our website address does not constitute incorporation by reference of the information contained at or available through our website, and you should not consider it to be a part of this Annual Report.

## ITEM 1A. RISK FACTORS.

*You should carefully consider the risks described below, as well as the other information in this Annual Report, including our consolidated financial statements and the related notes and "Management's discussion and analysis of financial condition and results of operations." The occurrence of any of the events or developments described below could adversely affect our business, financial condition, results of operations and growth prospects. In such an event, the market price of our Class A common shares could decline. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.*

### Risks Related to Our Financial Position and Capital Needs

***We are a biopharmaceutical company with a limited operating history and have not generated any revenue from product sales. We have incurred significant operating losses since our inception and anticipate that we will incur continued losses for the foreseeable future.***

We have incurred losses in each year since our inception in 2015 and anticipate incurring losses for the foreseeable future. To date, we have invested substantially all of our efforts and financial resources in identifying, acquiring, in-licensing and developing our product candidates, including commencing and conducting clinical trials and providing general and administrative support for these operations. Our future success is dependent on our ability to develop, obtain regulatory approval for and successfully commercialize one or more of our product candidates. We have not yet demonstrated our ability to successfully conduct and complete any Phase 3 or other pivotal clinical trials, obtain regulatory approvals, manufacture a commercial-scale drug, or conduct sales and marketing activities. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a marketable product. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree

of risk. Typically, it takes many years to develop one new drug from the time it is discovered to when it is available for treating patients, and development may cease for a number of reasons. Consequently, predictions about our future success or viability could be more accurate if we had a longer operating history.

We have incurred significant losses related to expenses for research and development and our ongoing operations. Our net losses for the years ended December 31, 2018 and 2017 were $103.2 million and $64.9 million, respectively. As of December 31, 2018, we had an accumulated deficit of $194.2 million. We expect to continue to incur losses for the foreseeable future, and we anticipate these losses will increase substantially as we:

- continue our research and pre-clinical or clinical development of our product candidates, including our single, pivotal, global, Phase 3 clinical trial for rilonacept for the treatment of recurrent pericarditis, named RHAPSODY, our ongoing open-label Phase 2 proof-of-concept clinical trial for rilonacept in recurrent pericarditis, our global Phase 2 proof-of-concept clinical trial with mavrilimumab for the treatment of GCA and the repeated single-dose cohort portion of our ongoing Phase 1b clinical trial of KPL-716 in subjects with atopic dermatitis;

- advance the development of our programs, including our plans for advancing KPL-716 into multiple chronic pruritic diseases, with an adaptive design Phase 2a/2b clinical trial with KPL-716 in prurigo nodularis as well as a Phase 2 exploratory pilot study in five other diseases characterized by chronic pruritus;

- initiate additional pre-clinical studies and clinical trials for our product candidates;

- increase our manufacturing needs or add additional manufacturers or suppliers;

- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;

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

- seek to identify, assess and study new or expanded indications for our product candidates, new or alternative dosing levels and frequency for our product candidates, or new or alternative administration of our product candidates, including method, mode or delivery device;

- seek to identify, assess, acquire or develop additional product candidates;

- make milestone or other payments under any license or purchase agreements;

- seek to maintain, protect and expand our intellectual property portfolio;

- seek to attract and retain skilled personnel;

- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and

- experience any delays or encounter issues with any of the above, including but not limited to failed trials, complex results, safety issues, other regulatory challenges that require longer follow-up of existing trials, additional major trials or additional supportive trials in order to pursue marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. We will also continue to incur additional costs associated with operating as a public company. Even if we achieve

profitability in the future, we may not be able to sustain profitability in subsequent periods. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our shareholders' equity and working capital.

***We will require substantial additional financing, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development, other operations or commercialization efforts.***

The development and commercialization of biopharmaceutical products is capital intensive. We are advancing our product candidates through pre-clinical and clinical development, including our multiple ongoing and planned global clinical trials for our product candidates, rilonacept, mavrilimumab and KPL-716. We expect our expenses to increase in connection with our ongoing activities as we continue the research and development of, and, if successful, seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to manufacturing, product sales, marketing and distribution. As our product candidates progress through development and towards commercialization, we will need to make milestone payments and if successful, eventually royalty payments, to the licensors and other third parties from whom we have acquired our product candidates. We may also need to raise additional funds sooner if we choose to pursue additional indications for our product candidates or otherwise expand more rapidly than we presently anticipate. Furthermore, we expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed on attractive terms, if at all, we will be forced to delay, reduce or eliminate certain of our clinical development plans, research and development programs or future commercialization efforts.

The development process for our product candidates is highly uncertain, and we cannot estimate with certainty the actual amounts necessary to successfully complete the development, regulatory approval process and commercialization of our product candidates. Our operating plans may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than expected, through public or private equity, debt financings or other sources. Our future capital requirements will depend on and could increase significantly as a result of many factors, including:

- the results, time and cost necessary for completing our single, pivotal, global, Phase 3 clinical trial for rilonacept for the treatment of recurrent pericarditis, named RHAPSODY, our ongoing open-label Phase 2 proof-of-concept clinical trial for rilonacept in recurrent pericarditis, our global Phase 2 proof-of-concept clinical trial with mavrilimumab for the treatment of GCA, and the repeated single-dose cohort portion of our ongoing Phase 1b clinical trial of KPL-716 in subjects with atopic dermatitis, and our plans for advancing KPL-716 into multiple chronic pruritic diseases, with an adaptive design Phase 2a/2b clinical trial with KPL-716 in prurigo nodularis as well as a Phase 2 exploratory pilot study in five other diseases characterized by chronic pruritus;

- the number, size and type of pre-clinical activities and any additional clinical trials;

- the costs, timing and outcomes of seeking and potentially obtaining approvals from the FDA or comparable foreign regulatory authorities, including the potential for the FDA or comparable regulatory authorities to require that we conduct more studies than those that we currently expect to conduct and the costs of post-marketing studies or risk evaluation and mitigation strategies, or REMS, that could be required by regulatory authorities;

- the costs and timing of transferring manufacturing technology to third-party manufacturers, producing product candidates to support clinical trials and preparing to manufacture mavrilimumab and KPL-716 on a commercial scale, as well as producing rilonacept in potential new final form configurations;

- the timing and amount of milestone and other payments we must make under our agreements with Regeneron, MedImmune, Biogen MA Inc., or Biogen, Novo Nordisk A/S, or Novo Nordisk, and the other

third parties from whom we have acquired or in-licensed our product candidates or from whom we may in the future acquire or in-license product candidates;

- our ability to successfully commercialize any of our product candidates, including the cost and timing of forming and expanding our sales organization and marketing capabilities;
- the amount of sales revenues from our product candidates, if approved, including the sales price and the availability of coverage and adequate third-party reimbursement;
- competitive and potentially competitive products and technologies and patients' receptivity to our product candidates and the technology underlying them in light of competitive products and technologies;
- the cash requirements of any future acquisitions, developments or discovery of additional product candidates, including any licensing, acquisition, collaboration or other strategic transaction agreements;
- the cash requirements for seeking to identify, assess and study new or expanded indications for our product candidates, new or alternative dosing levels or frequency for our product candidates, or new or alternative administration of our product candidates, including method, mode or delivery device;
- the time and cost necessary to respond to technological and market developments;
- the costs of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- any product liability or other lawsuits related to our product candidates or any products;
- the costs associated with being a public company;
- our need and ability to hire additional personnel; and
- the receptivity of the capital markets to financings by biotechnology companies generally and companies with product candidates and technologies such as ours specifically.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Disruptions in the financial markets may make equity and debt financing more difficult to obtain, and may have a material adverse effect on our ability to meet our fundraising needs when they arise. Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our pre-clinical studies, clinical trials or other research or development programs, or the commercialization of any product candidate. We may also be unable to expand our operations or otherwise capitalize on our business opportunities or may be required to relinquish rights to our product candidates or products. Any of these occurrences could materially affect our business, financial condition and results of operations.

***Raising additional capital may cause dilution to our shareholders, restrict our operations or require us to relinquish rights to our technologies or product candidates***.

Until such time as we can generate substantial product revenues, if ever, we expect to finance our cash needs through securities offerings or debt financings, or possibly, license and collaboration agreements or research grants. The terms of any financing may adversely affect the holdings or the rights of our shareholders and our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our Class A common shares to decline. The sale of additional equity or convertible securities would dilute all of our shareholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to

acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies, product candidates or future revenue streams, or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects. If we raise funds through research grants, we may be subject to certain requirements, which may limit our ability to use the funds or require us to share information from our research and development. Raising additional capital through any of these or other means could adversely affect our business and the holdings or rights of our shareholders, and may cause the market price of our Class A common shares to decline.

## Risks Related to Product Development and Regulatory Approval

***We depend heavily on the success of rilonacept, mavrilimumab and KPL-716, which are in various stages of clinical development. If we are unable to advance our product candidates in clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.***

We do not currently generate any revenue from sales of any products, and we may never be able to develop or commercialize marketable products. We have three product candidates in various stages of clinical development and two at the pre-clinical development stage. We may not be able to demonstrate that they are safe or effective in the indications for which we are studying them and they may not be approved. Although rilonacept is approved and marketed for human use for the treatment of CAPS, in the United States by Regeneron, we are studying rilonacept for the treatment of a different indication called recurrent pericarditis, which is currently in an ongoing Phase 2 proof-of-concept clinical trial for recurrent pericarditis, and we are enrolling subjects for a single, pivotal, global, Phase 3 clinical trial for rilonacept for the treatment of recurrent pericarditis, named RHAPSODY. Mavrilimumab has been through Phase 2 clinical trials conducted by MedImmune for the treatment of RA, but our global Phase 2 proof-of-concept clinical trial with mavrilimumab is for the treatment of GCA. Our third clinical-stage product candidate, KPL-716, is currently in the repeated-single-dose cohort portion of our ongoing Phase 1b clinical trial in subjects with atopic dermatitis, and we plan to advance KPL-716 into multiple chronic pruritic diseases, with an adaptive design Phase 2a/2b clinical trial with KPL-716 in prurigo nodularis as well as a Phase 2 exploratory pilot study in five other diseases characterized by chronic pruritus. We also have pre-clinical product candidates that would need to progress through studies to enable an IND prior to clinical development. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities. Our assumptions about why these product candidates are worthy of future development and potential approval in these, or any, indications are based on indirect data primarily collected by other companies and, as applicable, our pre-clinical or clinical trials.

We have not submitted, and we may never submit marketing applications to the FDA or comparable foreign regulatory authorities for our product candidates. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for one or more of our product candidates, we may not be able to continue our operations.

Each of our product candidates require additional pre-clinical or clinical development, regulatory approval in one or more jurisdictions, obtaining manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we are able to generate any revenue from product sales. The success of our product candidates will depend on several factors, including the following:

- successful completion of pre-clinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, conducted, where applicable, under the FDA's Good Laboratory Practice, or GLP, regulations;

- submission to the FDA of INDs and of clinical trial applications to foreign governmental authorities, for our product candidates to commence planned clinical trials or future clinical trials;

- successful site activation for and enrollment in, and completion of, clinical trials, the design and implementation of which are agreed to by the applicable regulatory authorities, and the ability of our contract research organizations, or CROs, to successfully conduct such trials within our planned budget and timing parameters and without materially adversely impacting our trials;

- successful data from our clinical programs that support an acceptable risk-benefit profile of our product candidates for the targeted indications in the intended populations to the satisfaction of the applicable regulatory authorities;

- timely receipt, if at all, of regulatory approvals from applicable regulatory authorities;

- establishment of arrangements with third-party manufacturers, as applicable, for continued clinical supply and commercial manufacturing;

- successful development of our manufacturing processes and transfer to new third-party facilities to support future development activities and commercialization that are operated by contract manufacturing organizations, or CMOs, in a manner compliant with all regulatory requirements;

- sufficient supply of our product candidates from our CMOs;

- establishment and maintenance of patent and trade secret protection or regulatory exclusivity for our product candidates;

- successful commercial launch of our product candidates, if and when approved;

- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;

- effective competition with other therapies;

- establishment and maintenance of adequate healthcare coverage and reimbursement;

- enforcement and defense of intellectual property rights and claims;

- continued compliance with any post-marketing requirements imposed by regulatory authorities, including any required post-marketing clinical trials, or REMS; and

- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not accomplish one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for one or more of our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to manufacture and market our product candidates, our revenues will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

We plan to seek regulatory approval to commercialize our product candidates in the United States and potentially in foreign countries. While the scope of regulatory approval is similar in many countries, to obtain separate regulatory approval in multiple countries will require us to comply with numerous and varying regulatory requirements of each such country or jurisdiction regarding safety and efficacy and governing, among other things, clinical trials and commercial sales, pricing and distribution, and we cannot predict success in any such jurisdictions.

***Clinical drug development is a lengthy and expensive process with uncertain timelines and uncertain outcomes. We may encounter substantial delays in our clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities. We may therefore be unable to obtain required regulatory approvals and be unable to commercialize our product candidates on a timely basis, if at all.***

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all.

We are enrolling a single, pivotal, placebo-controlled, RW design, global Phase 3 clinical trial of rilonacept in subjects with symptomatic recurrent pericarditis, continuing our ongoing open-label Phase 2 proof-of-concept clinical trial for rilonacept for the treatment of recurrent pericarditis, for which we have completed enrollment, and enrolling the repeated single-dose cohort portion of our ongoing Phase 1b clinical trial of KPL-716 in subjects with atopic dermatitis. We commenced dosing in multiple countries in a global Phase 2 proof-of-concept clinical trial with mavrilimumab for the treatment of GCA. We plan to advance KPL-716 into multiple chronic pruritic diseases, with an adaptive design Phase 2a/2b clinical trial in prurigo nodularis as well as a Phase 2 exploratory pilot study in five other diseases characterized by chronic pruritus. We are also continuing our pre-clinical activities with KPL-404 prior to initiating clinical trials and are evaluating the progression of KPL-045 pending preclinical data from the program in the context of our portfolio. Commencing our planned clinical trials is subject to acceptance by the FDA of an IND or an IND amendment, acceptance by European regulatory authorities of a Clinical Trial Application, or acceptance by other applicable regulatory authorities, and finalizing the trial design based on discussions with the FDA, European regulatory authorities or other applicable regulatory authorities.

Even after we receive and incorporate guidance from these regulatory authorities, such regulatory authorities could disagree that we have satisfied their requirements to commence our clinical trials, disagree with our interpretation of data from the relevant pre-clinical studies, clinical trials or chemistry, manufacturing and controls, or CMC, data, or disagree or change their position on the acceptability of our trial designs including the proposed dosing schedule, our definitions of the patient populations or the clinical endpoints selected, which may require us to complete additional pre-clinical studies, clinical trials, CMC development, other studies or impose stricter approval conditions than we currently expect. For example, prior to us licensing mavrilimumab, MedImmune submitted an IND to the FDA to conduct a clinical trial of mavrilimumab in RA, and the FDA issued a clinical hold based on its review of certain effects in the lungs observed in non-human primates in pre-clinical toxicity studies. However, following subsequent discussions between MedImmune and the FDA regarding the clinical hold and the availability of additional clinical safety data that MedImmune generated in human clinical trials conducted outside of the United States subsequent to the original IND submission, the FDA acknowledged that the risk/benefit assessment for investigation of mavrilimumab in a clinical trial may differ depending on the patient population studied. Specifically, the FDA acknowledged that the risk/benefit assessment for initiation of a clinical trial may be considered favorable in a patient population with high morbidity and limited effective treatment options, including refractory RA. As a result, we believed that the FDA's communications with MedImmune suggested that the FDA could find an acceptable risk/benefit for a clinical trial of mavrilimumab in the United States in GCA, a disease with high morbidity and limited treatment options. MedImmune has since withdrawn the IND for mavrilimumab for the treatment of RA. We completed our pre-IND meeting with the FDA and filed an IND for our Phase 2 proof-of-concept clinical trial of mavrilimumab in GCA. The FDA initially placed our IND on clinical hold in the United States due to its request for additional information on the 510(K)-cleared delivery device to be used in our trial. We have since provided the FDA with the requested information and our IND is now active.

Further, we could discover that our clinical trial design leads to enrollment difficulties which could require protocol amendments and further delay our study. In addition, the FDA or other regulatory authorities could require us to collect additional clinical data. For example, we anticipate that to help inform the benefit-risk profile for the use of mavrilimumab in GCA, we will need to demonstrate the effectiveness and safety of mavrilimumab after 26 weeks in our ongoing Phase 2 clinical trial, as well as evaluate its pharmacokinetic profile and the effectiveness of mavrilimumab at different doses. Successful completion of our clinical trials is a prerequisite to submitting a biologics license application, or BLA, to the FDA and a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, for each product candidate and, consequently, to obtaining approval and initiating commercial marketing of our current

and future product candidates. We do not know whether any of our clinical trials will begin or be completed on schedule, if at all.

A failure of one or more clinical trials can occur at any stage of testing, and our future clinical trials may not be successful. We may experience delays in our ongoing clinical trials and we do not know whether planned clinical trials will begin on time, will be allowed by regulatory authorities, need to be redesigned, or if we can activate sites or enroll patients on time or if they will be completed on schedule, if at all. Events that may prevent successful or timely completion of clinical development include but are not limited to:

- inability to generate sufficient pre-clinical, toxicology or other *in vivo* or *in vitro* data to support the initiation of human clinical trials;
- delays in reaching a consensus with regulatory agencies on trial design or implementation;
- delays in establishing the appropriate dosage levels or frequency of dosing or treatment in clinical trials;
- delays in reaching, or failing to reach, agreement on acceptable terms with prospective CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- difficulties in obtaining required IRB approval at each clinical trial site;
- delays in or failure to obtain regulatory approval to commence a trial, or imposition of a clinical hold by regulatory agencies, after review of an IND or IND amendment, or equivalent application or amendment, or an inspection of our clinical trial operations or study sites;
- challenges in recruiting and enrolling suitable patients to participate in our clinical trials;
- amendments to protocols amending study criteria and design;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties, or us to adhere to clinical trial requirements or to perform their obligations in a timely or compliant manner;
- failure to perform in accordance with the FDA's good clinical practices requirements, or GCPs, or applicable regulatory guidelines in other countries;
- patients not completing participation in a clinical trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a clinical trial;
- participating patients experiencing serious adverse events or undesirable side effects or are exposed to unacceptable health risks;
- safety issues, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- difficulty in identifying the populations that we are trying to enroll in a particular trial, which may delay enrollment and reduce the power of a clinical trial to detect statistically significant results;

- changes in regulatory requirements, policies and guidance that require amending or submitting new clinical protocols;

- the cost of clinical trials of our product candidates being greater than we anticipate;

- clinical trials of our product candidates producing negative or inconclusive results, which may result in us deciding, or regulators requiring us, to conduct additional clinical trials or abandon drug development programs; and

- delays in manufacturing, testing, releasing, validating or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing.

We could encounter delays if a clinical trial is rejected, suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, the Data Safety Monitoring Board for such trial or the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, 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 issues or adverse side effects that arise in the trial, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. 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.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or other regulatory authorities. The FDA or other regulatory authorities may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of a study. The FDA or other regulatory authorities may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authorities, as the case may be, and may ultimately lead to the denial of marketing approval of our product candidates.

If we experience delays in the completion of any clinical trial of our product candidates or any clinical trial of our product candidates is terminated, the commercial prospects of our product candidates may be harmed, and our ability to generate product revenues from our product candidates, if any, will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down the development and approval process of our product candidates and jeopardize our ability to commence product sales and generate revenue, if any. Significant clinical trial delays could also allow our competitors to bring products to market before we do or shorten any periods during which we have the exclusive right to commercialize our product candidates and could impair our ability to commercialize our product candidates. 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.

Clinical trials must be conducted in accordance with the laws and regulations of the FDA, European Union rules and regulations and other applicable regulatory authorities' legal requirements, regulations or guidelines, and are subject to oversight by these governmental agencies and IRBs at the medical institutions where the clinical trials are conducted. Further, conducting global clinical trials may require that we coordinate among the requirements, regulations or guidelines of regulatory authorities across a number of jurisdictions, including the United States, European Union and countries outside of those jurisdictions, which could require that we amend trial protocols or determine not to conduct a trial in one or more jurisdictions or to run separate trials in various jurisdictions due to the inability, cost or delay in harmonizing divergent requests from such regulatory authorities, all of which could increase costs. In addition, clinical trials must be conducted with supplies of our product candidates produced under current good manufacturing practice, or cGMP, requirements and other regulations. Furthermore, we rely on CROs and 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 depend on our collaborators and on medical institutions and CROs

to conduct our clinical trials in compliance with GCP requirements. To the extent our collaborators or the CROs fail to enroll participants for our clinical trials, fail to conduct the study to GCP standards or are delayed for a significant time in the execution of trials, including achieving full enrollment, we may be affected by increased costs, program delays or both. In addition, clinical trials that are conducted in countries outside the European Union and the United States may subject us to further delays and expenses as a result of increased shipment costs, additional regulatory requirements and the engagement of non-EU and non-U.S. CROs, as well as expose us to risks associated with clinical investigators who are unknown to the FDA or the EMA, and different standards of diagnosis, screening and medical care.

Further, conducting clinical trials in foreign countries, as we do for certain of our product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

We must produce, through third parties, sufficient stable quantities of our product candidates for use in our clinical trials. Any delays in the production of our product candidates may lead to a delay in our clinical trials. If we make manufacturing or formulation changes to our product candidates or change manufacturers or manufacturing processes, we may be unsuccessful in producing the product as compared to the process or manufacturer used in prior clinical trials, and therefore may need to conduct additional trials to bridge our modified product candidates to earlier versions, which could impact the timing of commencing or completing our clinical trials. Moreover, there is no assurance that future clinical trials utilizing a new formulation of a product candidate manufactured by different manufacturers or pursuant to a new process will result in the favorable result, if any, observed in the prior clinical trials of such product candidates. For example, we will need to produce mavrilimumab using different media and feed compared to the processes that were used by MedImmune to develop our existing inventory. Further, we transferred the manufacturing process of mavrilimumab to a third party to manufacture mavrilimumab for any Phase 3 clinical trials and commercialization efforts, if any. This manufacturer may be unsuccessful in producing the product in quantities or quality necessary to support our clinical trials or commercialization efforts, if any, which would delay development of mavrilimumab. In addition, we built small scale manufacturing capabilities to support certain pre-clinical and early clinical development for our product candidates. We may not be able to produce sufficient quantities of these product candidates or produce them at an acceptable quality, which could delay, prevent or impair our development or commercialization efforts.

Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to obtain orphan exclusivity for our products that potentially qualify for this designation, and to successfully commercialize our product candidates and may harm our business and results of operations. Any inability to successfully complete pre-clinical and clinical development could result in additional costs to us or impair our ability to generate revenue and harm our business, financial condition and prospects significantly.

***We may find it difficult to enroll patients in our clinical trials in a timely manner given the limited number of patients who have the diseases for which our product candidates are being studied, as well as particular enrollment criteria. Difficulty in enrolling patients could delay or prevent clinical trials of our product candidates, and our research and development efforts could be adversely affected.***

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends in part on the speed at which we can recruit patients to participate in testing our product candidates, and we may experience delays in our clinical trials if we encounter difficulties in enrollment. Patient enrollment depends on many factors, including the size and nature of the patient population, eligibility criteria for the clinical trial, the proximity of patients to clinical sites, the design of the clinical protocol, the availability of competing clinical trials, the availability of new drugs approved for the indication the clinical trial is investigating, the risk that patients enrolled in clinical trials will drop out of the trials before completion of their treatment and clinicians' and patients' perceptions as to the safety and potential advantages of the product candidate being studied in relation to other available therapies.

Many of the conditions for which we plan to evaluate our current product candidates in the near future are in small disease populations. Accordingly, there are limited patient pools from which to draw for clinical trials.

In addition to the rarity of these diseases, the eligibility criteria of our clinical trials will further limit the pool of available trial participants, as we will require patients to have specific characteristics that we can measure or to ensure their disease is either severe enough or not too advanced to include them in a trial. Further, we could learn that our clinical trial design increased the difficulty of enrolling patients, which could delay our trials. The process of finding and diagnosing patients may prove costly, especially since the rare diseases we are studying are commonly under diagnosed. We also may not be able to identify, recruit, enroll and retain a sufficient number of patients to complete our clinical trials because of the perceived risks and benefits of the product candidate under trial, the proximity and availability of clinical trial sites for prospective patients and the patient referral practices of physicians. The availability and efficacy of competing therapies and clinical trials, and clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to those available competing therapies and clinical trials, can also adversely impact enrollment. If patients are unwilling to participate in our trials for any reason, the timeline for recruiting patients, conducting trials and obtaining regulatory approval of potential products may be delayed, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. Moreover, failure to obtain and maintain patient consents can also lead to delay or prevent completion of clinical trials of our product candidates.

In addition, our clinical trials may compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition may further reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we may conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site. Delays in patient enrollment will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue. 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.

***Our product candidates may cause undesirable side effects or have other safety risks that could delay or prevent their regulatory approval, limit the commercial profile of an approved label or result in significant negative consequences, including withdrawal of approval, following any potential marketing approval.***

Treatment with our product candidates may produce undesirable side effects or adverse reactions or events. Undesirable 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.

All of our product candidates modulate the immune system and carry risks associated with immunosuppression, including the risk of serious infections and cancer. Some common side effects of rilonacept include, cold symptoms, nausea, stomach pain, diarrhea, numbness or tingly feeling and injection-site reaction. IL-1 blockade may interfere with immune response to infections. Serious, life-threatening infections have been reported in patients taking rilonacept. In our ongoing Phase 2 proof-of-concept clinical trial of rilonacept for recurrent pericarditis, the most common AEs were gastrointestinal disorders and injection site reactions. There was one treatment-related serious AE which resulted in discontinuation: a skin abscess which responded to medical treatment.

For mavrilimumab, there is a theoretical risk for the development of PAP. PAP is a rare lung disorder in which surfactant-derived lipoproteins accumulate excessively within pulmonary alveoli due to loss of GM-CSF function. The disease can range in severity from a sub-clinical reduction in diffusion capacity to significant dyspnea during mild exertion. In pre-clinical studies conducted by MedImmune, certain effects were observed in the lungs of non-human primates, which led the FDA to issue a clinical hold with respect to MedImmune's proposed clinical trial in RA. Pre-clinical data generated to date suggest mavrilimumab does not reach the lungs in sufficient quantities to induce PAP at clinically relevant doses and human trials thus far have not shown a clinical effect on pulmonary function tests

attributable to mavrilimumab. However, if the results of our clinical trials reveal a high or unacceptable severity and prevalence of these or other side effects, the FDA or applicable foreign regulatory agency may suspend or terminate our clinical trials that are initiated, not authorize us to initiate further trials, or if initiated, such further trials could be suspended or terminated. The FDA or comparable foreign regulatory authorities could order us to cease further development of, or deny or withdraw approval of, any of our product candidates for any or all targeted indications.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such product, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may withdraw approvals of such product and require us to take it off the market;
- regulatory authorities may require the addition of labeling statements, specific warnings, contraindications or field alerts to physicians and pharmacies;
- we may be required to create a registry or a REMS plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers or other elements to assure safe use;
- we may be required to change the way the product is administered, conduct additional clinical trials or change the labeling of the product;
- we may be subject to limitations on how we promote the product, or sales of the product may decrease significantly;
- we could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

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.

***Prior to our in-license or acquisition of rilonacept, mavrilimumab, KPL-716, KPL-404, and KPL-045, we were not involved in the development of these product candidates and, as a result, we are dependent on Regeneron, MedImmune, Biogen, Primatope and Novo Nordisk having accurately reported the results and correctly collected and interpreted the data from all pre-clinical and clinical trials conducted prior to our in-license or acquisition.***

We had no involvement with or control over the pre-clinical and clinical development of any of our product candidates prior to our in-license or acquisition of them. We are dependent on Regeneron, MedImmune, Biogen, Primatope and Novo Nordisk having conducted their research and development in accordance with the applicable protocols and legal, regulatory and scientific standards; having accurately reported the results of all pre-clinical studies and clinical trials conducted prior to our in-license or acquisition; and having correctly collected, interpreted, and completely transferred the data from these trials or other studies to us. If these activities were not compliant, accurate or correct, the clinical development, regulatory approval, or commercialization of one or more of our product candidates will be adversely affected.

***If we cannot replicate positive results from earlier pre-clinical studies and clinical trials conducted by us or the companies from whom we have licensed or acquired, or may in the future license or acquire, our product candidates in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.***

Positive results from our pre-clinical studies, and any positive results we may obtain from our early clinical trials of our product candidates or from the clinical trials conducted by the companies from whom we in-licensed or acquired or may in the future in-license or acquire our product candidates, may not necessarily be predictive of the results from any required later pre-clinical studies and clinical trials. Similarly, even if we are able to complete our planned pre-clinical studies or clinical trials of our product candidates, the positive results from the pre-clinical studies and clinical trials of our product candidates may not be replicated in our subsequent pre-clinical studies or clinical trial results. The safety and efficacy of our product candidates have not been established for the indications in which we are developing them, and we cannot provide any assurance that their development will be successful. For example, although rilonacept is FDA approved for the treatment of CAPS, and mavrilimumab has been studied in Phase 2 clinical trials for the treatment of RA, their safety and efficacy have not been determined in the indications we are pursuing, recurrent pericarditis or GCA, respectively, and each may fail to receive regulatory approval for those indications.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, pre-clinical findings made while clinical trials were underway or safety or efficacy observations made in pre-clinical studies and clinical trials, including previously unreported adverse events. Moreover, pre-clinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in pre-clinical studies and clinical trials nonetheless failed to obtain FDA or EMA approval. Furthermore, the approval policies or regulations of the FDA or the applicable foreign regulatory agencies may significantly change in a manner rendering our clinical data insufficient for approval, which may lead to the FDA or any foreign regulatory agencies delaying, limiting or denying approval of our product candidates.

***Interim, "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.***

From time to time, we may publish interim "top-line" or preliminary data from our clinical trials. For example, in December 2018, we released interim data from the open-label Phase 2 proof-of-concept clinical trial of rilonacept in recurrent pericarditis. Preliminary or interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

***Regulatory approval processes are lengthy, time consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, we will not be able to commercialize, or will be delayed in commercializing, our product candidates and our ability to generate revenue will be materially impaired.***

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Before we can commercialize any of our product candidates, we must obtain marketing approval. We have not received approval or clearance to market any of our product candidates from regulatory authorities in any jurisdiction and it is possible that none of our product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval or clearance. We have only limited experience in filing and supporting the applications necessary to gain regulatory approvals and may need to

rely on third-party CROs and regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive pre-clinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the biologic manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. The FDA and other regulatory authorities have substantial discretion in the approval process, and determining when or whether regulatory approval will be obtained for a product candidate. Even if we believe the data collected from clinical trials are promising, such data may not be sufficient to support approval by the FDA or any other regulatory authority.

The process of obtaining regulatory approvals, both in the United States and in other countries, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted BLA, or equivalent application types, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional pre-clinical, clinical or other trials. Approval by the FDA in the United States, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. Our product candidates could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the number, design or implementation of our clinical trials to support further development or approval;
- 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 pre-clinical studies or clinical trials;
- the FDA or comparable foreign regulatory authorities may disagree that we have provided sufficient safety data or adequately demonstrated clinical benefit in a patient population or subpopulation studied in the clinical trial;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authority could require us to collect additional data or conduct additional clinical studies, for example, based on FDA feedback, we anticipate that to help inform the benefit-risk profile for the use of mavrilimumab in GCA, we will need to demonstrate the effectiveness and safety of mavrilimumab after 26 weeks, as well as evaluate its pharmacokinetic profile and the effectiveness of mavrilimumab at different doses;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, participants may drop out of

these clinical trials at a higher rate than we anticipate or we may fail to recruit suitable patients to participate in a trial;

- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

- the FDA or comparable foreign regulatory authorities may not believe that we have sufficiently demonstrated our ability to manufacture the products to the requisite level of quality standards, or they may fail to approve our manufacturing processes or facilities, or the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies;

- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;

- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to reject, suspend or terminate the clinical trials; and

- 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.

In addition, even if we were to obtain approval for one or more of our product candidates, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request. For example, in connection with our KPL-716 program, regulatory authorities may recognize a narrower patient population as having prurigo nodularis or define the disease differently than we do. Furthermore, regulatory authorities may not approve the price we intend to charge, may grant approval contingent on the performance of costly post-marketing clinical trials, may impose certain post-marketing requirements that impose limits on our marketing and distribution activities, 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. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenue will be materially impaired.

***Our product candidates regulated as biologics in the United States may face competition sooner than anticipated.***

In the United States, the Biologics Price Competition and Innovation Act of 2009, or BPCIA, 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 pre-clinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when processes intended to implement the BPCIA may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects of our product candidates.

Rilonacept was approved as a biological product under a BLA for the treatment of CAPS in 2008, and we believe it should qualify for the 12-year period of exclusivity against any biosimilars. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider rilonacept, or any of our other product candidates, to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. In addition, we plan to submit a supplemental BLA for

rilonacept for the treatment of recurrent pericarditis, and the 12-year exclusivity period does not attach to the approval of a supplemental BLA.

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 a reference product 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.

***Even if we obtain marketing approval of our product candidates in a major pharmaceutical market such as the United States or the European Union, we may not obtain approval or commercialize our product candidates in other markets, which would limit our ability to realize their full market potential.***

In order to market any products in a country or territory, we must establish and comply with numerous and varying regulatory requirements of such country or territory regarding safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking regulatory approvals in all markets may require additional pre-clinical studies or clinical trials, which would be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates in those countries. Satisfying these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. In addition, our failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in other countries.

***We may seek orphan drug designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity, for any product candidate for which we obtain orphan drug designation.***

As part of our business strategy, we are pursuing orphan drug designation for certain of our product candidates, and we may be unsuccessful or unable to maintain the associated benefits. Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs or biologics intended to treat relatively small patient populations as orphan drug products. Under the U.S. Orphan Drug Act, the FDA may designate a drug or biologic as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States.

In the European Union, the European Commission grants orphan drug designation after receiving the opinion of the EMA's Committee for Orphan Medicinal Products on an Orphan Drug Designation application. In the European Union, orphan drug designation is intended to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, orphan drug designation is granted for drugs intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers, as well as potential marketing exclusivity.

In addition, if a drug or biologic with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug or biologic is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us, for products that constitute the "same drug" and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. The applicable period is seven years in the United States and ten years in the European Union. The European Union

exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect those product candidates from competition because different drugs can be approved for the same condition, and orphan drug exclusivity does not prevent the FDA from approving the same or a different drug in another indication. Even after an orphan drug is approved, the FDA can subsequently approve a later application for the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan-drug-exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. While we intend to pursue orphan drug designation for certain of our product candidates in addition to rilonacept, we may never receive such designation. Even if we do receive such designation, there is no guarantee that we will enjoy the benefits of such designation.

***We may seek Breakthrough Therapy designation or Fast Track designation by the FDA for one or more of our product candidates, but we may not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.***

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

If a product candidate is intended for the treatment of a serious or life-threatening condition and clinical or pre-clinical data demonstrate the potential to address unmet medical needs for this condition, the sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we have obtained Fast Track Designation for one or more of our product candidates, we may not experience a faster development process, review or approval compared to non-expedited FDA review procedures. In addition, the FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported. Fast Track Designation alone does not guarantee qualification for the FDA's priority review procedures.

Whether to grant Breakthrough Therapy or Fast Track Designation is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for these designations, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of either of these designations for a product candidate may not result in a faster development process, review or approval compared to product candidates considered for approval under non-expedited FDA review procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify for either of these designations, the FDA may later decide that the product candidate no longer meet the conditions for qualification.

***We have never completed a pivotal clinical trial or obtained marketing approval for any product candidate, and we may be unable to successfully do so for any of our product candidates. Failure to successfully complete any of these activities in a timely manner for any of our product candidates could have a material adverse impact on our business and financial performance.***

Conducting clinical trials and preparing, and obtaining marketing approval for, a product candidate is a complicated process. Although members of our management team have participated in pivotal trials and obtained marketing approvals for product candidates in the past while employed at other companies, we as a company have not done so. As a result, such activities may require more time and cost more than we anticipate. Failure to successfully complete, or delays in, any of our eventual pivotal trials or related regulatory submissions would prevent us from, or delay us in, obtaining regulatory approval for, or clearance of, our product candidates. In addition, it is possible that the FDA may refuse to accept for substantive review any BLA submissions that we submit for our product candidates or may conclude after review of our applications that they are insufficient to obtain marketing approval or clearance of our product candidates. If the FDA does not accept our applications or issue marketing authorizations for our product candidates, it may require that we conduct additional clinical, pre-clinical or manufacturing validation trials and submit that data before it will reconsider our applications. Depending on the extent of these or any other FDA-required trials, approval of any BLA or receipt of other marketing authorizations for any other applications that we submit may be delayed by several years, or may require us to expend more resources than we have available. It is also possible that additional trials, if performed and completed, may not be considered sufficient by the FDA to approve our BLAs or grant other marketing authorizations. Any delay in obtaining, or an inability to obtain, marketing approvals would prevent us from commercializing our product candidates, generating revenues and achieving and sustaining profitability. If any of these outcomes occur, we may be forced to abandon our development efforts for our product candidates, which could significantly harm our business.

***Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

**Risks Related to Manufacturing and Our Dependence on Third Parties**

***We contract with third parties for manufacturing our product candidates and for pre-clinical and clinical development and expect to continue to do so for our commercial supply. This reliance on third parties increases the risk that we may not have sufficient quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.***

We do not currently own or operate any large-scale manufacturing facilities. Although we have built small-scale manufacturing facilities to produce drug substance to support certain of our pre-clinical studies and certain of our Phase 1 clinical trials, we rely, and expect to continue to rely, on third parties for the manufacture of our product candidates for the majority of our development efforts, as well as for the commercial manufacture of our product candidates, if approved. We rely on these third parties to develop the processes necessary to produce our product candidates at sufficient quality and quantity to support our development and commercialization efforts. Our reliance

increases the risk that we will have insufficient quantities of our product candidates or that our product candidates are not produced at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

For example, we contract with Regeneron to produce rilonacept, and with CMOs for the manufacture of KPL-716 drug substance and drug product. Further, we have entered into an agreement with a CMO to produce mavrilimumab beyond our current inventory. While we have transferred the technology to manufacture mavrilimumab to the CMO, the CMO may be required to adopt different manufacturing protocols or processes. The CMO will need to produce mavrilimumab using different media and feed compared to the processes that were used by MedImmune to develop our existing inventory. We cannot provide any assurance that the technology transfer was successful, or that the process development or the CMO will be successful in producing mavrilimumab in sufficient quantities or of acceptable quality, if at all. In addition, we contract with CMOs in connection with certain production and testing of our pre-clinical product candidates, and while we have built small-scale manufacturing facilities to support certain pre-clinical and early clinical development for our product candidates, we and our CMOs may not be able to produce sufficient quantities of these product candidates or produce them at an acceptable quality, which could delay, prevent or impair our development or commercialization efforts and increase costs.

The facilities used by our CMOs to manufacture our product candidates may be inspected by the FDA and other comparable regulatory authorities in connection with the submission of our marketing applications to, and review by, the FDA or other comparable regulatory authorities. While we provide oversight of manufacturing activities, we do not and will not control the manufacturing process of, and will be completely dependent on, our CMOs for compliance with cGMPs and other regulatory requirements in connection with the manufacture of our product candidates. If our CMOs cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our CMOs to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Further, our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates, if approved, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect our business and supplies of our product candidates.

Although we have entered into certain agreements for the manufacture of clinical material for our product candidates, we may be unable to establish new agreements on acceptable terms, if at all, with third-party manufacturers for those product candidates. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

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

Our product candidates may compete with other product candidates and approved 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. Further, Regeneron has an exclusive right to produce rilonacept for a period of time.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. If our current CMOs cannot perform as agreed, we may be required to replace such manufacturers. Although we believe that there are several potential alternative manufacturers who could manufacture our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement.

Finding new CMOs or third-party suppliers involves additional cost and requires our management's time and focus. In addition, there is typically a transition period when a new CMO commences work. Although we generally do not begin a clinical trial unless we believe we have on hand, or will be able to obtain, a sufficient supply of our product candidates to complete the clinical trial, any significant delay in the supply of our product candidates or the raw materials needed to produce our product candidates, could considerably delay conducting our clinical trials and potential regulatory approval of our product candidates.

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

***We built our own small-scale manufacturing facilities to support the early development of our product candidates, and we may be unsuccessful in manufacturing product candidates in a timely, economic or compliant manner, which could delay or prevent the commencement of our planned clinical studies for these product candidates.***

We built small-scale manufacturing facilities to support pre-clinical and early clinical studies for our product candidates. We may not successfully establish sufficient manufacturing capabilities or manufacture our product candidates economically or in compliance with cGMPs and other regulatory requirements, or at all, and we may not be able to build or procure additional capacity in the required timeframe to meet our estimated timelines to commence our studies. As a result, our business, prospects, operating results, and financial condition could be materially harmed.

***Our business involves the use of hazardous materials and we and our third-party manufacturers and suppliers must comply with environmental laws and regulations, which can be expensive and restrict how we do business.***

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use and disposal of hazardous materials owned by us, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' and suppliers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers and suppliers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

***Manufacturing issues at our facilities and the facilities of our third-party service providers could cause product shortages, disrupt or delay our clinical trials or regulatory approvals, delay or stop commercialization of our product candidates, and adversely affect our business.***

The manufacture of our product candidates is highly regulated, complex and difficult, requiring a multi-step and controlled process, and even minor problems or deviations could result in the product candidates being out-of-spec, failed batches or other failures, such as defective products or manufacturing failures. We have limited experience

overseeing the manufacturing process of KPL-716 and our pre-clinical product candidates and no experience overseeing the manufacturing process of rilonacept and mavrilimumab. Due to the highly technical requirements of manufacturing our product candidates and the strict quality and control specifications, we and our third-party providers may be unable to manufacture or supply our product candidates despite our and their efforts. Failure to produce sufficient quantities of our product candidates could delay their development, result in supply shortages for our patients, result in lost revenue, if any, and diminish our potential profitability, which may lead to lawsuits or could delay the introduction of our product candidates to the market.

The manufacture of our product candidates is at high risk of product loss due to contamination, equipment malfunctions, human error or raw material shortages. Deviations from established manufacturing processes could result in reduced production yields, failed batches and other supply disruptions. If microbial, viral or other contaminations are discovered in our product candidates or manufacturing facilities, any related production lot could be lost and the relevant manufacturing facilities may need to close for an extended period of time to investigate and remediate the contaminant. Many additional factors could cause production interruptions at our facilities or at the facilities of our third-party providers, including natural disasters, accidents, labor disputes, acts of terrorism or war. The occurrence of any such event could adversely affect our ability to satisfy the required supply for any of our product candidates or successfully complete pre-clinical and clinical development, which would result in additional costs to us or impair our ability to generate revenue and would harm our business, financial condition and prospects significantly.

We and our third-party providers are required to maintain compliance with cGMP and other stringent requirements and are subject to inspections by the FDA and comparable agencies in other jurisdictions to confirm such compliance. Any delay, interruption or other issues that arise in the manufacture, fill-finish, packaging or storage of our product candidates as a result of a failure of the facilities or operations of third parties to pass any regulatory agency inspection could significantly impair our ability to supply our products and product candidates. Significant noncompliance could also result in the imposition of monetary penalties or other civil or criminal sanctions and damage our reputation.

Any adverse developments affecting the operations of our third-party providers could result in a product shortage of clinical or commercial requirements, withdrawal of our product candidates or any approved products, shipment delays, lot failures or recalls. We may also have to write off inventory and incur other charges and expenses for products that fail to meet specifications, undertake costly remediation efforts or seek more costly manufacturing alternatives. Such manufacturing issues could increase our cost of goods, cause us to lose potential revenue, reduce our potential profitability or damage our reputation.

***The third parties upon whom we rely for the supply of the drug substance and drug product used in our lead product candidates are our sole source of supply, and the loss of any of these suppliers could significantly harm our business.***

The drug substance and drug product used in rilonacept, mavrilimumab and KPL-716 are supplied to us from single-source suppliers. For example, although Regeneron has been producing rilonacept for over ten years, they have a contractual right to be our sole source manufacturer of the product, unless they have a persistent failure to satisfy our supply needs. Our ability to successfully develop our product candidates, and to ultimately supply our commercial products in quantities sufficient to meet the market demand, depends in part on our ability to obtain the drug substance and drug product for these product candidates in accordance with regulatory requirements and in sufficient quantities for commercialization and clinical testing. We do not currently have arrangements in place for a redundant or second-source supply of any such drug substance and drug product in the event any of our current suppliers of such drug substance and drug product cease their operations or stop offering us sufficient quantities of these materials for any reason.

We are not certain that our single-source suppliers will be able to meet our demand for their products, either because of the nature of our agreements with those suppliers, our limited experience with those suppliers or our relative importance as a customer to those suppliers. It may be difficult for us to assess their ability to timely meet our demand in the future based on past performance. While our suppliers have generally met our demand on a timely basis in the past, they may subordinate our needs in the future to their other customers.

In addition, to manufacture rilonacept, mavrilimumab and KPL-716 in the quantities that we believe would be required to meet anticipated market demand, our third-party manufacturers may need to increase manufacturing capacity and, in some cases, we could secure alternative sources of commercial supply, which could involve significant challenges and may require additional regulatory approvals. In addition, the development of commercial-scale manufacturing capabilities may require us and our third-party manufacturers to invest substantial additional funds and hire and retain the technical personnel who have the necessary manufacturing experience. Neither we nor our third-party manufacturers may successfully complete any required increase to existing manufacturing capacity in a timely manner, or at all.

Moreover, if there is a disruption to one or more of our third-party manufacturers' or suppliers' relevant operations the supply of rilonacept, mavrilimumab or KPL-716 will be delayed until such manufacturer or supplier restores the affected facilities or we or they procure alternative manufacturing facilities or sources of supply. Our ability to progress our pre-clinical and clinical programs could be materially and adversely impacted if any of the third-party suppliers upon which we rely were to experience a significant business challenge, disruption or failure due to issues such as financial difficulties or bankruptcy, issues relating to other customers such as regulatory or quality compliance issues, or other financial, legal, regulatory or reputational issues. Additionally, any damage to or destruction of our third-party manufacturers' or suppliers' facilities or equipment may significantly impair our ability to manufacture our product candidates on a timely basis.

Establishing additional or replacement suppliers for the drug substance and drug product used in our product candidates, if required, may not be accomplished quickly and can take several years, if at all. Furthermore, despite our efforts, we may be unable to procure a replacement supplier or do so on commercially reasonable terms, which could have a material adverse impact upon our business. If we are able to find a replacement supplier, such replacement supplier would need to be qualified and may require additional regulatory approval, which could result in further delay. While we seek to maintain adequate inventory of the drug substance and drug product used in our product candidates, any interruption or delay in the supply of components or materials, or our inability to obtain such drug substance and drug product from alternate sources at acceptable prices in a timely manner could impede, delay, limit or prevent our development efforts, which could harm our business, results of operations, financial condition and prospects.

Certain of the materials required in the manufacture and the formulation of our product candidates are derived from biological sources. Such materials are difficult to procure and may be subject to contamination or recall. Access to and supply of sufficient quantities of raw materials which meet the technical specifications for the production process is challenging, and often limited to single-source suppliers. Finding an alternative supplier could take a significant amount of time and involve significant expense due to the nature of the products and the need to obtain regulatory approvals. If we or our manufacturers are unable to purchase the materials necessary for the manufacture of our product candidates on acceptable terms, in a timely manner, at sufficient quality levels, or in adequate quantities, if at all, our ability to produce sufficient quantities of our products for clinical or commercial requirements would be negatively impacted. A material shortage, contamination, recall or restriction on the use of certain biologically derived substances or any other material used in the manufacture of our product candidates could adversely impact or disrupt manufacturing, which would impair our ability to generate revenues from the sale of such product candidates, if approved or cleared.

***We rely, and expect to continue to rely, on third parties, including independent investigators and CROs, to conduct our research, pre-clinical studies, clinical trials and other trials for our product candidates. If these third parties do not successfully carry out their contractual duties, comply with regulatory requirements or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.***

We do not have the ability to independently conduct pre-clinical studies or clinical trials that comply with the GLPs or GCP requirements, respectively. We rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct or otherwise support our GLP-compliant pre-clinical studies and GCP-compliant clinical trials for our product candidates properly and on time. We also rely on third parties to conduct other research related to our product candidates. We expect to rely heavily on these parties for execution of clinical trials for our product candidates and control only certain aspects of their activities. While we have agreements governing their activities, we control only certain aspects of their activities and have limited influence over their actual performance. The

third parties with whom we contract for execution of our GLP-compliant pre-clinical studies and our GCP-compliant clinical trials play a significant role in the conduct of these trials and the subsequent collection and analysis of data. These third parties are not our employees and, except for restrictions imposed by our contracts with such third parties, we have limited ability to control the amount or timing of resources that they devote to our programs. Although we rely on these third parties to conduct our GLP-compliant pre-clinical studies and GCP-compliant clinical trials, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on these third parties does not and will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our clinical trials, we could be subject to warning letters or enforcement actions that may include civil penalties and criminal prosecution.

We and our CROs will be required to comply with regulations, including GCPs, for conducting, monitoring, recording and reporting the results of clinical trials to ensure that the data and results are scientifically credible and accurate, and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any products in clinical development. The FDA enforces GCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot ensure that, upon inspection, the FDA or comparable foreign regulatory authorities will determine that any of our future clinical trials will comply with GCPs. In addition, our clinical trials must be conducted with product candidates produced under cGMPs regulations. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action. We also are required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so when required can result in fines, adverse publicity and civil and criminal sanctions.

Although we have and intend to continue to design the clinical trials for our product candidates, CROs will conduct all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future clinical trials will also result in less direct control over the management of data developed through clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CROs do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates, or our development program may be materially and irreversibly harmed. If we are unable to rely on clinical data collected by our CROs, we could be required to repeat, extend the duration of or increase the size of any clinical trials we conduct and this could significantly delay commercialization and require significantly greater expenditures.

These third parties are not our employees and we are not able to control, other than by contract, the amount of resources, including time, which they devote to our clinical trials. If our independent investigators or CROs fail to devote sufficient resources to the development of our product candidates, or if their performance is substandard, it may delay or compromise the prospects for approval and commercialization of our product candidates. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information is misappropriated.

If the third parties conducting our pre-clinical studies or our clinical trials do not perform their contractual duties or obligations, experience significant business challenges, disruptions or failures, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the data they obtain is compromised due to their failure to adhere to our protocols or to GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties. This could be difficult, costly or impossible, and our pre-clinical studies or clinical trials may need to be extended, delayed, terminated or repeated. As a result, we may not be able to obtain regulatory approval in a timely fashion, or at all, for the applicable product candidate, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative third-party service providers at all or on commercially reasonable terms. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to their failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any clinical trials such CROs are associated with may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, we believe that our financial results and the commercial prospects for our product candidates in the subject indication would be harmed, our costs could increase and our ability to generate revenue could be delayed.

***Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our product candidates.***

We may seek collaboration arrangements for the development, or potentially for the commercialization, of certain of our product candidates depending on the merits of retaining rights to develop or commercialize the product candidates ourselves as compared to entering into collaboration arrangements. In addition, we may seek to jointly develop or commercialize one or more of our product candidates. We will face, to the extent that we decide to enter into collaboration arrangements, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document, implement and maintain. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements should we so chose to enter into such arrangements. The terms of any collaborations or other arrangements that we may establish may not be favorable to us. In addition, our right to grant a sublicense of intellectual property licensed to us under certain of our current agreements requires the consent of the applicable licensor.

Any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include risks that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- a collaborator with development, commercialization, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that

could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;

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

- collaborators may own or co-own intellectual property covering products that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property, and even if we are able to license such exclusive rights, we may have to enter into a license agreement that include obligations to make milestone, royalty or other payments under such agreement;

- disputes may arise with respect to the ownership of any intellectual property developed pursuant to our collaborations; and

- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

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

Because we rely on third parties to develop and manufacture our product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may harm our business. To the extent that we share trade secrets of third parties that are licensed to us, unauthorized use or disclosure could expose us to liability.

**Risks Related to Competition, Retaining Key Employees and Managing Growth**

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

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

While we are not aware of any therapies currently approved or actively continuing clinical trials in recurrent pericarditis, there is one currently marketed product that modulates the signaling of IL-1α and IL-1β, anakinra (KINERET), and one currently marketed product that modulates the signaling of IL-1β, canakinumab (ILARIS). There are other therapies which modulate IL-1α and IL-1β in various stages of clinical development for diseases other than

recurrent pericarditis from companies that include AbbVie, Inc., or AbbVie, XBiotech Inc. and Handok Inc. We expect mavrilimumab, if approved, to experience competitive pressure from tocilizumab (ACTEMRA), which was approved in 2017 for use in GCA in combination with glucocorticoids. Additional competition may be experienced from Eli Lilly and AbbVie which are conducting clinical trials for oral janus kinase inhibitors, Sanofi S.A. and Regeneron which are recruiting a Phase 3 clinical trial with their anti-IL-6 program, Novartis International AG, which is recruiting a trial with its IL-17 antagonist secukinumab (Cosentyx) and Janssen which is testing ustekinumab (STELARA) in two small studies for GCA. KPL-716, if approved for atopic dermatitis, will face competitive pressure from dupilumab (DUPIXENT), which is approved to treat atopic dermatitis. KPL-716 may face additional competition from several systemically administered products currently in development for atopic dermatitis including upadacitinib, PF-04965842, ANB-020, nemolizumab, baricitinib, ASn002, GBR-830, ZPL-389, PF-06817024, MEDI9314, MOR106, ARGX-112, tralokinumab and lebrikizumab. Multiple therapies are in development for prurigo nodularis and any that receive FDA approval for this indication will be likely competitors to KPL-716. These products include nemolizumab, serlopitant and nalbuphine ER.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, pre-clinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products 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 commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any drugs that we or our collaborators may develop. Our competitors also may obtain FDA 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 or our collaborators are able to enter the market. The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, the effectiveness of companion diagnostics in guiding the use of related products, market acceptance by physicians and patients, the level of generic competition and the availability of reimbursement from government and other third-party payors.

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

We have a limited operating history and are highly dependent on the research and development, clinical, commercial and business development expertise of Sanj K. Patel, our Chief Executive Officer and Chairman of the Board of Directors, Stephen Mahoney, our President and Chief Operating Officer, and John F. Paolini, M.D., Ph.D., our Chief Medical Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel is also critical to our success. The failure to recruit, or the loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry

with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. Failure to succeed in clinical trials may make it more challenging to recruit and retain qualified scientific personnel. If we are not able to continue to attract and retain, on acceptable terms, the qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or growth.

***We need to continue to develop and expand our company, and we may encounter difficulties in managing this development and expansion, which could disrupt our operations.***

We expect to continue to increase our number of employees and the scope of our operations. To manage our anticipated development and expansion, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Also, our management may need to divert a disproportionate amount of its attention away from its day-to-day activities and devote a substantial amount of time to managing these development activities. Due to our limited resources, certain employees may need to perform activities that are beyond their regular scope of work, and we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate 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, if approved, and to compete effectively will depend, in part, on our ability to effectively manage the future development and expansion of our company.

## Risks Related to Intellectual Property

***If we are unable to adequately protect our proprietary technology or obtain and maintain patent protection for our technology and products, if the scope of the patent protection obtained is not sufficiently broad, or if the terms of our patents are insufficient to protect our product candidates for an adequate amount of time, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be materially impaired.***

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection in the United States and other countries for our product candidates, including rilonacept, mavrilimumab and KPL-716. We seek to protect our proprietary and intellectual property position by, among other methods, filing patent applications in the United States and abroad related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary and intellectual property position.

We acquire, in-license and file patent applications directed to our product candidates in an effort to establish intellectual property positions directed to their compositions of matter as well as uses of these product candidates in the treatment of diseases. Our intellectual property includes patents and patent applications that we own as well as patents and patent applications that we in-license. For example, we have a field-specific exclusive license under a license agreement with Regeneron to patent applications and patents relating to rilonacept, an exclusive license under a license agreement with MedImmune, or the MedImmune Agreement, to patent applications and patents relating to mavrilimumab, and an exclusive license under our license agreement with Novo Nordisk to patent applications and patents relating to KPL-045.

Certain provisions in our intellectual property agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could affect the scope of our rights to the relevant intellectual property or technology, or affect financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

We or our licensors have not pursued or maintained, and may not pursue or maintain in the future, patent protection for our products in every country or territory in which we may sell our products. In addition, we cannot be sure that any of our pending patent applications or pending trademark applications will issue or that, if issued, they have or will issue in a form that will be advantageous to us. The United States Patent and Trademark Office, or the USPTO, international patent offices or judicial bodies may deny or significantly narrow claims made under our patent applications and our issued patents may be successfully challenged, may be designed around, or may otherwise be of insufficient scope to provide us with protection for our commercial products. Further, the USPTO, international trademark offices or judicial bodies may deny our trademark applications and, even if published or registered, these trademarks may not effectively protect our brand and goodwill. Like patents, trademarks also may be successfully opposed or challenged.

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. The degree of patent protection we require to successfully commercialize our product candidates may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our owned or in-licensed patents have, or that any of our owned or in-licensed pending patent applications that mature into issued patents will have, claims with a scope sufficient to protect rilonacept, mavrilimumab, KPL-716 or our other product candidates. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions and adjustments may be available; however, the life of a patent, and the protection it affords, is limited. The actual protection afforded by a patent varies on a product-by-product basis, from country to country and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

Patents may be eligible for limited patent term extension in the United States under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act. Similar patent extensions exist in the European Union and Japan, subject to the applicable laws in those jurisdictions. We may not receive an extension if we fail to apply within applicable deadlines or fail to apply prior to expiration of relevant patents. For example, no patent term extension was obtained in the United States following the FDA's approval of rilonacept for the treatment of CAPS, and the deadline for applying for such extension has passed. Accordingly, patent term extension in the United States based on the FDA's approval of rilonacept for CAPS, or any other indication for which the FDA may grant approval in the future, is unavailable. Further, while patent term extension was awarded for relevant patents in certain European countries following the EMA's approval of rilonacept for the treatment of CAPS, in 2012 the marketing authorization for CAPs was withdrawn. Patent term extensions may no longer be in effect or available, subject to the applicable laws in those countries as well as other factors, such as whether a marketing approval for rilonacept is reissued and whether such reissuance is prior to the expiration of the patent's natural 20-year patent term. Moreover, the length of the extension could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner, impacting our revenue.

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, thereby limiting protection such patent would afford the respective product and any competitive advantage such patent may provide. In some cases, an in-licensed patent portfolio may have undergone a considerable loss of patent term prior to our initiation of development and commercialization of the product candidate. For example, the patents covering rilonacept as a composition of matter have a term that expires in 2019 in the United States, not including patent term adjustment, and in 2023 in Europe, not including any patent term extensions. As a result, our owned and in-licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from

commercializing products similar or identical to our product candidates. In such cases, we expect to rely on regulatory exclusivity for our product candidates, such as orphan drug exclusivity, which generally grants seven years of marketing exclusivity under the Federal Food, Drug, and Cosmetic Act, and up to ten years of marketing exclusivity in Europe. While, we expect to pursue orphan drug designation for rilonacept in the United States for the treatment of recurrent pericarditis, we may not be successful in obtaining such designation or we may not be able to maintain the benefits of the designation. Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect those product candidates from competition because different drugs can be approved for the same condition, and orphan drug exclusivity does not prevent the FDA from approving the same or a different drug in another indication. Even after an orphan drug is approved, the FDA can subsequently approve a later application for the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. See "—We may seek orphan drug designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity, for any product candidate for which we obtain orphan drug designation."

Other parties may have developed or may develop technologies that may be related or competitive to our own, and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our patent applications or issued patents, with respect to either the same methods or formulations or the same subject matter, in either case, that we may rely upon to dominate our patent position in the market. 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 know with certainty whether we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty.

In addition, 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. Patent prosecution is a lengthy process, during which the scope of the claims initially submitted for examination by the USPTO is often significantly narrowed by the time they issue, if at all. The claims of our issued patents or patent applications when issued may not cover our product candidates, proposed commercial technologies or the future products that we develop, or even if such patents provide coverage, the coverage obtained may not provide any competitive advantage. 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. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. In the case of our field-limited license from Regeneron, another licensee may have the right to enforce patents covering the product in their field. As a result, we may need to coordinate enforcement with another party, and the other party could enforce the patents in a manner adverse to our interests or otherwise put the patents at risk of invalidation.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or license may fail to result in issued patents in the United States or in foreign countries. Even if we acquire patent protection that we expect should enable us to maintain a competitive advantage, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. The issuance of a patent is not conclusive as to its inventorship, scope, validity, enforceability or term, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the USPTO challenging the priority of an invention claimed within one of our patents, which submissions may also be made prior to a patent's issuance, precluding the granting of any of our pending patent applications. We may become involved in contested proceedings challenging our patent rights or the patent rights of others from whom we have obtained licenses to such rights. For example, patents granted by the USPTO may be subject to third-party challenges

such as (without limitation) derivation, re-examination, interference, post-grant review or *inter partes* review proceedings, and patents granted by the European Patent Office may be challenged by any person in an opposition proceeding within nine months from the publication of the grant. Similar proceedings are available in other jurisdictions, and in some jurisdictions third parties can raise questions of validity with a patent office even before a patent has granted. Competitors may claim that they invented the inventions claimed in our issued patents or patent applications prior to us, or may file patent applications before we do. In such case, we may have to participate in interference or derivation proceedings in the USPTO, to determine which party is entitled to a patent on the disputed invention. We may also become involved in similar opposition proceedings in the European Patent Office or similar offices in other jurisdictions regarding our intellectual property rights with respect to our products and technology.

Such proceedings can be expensive, time consuming and may divert the efforts of our technical and managerial personnel, which could in turn harm our business, whether or not we receive a determination favorable to us. We may not be able to correctly estimate or control our future operating expenses in relation to such proceedings, which could affect operating expenses. Our operating expenses may fluctuate significantly in the future as a result of a variety of factors, including the costs of such proceedings.

Since patent applications are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to our product candidates. Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, rights to those challenged patents may be diminished or lost.

In addition, we may in the future be subject to claims by our or our licensors' former employees or consultants asserting an ownership right in our patents or patent applications, as a result of the work they performed on our or their behalf, respectively. Although we generally require all of our employees and consultants and any other partners or collaborators who have access to our proprietary know-how, information or technology to assign or grant similar rights to their inventions to us, we cannot be certain that we or our licensors have executed such agreements with all parties who may have contributed to our intellectual property, nor can we be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy.

An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, without payment to us, or could limit the duration of the patent protection covering our technology and product candidates. Such challenges may also result in our inability to manufacture or commercialize our product candidates without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if they are unchallenged, our issued patents and our pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. For example, a third party may develop a competitive drug that provides benefits similar to one or more of our product candidates but that has a different composition that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, or if the breadth, strength or term (including any extensions or adjustments) of protection provided by the patents and patent applications we hold or pursue with respect to our product candidates or any future product candidates is successfully challenged, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates or any future product candidates under patent protection would be reduced.

***Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. If we breach any of the agreements under which we acquired our product candidates, we could lose the ability to continue the development and commercialization of the related product. Additionally, our current licensing and acquisition agreements contain limitations and restrictions that could limit or adversely affect our ability to develop and commercialize other products in the future.***

We entered into agreements to acquire the rights to develop or commercialize our product candidates, rilonacept, mavrilimumab, KPL-716, KPL-045 and KPL-404. In September 2017, we entered into a license agreement with Regeneron to obtain an exclusive license under certain intellectual property rights controlled by Regeneron to develop and commercialize rilonacept. In December 2017, we entered into a license agreement with MedImmune to obtain exclusive worldwide rights to research, develop, manufacture, market and sell mavrilimumab and any other products covered by the licensed patent rights. In September 2016, pursuant to an asset purchase agreement with Biogen, or the Biogen Agreement, we acquired all of Biogen's right, title and interest in and to certain assets used in or relating to KPL-716, including patents and other intellectual property rights, clinical data, know-how and inventory. Each of these agreements requires us to use commercially reasonable efforts to develop and commercialize the related product candidates, make timely milestone and other payments, provide certain information regarding our activities with respect to such product candidates and indemnify the other party with respect to our development and commercialization activities under the terms of the agreements. In August 2017, we licensed KPL-045 from Novo Nordisk. These agreements and any future such agreements that we enter into impose a variety of obligations and related consequences.

We are a party to a number of license and acquisition agreements of importance to our business and to our current product candidates, and we expect to be subject to additional such agreements in the future. Disputes may arise between us and any of these counterparties regarding intellectual property subject to and each parties' obligations under such agreements, including:

- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;
- the scope of rights granted under the agreement and other interpretation-related issues;
- our obligations to make milestone, royalty or other payments under those agreements;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the agreement;
- our right to sublicense patent and other rights to third parties;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners;
- our right to transfer or assign the license; and
- the effects of termination.

These or other disputes over our obligations or intellectual property that we have licensed or acquired may prevent or impair our ability to maintain our current arrangements on acceptable terms, or may impair the value of the arrangement to us. Any such dispute could have an adverse effect on our business.

If we fail to meet our obligations under these agreements in a material respect, the respective licensor/seller would have the right to terminate the respective agreement and upon the effective date of such termination, have the right to re-obtain the related technology as well as aspects of any intellectual property controlled by us and developed during the period the agreement was in force that relate to the applicable technology. This means that the licensor/seller to each of these agreements could effectively take control of the development and commercialization of our product

candidates after an uncured, material breach of the agreement by us. This would also be the case if we voluntarily terminate the relevant agreement. While we would expect to exercise our rights and remedies available to us in the event we fail to meet our obligations under these agreements in any material respect, including seeking to cure any breach by us, and otherwise seek to preserve our rights under the technology licensed to or acquired by us, we may not be able to do so in a timely manner, at an acceptable cost or at all. Any uncured, material breach under the license could result in our loss of exclusive rights and may lead to a complete termination of our product development and any commercialization efforts for each of our product candidates.

Regeneron has rights to develop rilonacept in its retained fields of local administration to the eye and ear, oncology, deficiency of the interleukin-1 receptor, and CAPS. Regeneron may also develop rilonacept in fields to which we have licensed the rights, but we retain the commercial benefit related to that development upon approval of rilonacept in any field that we have licensed. We and Regeneron communicate with each other concerning our related development activities, and we have approval rights over Regeneron's development in the fields that we have licensed, including pericarditis. Outside of the United States and Japan, Regeneron has granted a third-party licensee the right to develop and commercialize rilonacept in CAPS and certain periodic fever syndromes. The development of rilonacept in other fields could increase the possibility of identification of adverse safety results that impact our development of rilonacept for recurrent pericarditis. In addition, if approved, commercialization of rilonacept in other fields could result in an increased threat of off-label use to compete with the sale of rilonacept to treat these indications, which may diminish sales of rilonacept in fields licensed exclusively to us.

Certain of our agreements may limit or delay our ability to consummate certain transactions, may impact the value of those transactions, or may limit our ability to pursue certain activities. For example, under the MedImmune Agreement, we cannot sublicense the rights licensed or sublicensed to us without the consent of MedImmune and certain applicable third-party licensors, if required by agreements between MedImmune and such third-party licensors. Under the Biogen Agreement, Biogen has a right of first negotiation under certain circumstances to purchase the assets we acquired from Biogen or to obtain a license to exploit the applicable products. This right of first negotiation remains in effect until the earlier of 12 years from the date of the agreement or the first commercial sale of a product under the agreement, and applies to a variety of transactions, including licensing transactions and the sale of our company. In addition, under the Biogen Agreement, we are subject to an exclusivity obligation, pursuant to which we may not conduct any activity alone or through a third party related to a product that modulates the OSM receptor (other than for the development and commercialization of products that are the subject of the Biogen Agreement). This exclusivity obligation runs from the earlier of the eighth anniversary of the agreement or the first commercial sale of a product that is the subject of the agreement.

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.***

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and frequent litigation regarding patents and other intellectual property rights. We cannot assure you that our product candidates or any future product candidates, including methods of making or using these product candidates, will not infringe existing or future third-party patents. We may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including contested proceedings before the USPTO. Our competitors or other third parties may assert infringement claims against us, alleging that our products are covered by their patents.

Given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future. Many companies have filed, and continue to file, patent applications related to immunomodulation. Some of these patent applications have already been allowed or issued, and others may issue in the future. For example, we are aware of third-party patents that contain claims potentially relevant to certain therapeutic uses of mavrilimumab and KPL-716. If the claims of any of these patents are asserted against us, we do not believe our proposed activities related to mavrilimumab and KPL-716 would be found to infringe any valid claim of these patents. While we may decide to initiate proceedings to challenge the

validity of these or other patents in the future, we may be unsuccessful, and courts or patent offices in the United States and abroad could uphold the validity of any such patent. If we were to challenge the validity of any issued U.S. patent in court, we would need to overcome a statutory presumption of validity that attaches to every U.S. patent. This means that in order to prevail, we would have to present clear and convincing evidence as to the invalidity of the patent's claims. In order to avoid infringing these or any other third-party patents, we may find it necessary or prudent to obtain licenses to such patents from such third-party intellectual property holders. However, we may be unable to secure such licenses or otherwise acquire or in-license any compositions, methods of use, processes, or other intellectual property rights from third parties that we identify as necessary for our current or future product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may also pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Since our product candidates are being developed for use in fields that are competitive and of strong interest to pharmaceutical and biotechnology companies, we will likely seek to file additional patent applications and may have additional patents granted in the future, based on our future research and development efforts. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our product candidates. Regardless of when filed, we may fail to identify relevant third-party patents or patent applications, or we may incorrectly conclude that a third-party patent is invalid or not infringed by our product candidates or activities. If a patent holder believes our product candidate infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the product candidate that is the subject of the actual or threatened suit.

If we are found to infringe a third-party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our product candidates and technology. Under any such license, we would most likely be required to pay various types of fees, milestones, royalties or other amounts. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain such a license, it could be granted on non-exclusive terms, thereby providing our competitors and other third parties access to the same technologies licensed to us. Without such a license, we could be forced, including by court order, to cease developing and commercializing the infringing technology or product candidate, or forced to redesign it, or to cease some aspect of our business operations. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed such third-party patent rights. We may be required to indemnify collaborators or contractors against such claims. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Even if we are successful in defending against such claims, litigation can be expensive and time consuming and would divert management's attention from our core business. Any of these events could harm our business significantly.

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

Competitors and other third parties may infringe, misappropriate or otherwise violate our patents and other intellectual property rights, whether owned or in-licensed. To counter infringement or unauthorized use, we or our current or future collaborators may be required to file infringement claims against these infringers. A court may disagree with our allegations, however, and may refuse to stop the other party from using the technology at issue on the grounds

that our patents do not cover the third-party technology in question. Further, such third parties could counterclaim that we infringe their intellectual property or that a patent we have asserted against them is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims challenging the validity, enforceability or scope of asserted patents are commonplace. In addition, third parties may initiate legal proceedings against us to assert such challenges to our intellectual property rights. The outcome of any such proceeding is generally 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. Patents may be unenforceable if someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. It is possible that prior art of which we or our licensors and the patent examiner were unaware during prosecution exists, which could render our patents invalid. Moreover, it is also possible that prior art may exist that we are aware of but do not believe is relevant to our current or future patents, but that could nevertheless be determined to render our patents invalid.

Some of our competitors may be able to devote significantly more resources to intellectual property litigation, and may have significantly broader patent portfolios to assert against us if we assert our rights against them. Further, because of the substantial discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be disclosed or otherwise compromised during litigation.

An adverse result in any litigation proceeding could put one or more of our patents, whether owned or in-licensed, at risk of being invalidated or interpreted narrowly. If a defendant were to prevail on a legal assertion of invalidity or unenforceability of our patents covering one of our product candidates, we would lose at least part, and perhaps all, of the patent protection covering such product candidate. Competing products may also be sold in other countries in which our patent coverage might not exist or be as strong. If we lose a foreign patent lawsuit, alleging our infringement of a competitor's patents, we could be prevented from marketing our products in one or more foreign countries. Any of these outcomes would have a materially adverse effect on our business.

***Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.***

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming and is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. 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. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our Class A common shares. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating or from successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

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

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance fees on issued patents often must be paid to the USPTO and foreign patent agencies over the lifetime of the patent. While an unintentional lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or

lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our products or technologies, we may not be able to stop a competitor from marketing products that are the same as or similar to our product candidates, which would have a material adverse effect on our business. In addition, if we fail to apply for applicable patent term extensions or adjustments, we will have a more limited time during which we can enforce our granted patents. In addition, if we are responsible for patent prosecution and maintenance of patent rights in-licensed to us, any of the foregoing could expose us to liability to the applicable patent owner.

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

Filing, prosecuting and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive. The requirements for patentability may differ in certain countries, particularly in developing countries. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. In addition, the patent laws of some foreign countries do not afford intellectual property protection to the same extent as the laws of the United States. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. Varying filing dates in international countries may also permit intervening third parties to allege priority to patent applications claiming certain technology. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. In addition, many countries limit the enforceability of patents against certain parties, including government agencies or government contractors. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our product candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Proceedings to enforce our patent rights, whether owned or in-licensed, in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Furthermore, while we intend to pursue protection for our intellectual property rights in the major markets for our product candidates, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate. In addition, changes in the law and legal decisions by courts in the United States and foreign countries may affect our ability to obtain and enforce adequate intellectual property protection for our technology.

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or Leahy-Smith Act, signed into law on September 16, 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a first-to-file system. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions, whether owned or

in-licensed, and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, in each case whether owned or in-licensed, all of which could harm our business, results of operations and financial condition.

Among some of the other changes introduced by the Leahy-Smith Act are changes that limit where a patentee may file a patent infringement suit and provide new opportunities for third parties to challenge issued patents in the USPTO. We may be subject to the risk of third-party prior art submissions on pending applications or become a party to opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patents. There is a lower standard of evidence necessary to invalidate a patent claim in a USPTO proceeding relative to the standard in U.S. district or federal court. This could lead third parties to challenge and successfully invalidate our patents that would not otherwise be invalidated if challenged through the court system.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to obtain or maintain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents; enforce or shorten the term of our existing patents and patents that we might obtain in the future; shorten the term that has been lengthened by patent term adjustment of our existing patents or patents that we might obtain in the future; or challenge the validity or enforceability of patents that may be asserted against us by our competitors or other third parties.

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

In addition to the protection afforded by patents, we may rely upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain our competitive position. Although we seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our collaborators, scientific advisors, contractors, employees and consultants, and invention assignment agreements with our consultants, scientific advisors and employees, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Moreover, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators, or others is inadvertently disclosed or subject to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets.

We cannot be certain that the steps we have taken will prevent unauthorized use or unauthorized reverse engineering of our technology. Monitoring unauthorized use of our intellectual property is difficult and costly. We may not be able to detect unauthorized use of, or take appropriate steps to enforce, our intellectual property rights. The steps we have taken to protect our proprietary rights may not be adequate to prevent misappropriation of our intellectual property.

We also seek to preserve the integrity and confidentiality of our data and other confidential information by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached. Detecting the disclosure or misappropriation of confidential information and enforcing a claim that a party illegally disclosed or misappropriated confidential information is difficult, expensive and time consuming, and the

outcome is unpredictable. Further, we may not be able to obtain adequate remedies for any breach. In addition, our confidential information may otherwise become known or be independently discovered by competitors, in which case we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. We may in the future rely on trade secret protection, which would be subject to the risks identified above with respect to confidential information.

Our trade secrets could otherwise become known or be independently discovered by our competitors. Competitors could purchase our product candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products, our competitive position could be adversely affected, as could our business.

***If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.***

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and prospects.

***We have not yet registered trademarks for a commercial trade name for our lead product candidates in the United States or foreign jurisdictions and failure to secure such registrations could adversely affect our business.***

We have not yet registered trademarks for a commercial trade name for some of our lead product candidates in the United States or any foreign jurisdiction. During trademark registration proceedings, we may receive rejections. Although we are given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use with our product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

## Risks Related to Commercialization

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

The precise incidence and prevalence for all the conditions we aim to address with our programs are unknown. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research, and may prove to be incorrect. Further, new trials may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected.

The total addressable market across all of our product candidates will ultimately depend upon, among other things, the diagnosis criteria included in the final label for each of our product candidates approved for sale for these indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients in the United States and other major markets and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our products or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

***If, in the future, we are unable to establish our own sales, marketing and distribution capabilities, or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing our product candidates if and when they are approved, and we may not be able to generate any revenue.***

We do not currently have a sales, marketing or distribution infrastructure. We have never sold, marketed or distributed any therapeutic products. To achieve commercial success for any approved product candidate, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We currently plan to establish our own sales and marketing capabilities and directly commercialize any approved product candidate.

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

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

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- our inability to equip medical and sales personnel with effective materials, including medical and sales literature to help them educate physicians and other healthcare providers regarding applicable diseases and our future products;

- our inability to develop or obtain sufficient operational functions to support our commercial activities; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing, distribution and other commercial support services, our product revenues or the profitability of these revenues to us are likely to be lower than if we were to market and sell any product candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our product candidates effectively. Developing a sales and marketing organization requires significant investment, is time consuming and could delay the launch of our product candidates. We may not be able to build an effective sales and marketing organization in the United States, the European Union or other key global markets. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates. Further, our business, results of operations, financial condition and prospects will be materially adversely affected.

***Our current or future product candidates may not gain market acceptance by physicians or patients, in which case our ability to generate product revenues will be compromised.***

Even if the FDA or any other regulatory authority approves the marketing of our product candidates, whether developed on our own or with a collaborator, physicians, healthcare providers, patients or the medical community may not accept or use our product candidates. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenue or any profits from operations. The degree of market acceptance of our product candidates will depend on a variety of factors, including:

- the timing of market introduction;
- the number and clinical profile of competing products;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the clinical indications for which our product candidates are approved;
- our ability to provide acceptable evidence of safety and efficacy;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- limitations or warnings contained in the labeling approved by the FDA or other regulatory authorities;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;
- cost effectiveness, particularly in relation to alternative treatments;
- the effectiveness of our sales, marketing and distribution support;
- availability of adequate coverage, reimbursement and payment from health maintenance organizations and other insurers, both public and private; and
- other potential advantages over alternative treatment methods.

If our product candidates fail to gain market acceptance, our ability to generate revenues will be adversely affected. Even if our product candidates achieve market acceptance, the market may prove not to be large enough to allow us to generate significant revenues.

***The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish adequate coverage, reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.***

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

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

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative drug therapies before they will reimburse healthcare providers who use such therapies. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Third-party payors increasingly are challenging prices charged for pharmaceutical or biologic products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing products may limit the amount we will be able to charge for our product candidates. These

payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in our product candidates. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates, and may not be able to obtain a satisfactory financial return on our product candidates.

The regulations that govern regulatory approvals, pricing and reimbursement for new products vary widely from country to country. Our operations are subject to extensive governmental price control or other market regulations in other countries outside of the United States, and we believe the increasing emphasis on cost-containment initiatives in European and other countries have and will continue to put pressure on the pricing and usage of our product candidates. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product candidate in a particular country, but then be subject to price regulations that delay our commercial launch of the product candidate, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product candidate in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products.

***Our future growth may depend, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.***

Our future profitability may depend, in part, on our ability to commercialize our product candidates in foreign markets for which we may rely on collaboration with third parties. For example, we are actively recruiting and screening subjects for a single, pivotal, global Phase 3 clinical trial with rilonacept for the treatment of recurrent pericarditis. Although we do not have immediate plans to pursue the commercialization of rilonacept for recurrent pericarditis outside of the United States, we are evaluating the opportunities for the development and commercialization of our product candidates in certain foreign markets. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the applicable regulatory authority in that foreign market, and we may never receive such regulatory approval for any of our product candidates. To obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales, pricing and distribution of our product candidates, and we cannot predict success in these jurisdictions. If we obtain approval of our product candidates and ultimately commercialize our product candidates in foreign markets, we would be subject to additional risks and uncertainties, including:

- our customers' ability to obtain reimbursement for our product candidates in foreign markets;
- our inability to directly control commercial activities because we are relying on third parties;
- the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements;
- different medical practices and customs in foreign countries affecting acceptance in the marketplace;

- import or export licensing requirements;
- longer accounts receivable collection times;
- longer lead times for shipping;
- language barriers for technical training and the need for language translations;
- reduced protection of intellectual property rights in some foreign countries;
- the existence of additional potentially relevant third-party intellectual property rights;
- foreign currency exchange rate fluctuations; and
- the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of our product candidates could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs.

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

***We are currently, and if we receive regulatory approval for any of our product candidates, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products***.

If the FDA or a comparable foreign regulatory authority approves any of our product candidates, it will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, distribution, storage, advertising, promotion, sampling, record-keeping adverse event reporting, conduct of post-marketing trials and submission of safety, efficacy and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, and comparable foreign regulatory authority, requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our CMOs will be subject to user fees and continual review and inspections to assess compliance with cGMP and adherence to commitments made in any BLA or MAA. 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.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. We will be required to report certain adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance.

We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs and biologics are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have approval. Even our current clinical and medical affairs activities are subject to certain ongoing regulatory requirements concerning appropriate exchange of medical and scientific information.

The holder of an approved BLA or MAA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling or manufacturing process. We could also be asked to conduct post-marketing clinical trials to verify the safety and efficacy of our products in general or in specific patient subsets. If original marketing approval were obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical trial to confirm clinical benefit for our products. An unsuccessful post-marketing trial or failure to complete such a trial could result in the withdrawal of marketing approval. The FDA also may place other conditions on approvals including the requirement for a REMS, to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS before it can obtain approval. A REMS could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we discover previously unknown problems with a product candidate, including adverse events of unanticipated severity or frequency, or with our manufacturing processes, or fail to comply with regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our CMOs' facilities; or
- seize or detain products, or require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected.

If there are changes in the application of legislation or regulatory policies, or if problems are discovered with a product or the manufacture of a product, or if we or one of our distributors, licensees or co-marketers fails to comply with regulatory requirements, the regulatory authorities could take various actions. These include imposing fines on us, imposing restrictions on our product or its manufacture and requiring us to recall or remove the product from the market. The regulatory authorities could also suspend or withdraw our marketing authorizations, or require us to conduct additional clinical trials, change our product labeling or submit additional applications for marketing authorization. If any of these events occurs, our ability to sell our product may be impaired, and we may incur substantial additional expense to comply with regulatory requirements.

The policies of the FDA and other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States, Europe or in other jurisdictions. For example, the current U.S. presidential administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. It is difficult to predict how these Executive Orders will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose restrictions on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. In addition, if we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

***Our business operations and current and future relationships with investigators, healthcare professionals, consultants, customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse, physician payment transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, exclusion from government healthcare programs, contractual damages, reputational harm and diminished profits and future earnings.***

Although we do not currently have any products on the market, as we near commercialization and as we begin commercializing our product candidates, if approved, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our product candidates for which we obtain marketing approval.

Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. The U.S. federal Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other hand;

- the U.S. federal False Claims Act and civil monetary penalties laws, which, among other things, impose criminal and civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim includes "any request or demand" for money or property presented to the federal government. In addition, manufacturers can be held liable under the False Claims Act even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims;

- the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or obtain, by

means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or service. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the U.S. federal physician payment transparency requirements, sometimes referred to as the "Sunshine Act" created under Section 6002 of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act, which requires manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid or the Children's Health Insurance Program to report to the Department of Health and Human Services information related to certain financial interactions with physicians and teaching hospitals (and additional categories of health care practitioners beginning with reports due on or after January 1, 2022) and the ownership and investment interests of physicians and their immediate family members;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and its implementing regulations, which also imposes obligations on certain covered entity healthcare providers, health plans and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information; and

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

These laws and regulations, among other things, may constrain our business, marketing and other promotional activities by limiting the kinds of financial arrangements we may have with hospitals, physicians or other potential purchasers of our product candidates, if approved. We have entered into consulting and advisory board agreements with physicians, some of whom are paid in the form of shares or options to acquire our common shares. We could be adversely affected if regulatory agencies determine our financial relationships with such physicians to be in violation of applicable laws. Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our business activities could be subject to challenge under one or more of such laws. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations.

Interactions between biopharmaceutical companies and physicians are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct in the individual EU member states. The provision of any inducements to physicians to prescribe, recommend, endorse, order, purchase, supply, use or administer a drug product is prohibited. A number of EU member states have established additional rules requiring pharmaceutical companies to publicly disclose their interactions with physicians and to obtain approval from employers, professional organizations or competent authorities before entering into agreements with physicians.

Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including anticipated activities to be conducted by our sales

team, were to be found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, individual imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Further, defending against any such actions can be costly, time consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

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

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

- decreased demand for any product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- regulatory investigations that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of potential revenue;
- the diversion of management's attention away from managing our business; and
- the inability to commercialize any product candidates that we may develop.

Although we maintain product liability insurance coverage, it may not be adequate to cover all liabilities that we may incur and is subject to deductibles and coverage limitations. We anticipate that we will need to increase our insurance coverage when and if we successfully commercialize any product candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If we are unable to obtain insurance at acceptable cost or otherwise protect against potential product liability claims, we will be exposed to significant liabilities, which may materially and adversely affect our business and financial position. These liabilities could prevent or interfere with our commercialization efforts.

## Other Risks Related to Our Business

***We may not be successful in executing our growth strategy to identify, discover, develop, in-license or acquire additional product candidates or technologies, and our growth strategy may not deliver the anticipated results. We may seek to acquire businesses or undertake business combinations, collaborations, or other strategic transactions but we may not realize the intended benefits of such transactions.***

We have acquired and in-licensed our existing product candidates, and we plan to identify new product candidates or technologies that we believe are complementary to our existing product candidates. We may do this through our internal discovery program, or by acquiring the rights to product candidates and technologies through a variety of transactions types, including in-licensing, strategic transactions, mergers or acquisitions. If we are unable to identify, discover, develop, in-license or otherwise acquire and integrate product candidates, or their related companies, in accordance with this strategy, our ability to pursue this component of our growth strategy would be limited. We cannot assure you that we will be successful in such efforts or that, following any such discovery or transaction, we will be able to realize the intended benefits of it.

Research programs and business development efforts to identify new product candidates and technologies require substantial technical, financial and human resources. We may focus our efforts and resources on potential product candidates, technologies or businesses that ultimately prove to be unsuccessful. In-licensing and acquisitions of technology or businesses often require significant payments and expenses and will consume additional resources. We will need to continue to devote a substantial amount of time and personnel to research, develop and commercialize any in-licensed or acquired technology, or integrate any new business, in addition to our efforts on our existing portfolio of programs. Our research programs and business development efforts, including businesses or technology acquisitions, collaborations or licensing attempts, may fail to yield additional complementary or successful product candidates for clinical development and commercialization or successful business combinations for a number of reasons, including, but not limited to, the following:

- our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates or businesses with a high probability of success for development progression;
- we may not be able or willing to assemble sufficient resources or expertise to in-license, acquire or discover additional product candidates or acquire businesses or undertake business combinations, collaborations, or other strategic transactions;
- for product candidates we seek to in-license or acquire or for businesses we seek to acquire or undertake business combinations, collaborations or other strategic transactions with, we may not be able to agree to acceptable terms with the licensor or owner of those product candidates or businesses;
- our product candidates may not succeed in pre-clinical studies or clinical trials;
- we may not succeed in formulation or process development;
- any product candidates to which we acquire the rights or that we discover may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive regulatory approval;
- competitors may develop alternatives that render any product candidates or technologies to which we acquire the rights or that we discover, obsolete or less attractive;
- any product candidates or technologies to which we acquire the rights may be covered by third parties' patents or other exclusive rights;

- any product candidates or technologies to which we acquire the rights or that we discover may not allow us to leverage our expertise and our development and commercial infrastructure as currently expected;

- the market for any product candidates or technologies to which we acquire the rights or that we discover may change during our program so that such a product or technology may become unreasonable to continue to develop;

- any product candidate to which we acquire the rights or that we discover may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and

- any product candidate to which we acquire the rights or that we discover may not be accepted as safe and effective by patients, the medical community or third-party payors.

If any of these events occurs, we may not be successful in executing our growth strategy or strategic transactions, or our growth strategy or strategic acquisitions may not deliver the anticipated results.

The consummation or performance of any acquisition, business combination, collaboration or other strategic transaction we may undertake in furtherance of our growth strategy, may involve additional risks, such as difficulties in assimilating different cultures, retaining personnel and integrating operations, which may be geographically dispersed, increased costs, exposure to liabilities, incurrence of indebtedness or use a substantial portion of our available cash for all or a portion of the consideration, or cause dilution to our existing shareholders if we issue equity securities for all or a portion of the consideration. For example, in March 2019, we acquired all of the issued and outstanding equity of Primatope in exchange for upfront consideration of $10.0 million at closing as well as potential milestone payments of up to $8.0 million ($5.0 million of which had been achieved as of the closing date and was paid at closing), each paid or payable in a combination of cash and our Class A common shares (inclusive of escrow and holdback amounts) in accordance with the terms and conditions of our stock purchase option agreement with Primatope. If any of these events occurs or we are unable to meet our strategic objectives for any such transaction, we may not be able to achieve the expected benefits for the transaction.

***Enacted and future healthcare legislation may have a material adverse effect on our business and results of operations.***

In the United States, European Union and other jurisdictions, there have been and we expect there will continue to be a number of legislative and regulatory initiatives and proposed changes to the healthcare system that could affect our future operations. For example, in the United States, the Affordable Care Act substantially changes the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act, among other things, subjects biologic products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs and biologics that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs and biologics, including our product candidates, and a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts which, through subsequent legislative amendments, was increased to 70%, off negotiated prices of applicable brand drugs and biologics to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient products to be covered under Medicare Part D.

Since its enactment, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act. The current Presidential Administration and U.S. Congress have attempted and will likely continue to seek to modify, repeal or otherwise invalidate all, or certain provisions of, the Affordable Care Act. Most recently, the Tax Cuts and Jobs Act was enacted, which, among other things, removes penalties for not complying with the Affordable Care Act's individual mandate to carry health insurance. A recent federal district court ruling struck down the Affordable Care Act in its entirety. This decision means numerous reforms enacted as part of the Affordable Care Act, but not specifically related to health insurance, such as the BPCIA, are invalid as well. While the presidential administration and

The Centers for Medicare & Medicaid Services, or CMS, have both stated that the ruling will have no immediate effect, it is unclear how this decision, subsequent appeals, if any, and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business.

In addition, other legislative changes have been proposed and adopted in the United States since the Affordable Care Act was enacted. The Budget Control Act of 2011, among other things, led to aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2027 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several types of providers.

Additionally, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug and biologic pricing, reduce the cost of prescription drugs and biologics under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs and biologics. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved.

In markets outside of the United States and European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States, the European Union or elsewhere. If we or any third party we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or

policies, or if we or such third party are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

***Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Doing business internationally involves a number of risks, including but not limited to:

- multiple, conflicting and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions;
- employment laws, regulatory requirements and other governmental approvals, permits and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our products in various countries;
- additional potentially relevant third-party patent rights;
- complexities and difficulties in obtaining protection and enforcing our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products and exposure to foreign currency exchange rate fluctuations;
- natural disasters, political and economic instability, including wars, terrorism, political unrest, outbreak of disease and boycotts;
- curtailment of trade, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation and insurance; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions or its anti-bribery provisions.

Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

***Our internal computer systems, or those of our third-party CMOs, CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product candidates' development programs.***

Despite the implementation of security measures, our internal computer systems and those of our third-party CMOs, CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, theft, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data for our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or product candidates, or inappropriate disclosure or theft of confidential or proprietary information, we could incur liabilities and the further development of our product candidates could be delayed.

***We face potential liability related to the privacy of health information we obtain from clinical trials sponsored by us.***

Most healthcare providers, including research institutions from which we obtain patient health information, are subject to privacy and security regulations promulgated under HIPAA, as amended by the HITECH. We are not currently classified as a covered entity or business associate under HIPAA and thus are not subject to its requirements or penalties. However, any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information. In addition, we may maintain sensitive personally identifiable information, including health information, that we receive throughout the clinical trial process, in the course of our research collaborations, and directly from individuals (or their healthcare providers) who enroll in our patient assistance programs. As such, we may be subject to state laws requiring notification of affected individuals and state regulators in the event of a breach of personal information, which is a broader class of information than the health information protected by HIPAA. Our clinical trial programs outside the United States may implicate international data protection laws, including the EU Data Protection Directive and legislation of the EU member states implementing it.

Our activities outside the United States impose additional compliance requirements and generate additional risks of enforcement for noncompliance. Failure by our CROs and other third-party contractors to comply with the strict rules on the transfer of personal data outside of the European Union into the United States may result in the imposition of criminal and administrative sanctions on such collaborators, which could adversely affect our business.

Furthermore, certain health privacy laws, data breach notification laws, consumer protection laws and genetic testing laws may apply directly to our operations or those of our collaborators and may impose restrictions on our collection, use and dissemination of individuals' health information. Moreover, patients about whom we or our collaborators obtain health information, as well as the providers who share this information with us, may have statutory or contractual rights that limit our ability to use and disclose the information. We may be required to expend significant capital and other resources to ensure ongoing compliance with applicable privacy and data security laws. Claims that we have violated individuals' privacy rights or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend and could result in adverse publicity that could harm our business.

If we or third-party CMOs, CROs or other contractors or consultants fail to comply with applicable federal, state or local regulatory requirements, we could be subject to a range of regulatory actions that could affect our or our contractors' ability to develop and commercialize our product candidates and could harm or prevent sales of any affected products that we are able to commercialize, or could substantially increase the costs and expenses of developing, commercializing and marketing our products. Any threatened or actual government enforcement action could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Increasing use of social media could give rise to liability, breaches of data security or reputational damage.

***The EU's new data privacy regulation, the General Data Protection Regulation, has taken effect and violations of this could subject us to significant fines.***

In May 2018, a new privacy framework, the General Data Protection Regulation, or the GDPR, took effect in the European Union and became binding across all EU member states. The GDPR is in the process of taking effect in the European Economic Area, or the EEA. The GDPR imposes several stringent requirements for controllers and processors of personal data, particularly with respect to clinical trials. The GDPR provides that EU member states may make their own further laws and regulations limiting the processing of genetic, biometric or health data, which could limit our ability to use and share personal data or could cause our costs to increase and harm our business and financial condition. In addition, the GDPR increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. There are currently a number of legal challenges to the validity of EU mechanisms for adequate data transfers (such as the commonly-used EU-Commission-approved model clauses) or review of these mechanisms (such as the U.S. Privacy Shield), and our work could be impacted by changes in law as a result of a future review of these transfer mechanisms by EU regulators under the GDPR, as well as current challenges to these mechanisms in the EU courts. The GDPR imposes substantial fines for breaches of data protection requirements, which can be up to four percent of global revenue for the preceding financial year or €20 million, whichever is greater, and it also confers a private right of action on data subjects for breaches of data protection requirements. Compliance with EU data protection law is a rigorous and time-intensive process that may increase our cost of doing business, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation and reputational harm in connection with our European activities.

***We and our employees are increasingly utilizing social media tools as a means of communication both internally and externally, and noncompliance with applicable requirements, policies or contracts due to social media use or negative posts or comments could have an adverse effect on our business.***

Despite our efforts to monitor evolving social media communication guidelines and comply with applicable rules, there is risk that the use of social media by us or our employees to communicate about our product candidates or business may cause us to be found in violation of applicable requirements. In addition, our employees may knowingly or inadvertently make use of social media in ways that may not comply with our social media policy or other legal or contractual requirements, which may give rise to liability, lead to the loss of trade secrets or other intellectual property or result in public exposure of personal information of our employees, clinical trial patients, customers and others. Furthermore, negative posts or comments about us or our product candidates in social media could seriously damage our reputation, brand image and goodwill. Any of these events could have a material adverse effect on our business, prospects, operating results and financial condition and could adversely affect the price of our Class A common shares.

***Our employees, principal investigators, CROs, consultants and other third-party service providers may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading.***

We are exposed to the risk that our employees, principal investigators, CROs, consultants and other third-party service providers may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless or negligent conduct or disclosure of unauthorized activities to us that violate the regulations of the FDA and other regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities; healthcare fraud and abuse laws and regulations in the United States and abroad; or laws that require the reporting of financial information or data accurately.

In particular, sales, marketing and 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 promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials or creating fraudulent data in our pre-clinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation.

We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter misconduct by employees and other third parties. 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 comply with these 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, including the imposition of 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, any of which could adversely affect our ability to operate our business and our results of operations.

## Risks Related to Our Common Shares

***The holders of our Class B common shares, which consist primarily of our executive officers and certain other members of our senior management, collectively control over a majority of the combined voting power of our common shares and therefore are able to control all matters submitted to our shareholders for approval. This concentration of ownership of our Class B common shares may have an adverse effect on the price of our Class A common shares and may result in our Class A common shares being undervalued.***

Our Class A1 common shares and Class B1 common shares have no voting rights. As a result, all matters submitted to our shareholders will be decided by the vote of holders of our Class A common shares and Class B common shares. Each Class A common share is entitled to one vote per Class A common share and each Class B common share is entitled to ten votes per Class B common share. As a result of the multi-class voting structure of our common shares, the holders of our Class B common shares, which consist primarily of our executive officers and certain other members of our senior management, collectively control over a majority of the combined voting power of our common shares and therefore are able to control all matters submitted to our shareholders for approval. As of March 1, 2019, the holders of Class A common shares accounted for 28.6% of our aggregate voting power and the holders of Class B common shares accounted for the remaining 71.4% of our aggregate voting power. As a result of the Class A common shares and Class B common shares they hold, as of March 1, 2019, our executive officers and certain other members of our senior management hold 66.6% of our voting power and have the ability to control the outcome of all matters submitted to our shareholders for approval. This concentrated control limits other shareholders' ability to influence corporate matters and may have an adverse effect on the price of our Class A common shares, including our Class A common shares being undervalued. Holders of our Class B common shares collectively control our management and affairs and the outcome of matters submitted to our shareholders for approval, including the election of directors. These holders may have interests, with respect to their investment, that are different from our other shareholders. In addition, this concentration of ownership might adversely affect certain corporate actions that our other shareholders may view as beneficial, for example, by:

- delaying, deferring or preventing a change of control of us;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

However, this percentage may change depending on any conversion of our Class B common shares, Class A1 common shares or Class B1 common shares. Each holder of our Class B1 common shares has the ability to convert any portion of its Class B1 common shares into Class A common shares or Class B common shares at any time, and each holder of our Class A1 common shares has the ability to convert any portion of its Class A1 common shares into Class A common shares at any time. Our Class A1 common shares and Class B1 common shares cannot be converted if, immediately prior to or following such conversion, the holder and its affiliates beneficially own, or would beneficially own, more than 4.99% of our issued and outstanding Class A common shares unless such holders provide us with 61-days' prior notice that they intend to increase, decrease or waive such threshold upon conversion. Due to these

conversion rights, holders of our Class A1 common shares and our Class B1 common shares could, at any time, significantly increase their voting control of us, which could result in their ability to significantly influence or control matters submitted to our shareholders for approval. In addition, the conversion of Class B common shares to Class A or Class B1 common shares will have the effect of increasing the relative voting power of those individual holders of Class B common shares who retain their shares in the long term. In addition, such conversion would decrease the ability of the current holders of our Class B common shares, Class A1 common shares or our Class B1 common shares to significantly influence or control matters submitted to our shareholders for approval.

As of March 1, 2019, entities managed by Baker Brothers held 65.0% of our Class A1 common shares and 100% of our Class B1 common shares. Upon 61-days' prior written notice, these entities could convert their Class A1 common shares and Class B1 common shares into Class A common shares and Class B common shares, which in the aggregate would result in such entities holding over 70% of the voting power of our outstanding share capital.

***The price of our Class A common shares is likely to be volatile and fluctuate substantially, which could result in substantial losses for holders of our Class A common shares.***

Our share price is likely to be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, shareholders may not be able to sell their Class A common shares at or above the price these holders paid for their shares. The market price for our Class A common shares may be influenced by many factors, including:

- the results of clinical trials for our product candidates;
- delays in in-licensing or acquiring additional complementary product candidates;
- delays in the commencement, enrollment and the ultimate completion of clinical trials;
- the results and potential impact of competitive products or technologies;
- our ability to manufacture and successfully produce our product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- the level of expenses related to any of our product candidates or clinical development programs;
- variations in our financial results or those of companies that are perceived to be similar to us;
- financing or other corporate transactions, or inability to obtain additional funding;
- failure to meet or exceed expectations of the investment community;
- regulatory or legal developments in the United States and other countries;
- the recruitment or departure of key personnel;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- changes in the structure of healthcare payment systems;

- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions;
- changes in voting control of our executive officers and certain other members of our senior management or affiliates who hold our shares; and
- the other factors described in this "Risk factors" section.

***If securities or industry analysts cease publishing or publish unfavorable research or reports, about us, our business or our market, our shares price and trading volume could decline.***

The trading market for our Class A common shares is influenced by the research and reports that equity research analysts publish about us and our business. We do not have any control over the analysts or the content and opinions included in their reports. The price of our Class A common shares could decline if one or more equity research analysts downgrades our shares or issues other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our Class A common shares could decrease, which in turn could cause the price of our Class A common shares or its trading volume to decline.

***Sales of a substantial number of our Class A common shares in the public market could cause our share price to fall.***

In connection with our follow-on offering that closed in the first quarter 2019, our directors and executive officers entered into lock-up agreements with the underwriters of the offering, which restrict their ability to sell or transfer their common shares for 90 days from the date of the final prospectus related to our follow-on offering, subject to certain exceptions. If our existing shareholders sell, or indicate an intention to sell, substantial amounts of our Class A common shares in the public market after their lock-ups and other legal restrictions on resale lapse, the market price of our Class A common shares could decline. As of March 1, 2019, we have outstanding a total of 18,639,733 Class A common shares, 4,638,855 Class B common shares, 14,995,954 Class A1 common shares and 16,057,618 Class B1 common shares.

After the lock-up agreements expire, up to an additional 3,740,076 Class A common shares held by our directors and executive officers will be eligible for sale in the public market (including Class A common shares issuable upon the conversion of our Class B common shares). However, these shares will remain subject to certain limitations on sales made by affiliates pursuant to Rule 144 under the Securities Act for so long as the holders are affiliates for purposes of Rule 144.

In addition, our Class B common shares and Class B1 common shares automatically convert into Class A common shares upon transfer to non-affiliates. As a result, as of March 1, 2019, up to 20,696,473 of our Class A common shares may be issued upon such transfers and may become eligible for sale in the public market, subject to any lock-up agreements and Rule 144 under the Securities Act. As of March 1, 2019, there were also 6,026,129 of our Class A common shares subject to outstanding options under our equity incentive plans that may become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, any lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If any of these additional Class A common shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our Class A common shares could decline.

As of March 1, 2019, holders of approximately 37,670,093 Class A common shares (including Class A common shares issuable upon the conversion of our Class A1 common shares, Class B common shares, and Class B1 common shares) are entitled to rights with respect to the registration of their shares under the Securities Act, subject, as applicable, to the lock-up agreements described above. Notwithstanding any of these rights, when we become eligible to use a Form S-3 registration statement, or S-3 eligible, we will be obligated, without request from holders of a requisite number of registrable securities, to file a Form S-3 registration statement covering registrable securities held by all holders who, at the time, would be considered an "affiliate" of the company. We expect to be S-3 eligible early June 2019. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without

restriction under the Securities Act, except for shares purchased by affiliates. Any sales of securities by these shareholders could have a material adverse effect on the market price of our Class A common shares.

***Future sales and issuances of our common shares or rights to purchase common shares, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our shareholders and could cause our Class A common share price to fall.***

We will need additional capital in the future to continue our planned operations. To the extent we raise additional capital by issuing additional Class A common shares, Class B common shares, Class A1 common shares, Class B1 common shares or other equity securities, our shareholders may experience substantial dilution. We may sell common shares, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common shares, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing shareholders, and new investors could gain rights superior to our existing shareholders.

***We are an "emerging growth company" and a "smaller reporting company" and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our Class A common shares less attractive to investors.***

We are an "emerging growth company," as defined in the JOBS Act, and a "smaller reporting company" as defined under the rules promulgated under the Securities Act. As an emerging growth company and a smaller reporting company we may follow reduced disclosure requirements and do not have to make all of the disclosures that public companies that are not emerging growth companies or smaller reporting companies do. We will remain an emerging growth company until the earlier of (a) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (b) the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO; (c) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (d) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, which means the market value of our voting and non-voting common shares that is held by non-affiliates exceeds $700 million as of the prior June 30th. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404;
- an exemption from compliance with the requirement of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on the financial statements;
- progressively adding to the number of years of audited financial statements required to be included in our periodic reports; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation, shareholder approval of any golden parachute payments not previously approved, and having to disclose the ratio of the compensation of our chief executive officer to the median compensation of our employees.

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

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common shares held by non-affiliates is more than

$250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and our voting and non-voting common shares held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide selected financial data, supplemental financial information or risk factors.

We may choose to take advantage of some, but not all, of the available exemptions for emerging growth companies and smaller reporting companies. We cannot predict whether investors will find our Class A common shares less attractive if we rely on these exemptions. If some investors find our Class A common shares less attractive as a result, there may be a less active trading market for our Class A common shares and our shares price may be more volatile.

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

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the Securities and Exchange Commission and The Nasdaq Global Select Market, or Nasdaq, where our Class A common shares are listed, impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly, particularly after we are no longer an emerging growth company and a smaller reporting company. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

***We have anti-takeover provisions in our amended and restated bye-laws that may discourage a change of control.***

Our amended and restated bye-laws contain provisions that could make it more difficult for a third party to acquire us without the consent of our board of directors. These provisions provide for:

- a classified board of directors with staggered three-year terms;
- directors only to be removed for cause;
- an affirmative vote of $66^{2}/_{3}\%$ of the voting power of our voting shares for certain "business combination" transactions that have not been approved by our board of directors;

- our multi-class common share structure, which provides our holders of Class B common shares with the ability to significantly influence the outcome of matters requiring shareholder approval, even if they own less than a majority of our outstanding Class A common shares, Class B common shares, Class A1 common shares, and Class B1 common shares;

- restrictions on the time period in which directors may be nominated; and

- our board of directors to determine the powers, preferences and rights of our preferred shares and to issue the preferred shares without shareholder approval.

These anti-takeover defenses could discourage, delay or prevent a transaction involving a change in control of our company and may prevent our shareholders from receiving the benefit from any premium to the market price of our Class A common shares offered by a bidder in a takeover context. Even in the absence of a takeover attempt, the existence of these provisions may adversely affect the prevailing market price of our Class A common shares if the provisions are viewed as discouraging takeover attempts in the future. These provisions could also discourage proxy contests, make it more difficult for our shareholders to elect directors of their choosing and cause us to take corporate actions other than those our shareholders desire.

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

We have never declared or paid cash dividends on our shares. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. Additionally, the proposal to pay future dividends to shareholders will effectively be at the sole discretion of our board of directors after taking into account various factors our board of directors deems relevant, including our business prospects, capital requirements, financial performance and new product development. As a result, capital appreciation, if any, of our Class A common shares will be the sole source of gain for our shareholders for the foreseeable future.

**Risks Related to Owning Shares in a Bermuda Exempted Company and Certain Tax Risks**

***We are a Bermuda company and it may be difficult for our shareholders to enforce judgments against us or our directors and executive officers.***

We are a Bermuda exempted company. As a result, the rights of holders of our Class A common shares will be governed by Bermuda law and our memorandum of association and amended and restated bye-laws. The rights of shareholders under Bermuda law may differ from the rights of shareholders of companies incorporated in other jurisdictions. It may be difficult for investors to enforce in the United States judgments obtained in U.S. courts against us based on the civil liability provisions of the U.S. securities laws. It is doubtful whether courts in Bermuda will enforce judgments obtained in other jurisdictions, including the United States, against us or our directors or officers under the securities laws of those jurisdictions or entertain actions in Bermuda against us or our directors or officers under the securities laws of other jurisdictions.

***Our amended and restated bye-laws designate the Supreme Court of Bermuda as the choice of jurisdiction for disputes that arise concerning the Bermuda Companies Act 1981, as amended, or out of or in connection with our amended and restated bye-laws, which could limit our shareholders' ability to choose the judicial forum for disputes with us or our directors or officers.***

Our amended and restated bye-laws provide that, unless we consent in writing to the selection of an alternative jurisdiction, any dispute that arises concerning the Bermuda Companies Act 1981, as amended, or the Companies Act, or out of or in connection with our bye-laws, including any question regarding the existence and scope of any bye-law or whether there has been a breach of the Companies Act or the bye-laws by any of our officers or directors (whether or not such a claim is brought in the name of a shareholder or in the name of our company) shall be subject to the jurisdiction of the Supreme Court of Bermuda.

Any person or entity purchasing or otherwise acquiring any interest in any of our shares shall be deemed to have notice of and consented to this provision. This choice of jurisdiction provision may limit a shareholder's ability to bring a claim in a judicial forum of its choosing for disputes with us or our directors or officers, which may discourage lawsuits against us and our directors and officers. If a court were to find either choice of jurisdiction provision in our amended and restated bye-laws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could harm our results of operations.

***Bermuda law differs from the laws in effect in the United States and may afford less protection to our shareholders.***

We are organized under the laws of Bermuda. As a result, our corporate affairs are governed by the Companies Act, which differs in some material respects from laws typically applicable to U.S. corporations and shareholders, including the provisions relating to interested directors, amalgamations, mergers and acquisitions, takeovers, shareholder lawsuits and indemnification of directors. Generally, the duties of directors and officers of a Bermuda company are owed to the company only. Shareholders of Bermuda companies typically do not have rights to take action against directors or officers of the company and may only do so in limited circumstances. Shareholder class actions are not available under Bermuda law. The circumstance in which shareholder derivative actions may be available under Bermuda law are substantially more proscribed and less clear than they would be to shareholders of U.S. corporations. The Bermuda courts, however, would ordinarily be expected to permit a shareholder to commence an action in the name of a company to remedy a wrong to the company where the act complained of is alleged to be beyond the corporate power of the company or illegal, or would result in the violation of the company's memorandum of association or bye-laws. Furthermore, consideration would be given by a Bermuda court to acts that are alleged to constitute a fraud against the minority shareholders or, for instance, where an act requires the approval of a greater percentage of the company's shareholders than those who actually approved it.

When the affairs of a company are being conducted in a manner that is oppressive or prejudicial to the interests of some shareholders, one or more shareholders may apply to the Supreme Court of Bermuda, which may make such order as it sees fit, including an order regulating the conduct of the company's affairs in the future or ordering the purchase of the shares of any shareholders by other shareholders or by the company. Additionally, under our amended and restated bye-laws and as permitted by Bermuda law, each shareholder has waived any claim or right of action against our directors or officers for any action taken by directors or officers in the performance of their duties, except for actions involving fraud or dishonesty. In addition, the rights of our shareholders and the fiduciary responsibilities of our directors under Bermuda law are not as clearly established as under statutes or judicial precedent in existence in jurisdictions in the United States, particularly the State of Delaware. Therefore, our shareholders may have more difficulty protecting their interests than would shareholders of a corporation incorporated in a jurisdiction within the United States.

***There are regulatory limitations on the ownership and transfer of our common shares.***

Common shares may be offered or sold in Bermuda only in compliance with the provisions of the Companies Act and the Bermuda Investment Business Act 2003, as amended, which regulates the sale of securities in Bermuda. In addition, the Bermuda Monetary Authority must approve all issues and transfers of shares of a Bermuda exempted company. However, the Bermuda Monetary Authority has, pursuant to its statement of June 1, 2005, given its general permission under the Exchange Control Act 1972 and related regulations for the issue and free transfer of our common shares to and among persons who are non-residents of Bermuda for exchange control purposes as long as the shares are listed on an appointed shares exchange, which includes Nasdaq. This general permission would cease to apply if we were to cease to be listed on Nasdaq.

***We may become subject to unanticipated tax liabilities.***

Although we are incorporated under the laws of Bermuda, we may become subject to income, withholding or other taxes in certain jurisdictions by reason of our activities and operations, and it is also possible that taxing authorities in any such jurisdictions could assert that we are subject to greater taxation than we currently anticipate. Any such non-Bermudan tax liability could materially adversely affect our results of operations.

***Taxing authorities could reallocate our taxable income among our subsidiaries, which could increase our overall tax liability.***

We are incorporated under the laws of Bermuda and currently have a subsidiary in the United States. If we succeed in growing our business, we expect to conduct increased operations through our subsidiaries in various tax jurisdictions pursuant to transfer pricing arrangements between us and our subsidiaries. If two or more affiliated companies are located in different countries, the tax laws or regulations of each country generally will require that transfer prices be the same as those between unrelated companies dealing at arms' length and that appropriate documentation is maintained to support the transfer prices. While we believe that we operate in compliance with applicable transfer pricing laws and intend to continue to do so, our transfer pricing procedures are not binding on applicable tax authorities.

If tax authorities in any of these countries were to successfully challenge our transfer prices as not reflecting arms' length transactions, they could require us to adjust our transfer prices and thereby reallocate our income to reflect these revised transfer prices, which could result in a higher tax liability to us. In addition, if the country from which the income is reallocated does not agree with the reallocation, both countries could tax the same income, resulting in double taxation. If tax authorities were to allocate income to a higher tax jurisdiction, subject our income to double taxation or assess interest and penalties, it would increase our consolidated tax liability, which could adversely affect our financial condition, results of operations and cash flows.

***Changes in laws related to tax practices and substance requirements in Bermuda and other jurisdictions could adversely affect our operations.***

Our tax position could be adversely impacted by changes in tax rates, tax laws, tax practice, tax treaties or tax regulations or changes in the interpretation thereof by the tax authorities in Europe (including the United Kingdom), the United States, Bermuda and other jurisdictions, as well as being affected by certain changes currently proposed by the Organization for Economic Co-operation and Development and their action plan on Base Erosion and Profit Shifting. Such changes may become more likely as a result of recent economic trends in the jurisdictions in which we operate, particularly if such trends continue. If such a situation was to arise, it could adversely impact our tax position and our effective tax rate. Failure to manage the risks associated with such changes, or misinterpretation of the laws providing such changes, could result in costly audits, interest, penalties and reputational damage, which could adversely affect our business, results of our operations and our financial condition.

Our actual effective tax rate may vary from our expectation and that variance may be material. A number of factors may increase our future effective tax rates, including:

- the jurisdictions in which profits are determined to be earned and taxed;
- the resolution of issues arising from any future tax audits with various tax authorities;
- changes in the valuation of our deferred tax assets and liabilities;
- increases in expenses not deductible for tax purposes, including transaction costs and impairments of goodwill in connection with acquisitions;
- changes in the taxation of share-based compensation;
- changes in tax laws or the interpretation of such tax laws, and changes in generally accepted accounting principles; and
- challenges to the transfer pricing policies related to our structure.

In late 2017, the European Union ("EU") Economic and Financial Affairs Council ("ECOFIN") released a list of non-cooperative jurisdictions for tax purposes. The stated aim of this list, and accompanying report, was to promote the EU's view for good governance worldwide in order to maximize efforts to prevent tax fraud and tax evasion. While Bermuda was not on the original EU list of non-cooperative jurisdictions, it committed to address EU concerns relating to economic substance by December 31, 2018. In accordance with that commitment, Bermuda enacted the Economic Substance Act 2018 (the "Substance Act") requiring certain entities in Bermuda engaged in "relevant activities" to maintain a substantial economic presence in Bermuda and to satisfy economic substance requirements commencing as of July 1, 2019. The list of "relevant activities" includes carrying on as a business in any one or more of: banking, insurance, fund management, financing, leasing, headquarters, shipping, distribution and service center, intellectual property and holding entities. Under the Substance Act, any entity that must satisfy economic substance requirements but fails to do so could face automatic disclosure to competent authorities in the EU of the information filed by the entity with the Bermuda Registrar of Companies in connection with the economic substance requirements and may also face financial penalties, restriction or regulation of its business activities or may be struck as a registered entity in Bermuda. On March 12, 2019, ECOFIN announced the addition of 10 new jurisdictions to its list of non-cooperative jurisdictions for tax purposes, including Bermuda. The Premier of Bermuda subsequently announced that Bermuda is committed to being removed from this list at the earliest opportunity. The impact of the foregoing developments is unclear, including whether additional or revised requirements may be enacted by Bermuda in response to being added to the EU's list of non-cooperative jurisdictions for tax purposes. Accordingly, we cannot predict the nature and effect of Bermuda being added to the EU's list of non-cooperative jurisdictions for tax purposes or Bermuda's current or future economic substance requirements on our business, all of which may impact the manner and jurisdictions in which we operate, which could adversely affect our business, financial condition or results of operations.

***We believe we will likely be classified as a passive foreign investment company for U.S. federal income tax purposes for the year ended December 31, 2018, which could result in adverse U.S. federal income tax consequences to U.S. investors in our common shares.***

Because we did not earn revenue from our business operations during the year ended December 31, 2018, and because our sole source of income currently is interest on bank accounts held by us, we believe we will likely be classified as a "passive foreign investment company," or PFIC, for the taxable year ended December 31, 2018. A non-U.S. company will be considered a PFIC for any taxable year if (i) at least 75% of its gross income is passive income (including interest income), or (ii) at least 50% of the value of its assets (based on an average of the quarterly values of the assets during a taxable year) is attributable to assets that produce or are held for the production of passive income. If we are classified as a PFIC in any year with respect to which a U.S. Holder owns our common shares, we will continue to be treated as a PFIC with respect to such U.S. Holder in all succeeding years during which the U.S. Holder owns the common shares, regardless of whether we continue to meet the PFIC test described above, unless the U.S. Holder makes or has made a specified election and we cease to be a PFIC. A "U.S. Holder" is a beneficial owner of our Class A common shares that, for U.S. federal income tax purposes, is or is treated as any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation (or another entity taxable as a corporation) created or organized under the laws of the United States, any state thereof, or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust that (1) is subject to the supervision of a U.S. court and the control of one or more "United States persons" (within the meaning of Section 7701(a)(30) of the U.S. Internal Revenue Code of 1986, as amended), or (2) has a valid election in effect to be treated as a United States person for U.S. federal income tax purposes.

If we are classified as a PFIC for any taxable year during which a U.S. Holder holds our common shares, certain adverse U.S. federal income tax consequences could apply to such U.S. Holder, including (i) the treatment of all or a portion of any gain on disposition as ordinary income, (ii) the application of a deferred interest charge on such gain and the receipt of certain dividends and (iii) the obligation to comply with certain reporting requirements.

***If a U.S. person is treated as owning at least 10% of our common shares, such holder may be subject to adverse U.S. federal income tax consequences.***

We believe we are classified as a controlled foreign corporation for the taxable year ended December 31, 2018. Even if we were not classified as a controlled foreign corporation, if our group includes one or more U.S. subsidiaries, certain of our non-U.S. subsidiaries could be treated as controlled foreign corporations. If a U.S. Holder is treated as owning (directly, indirectly or constructively) at least 10% of the value or voting power of our common shares, such U.S. Holder may be treated as a "United States shareholder" with respect to us (if we are classified as a controlled foreign corporation) and each controlled foreign corporation in our group (if any). A United States shareholder of a controlled foreign corporation may be required to annually report and include in its U.S. taxable income its pro rata share of "Subpart F income," "global intangible low-taxed income," or GILTI, and investments in U.S. property by controlled foreign corporations, regardless of whether we make any distributions. An individual that is a United States shareholder with respect to a controlled foreign corporation generally would not be allowed certain tax deductions or foreign tax credits that would be allowed to a United States shareholder that is a U.S. corporation. Failure to comply with these reporting obligations may subject such shareholder to significant monetary penalties and may prevent the statute of limitations with respect to such shareholder's U.S. federal income tax return for the year for which reporting was due from starting. We cannot provide any assurances that we will assist investors in determining whether such investor is treated as a United States shareholder with respect us or any of our non-U.S. subsidiaries. Further, we cannot provide any assurances that we will furnish to any United States shareholders information that may be necessary to comply with the reporting and tax paying obligations discussed above. U.S. Holders should consult their tax advisors regarding the potential application of these rules to any investment in our common shares.

***Comprehensive tax reform legislation could adversely affect our business and financial condition.***

In 2017, the U.S. government has enacted comprehensive tax legislation that includes significant changes to the taxation of business entities, referenced herein as the Tax Reform Act. These changes include, among others, a permanent reduction to the corporate income tax rate, introduction of the GILTI provision, limiting interest deductions, adopting elements of a territorial tax system and introducing certain anti-base erosion provisions. We continue to examine the impact this tax reform legislation may have on our business. The effect of the Tax Reform Act on our business, whether adverse or favorable, is uncertain, and may not become evident for some period of time. U.S. Holders should consult with their legal and tax advisors regarding any such legislation and the potential tax consequences of investing in our common shares.

## ITEM 1B. UNRESOLVED STAFF COMMENTS.

None.

## ITEM 2. PROPERTIES.

Our U.S. headquarters are located in Lexington, Massachusetts, where Kiniksa US has leased approximately 55,924 square feet of office and laboratory space, under a lease which expires in July 2021. Kiniksa US has also leased approximately 4,400 square feet of office space in San Diego, California which expires in December 2020. We believe that our offices are sufficient to meet our current needs and that suitable additional space will be available as and when needed.

## ITEM 3. LEGAL PROCEEDINGS.

We are not party to any material legal proceedings.

## ITEM 4. MINE SAFETY DISCLOSURES.

Not applicable.

## EXECUTIVE OFFICER AND DIRECTOR BIOGRAPHIES

***Directors of the Registrant***

*Sanj K. Patel* has served as our Chief Executive Officer and Chairman of our Board of Directors since our formation in July 2015. In June 2008, Mr. Patel formed Synageva BioPharma Corp., or Synageva, a biotechnology company focused on rare diseases, where he acted as President and Chief Executive Officer until its sale to Alexion Pharmaceuticals, Inc., or Alexion, in June 2015. Prior to Synageva, Mr. Patel held various roles at Genzyme Corporation, or Genzyme, from 1999 to 2008, most recently as head of U.S. Sales, Marketing and Commercial Operations for the Genzyme Therapeutics franchise. Mr. Patel is a member of the board of directors of Syros Pharmaceuticals and BioCryst Pharmaceuticals, and, from 2013 to 2015, sat on the board of directors of Intercept Pharmaceuticals. He is also the founder and director of the Sanj K. Patel and Family Foundation, a philanthropic organization that supports charities for patients with rare and devastating diseases. Mr. Patel holds a B.Sc. with Honors from the University of the South Bank, London and completed his management and business studies at Ealing College, London and his Pharmacology research program at the Wellcome Foundation.

*Felix J. Baker, Ph.D.*, has served as our lead director and on our Board of Directors since October 2015. Dr. Baker is Co-Managing Member of Baker Bros. Advisors LP, or Baker Brothers, a registered investment adviser focused on long-term investments in life-sciences companies. Dr. Baker and his brother, Julian Baker, started their fund management careers when they co-founded a biotechnology investing partnership with the Tisch Family. In 2000, they founded Baker Brothers. Dr. Baker serves on the boards of directors of Alexion, Genomic Health, Inc., Seattle Genetics, Inc., and Kodiak Sciences Inc. and previously served on the board of directors of Synageva. Dr. Baker holds a B.S. and a Ph.D. in Immunology from Stanford University, where he also completed two years of medical school.

*Stephen R. Biggar, M.D., Ph.D.*, has served as a member of our Board of Directors since October 2015. Dr. Biggar is a Partner at Baker Brothers. Dr. Biggar joined Baker Brothers in 2000. Dr. Biggar is currently Chairman of the board of directors of ACADIA Pharmaceuticals Inc. and previously served on the board of directors of Synageva. Dr. Biggar received an M.D. and a Ph.D. in Immunology from Stanford University and a B.S. in Genetics from the University of Rochester.

*Richard S. Levy, M.D.* has served on our Board of Directors since March 2019. Dr. Levy has been a Senior Advisor at Baker Brothers since December 2016. Prior to joining Baker Brothers, Dr. Levy served as Executive Vice President and Chief Drug Development Officer at lncyte Corporation, a biopharmaceutical company, from January 2009 until June 2018, and as Senior Vice President of Drug Development from August 2003 to January 2009. Dr. Levy serves on the Boards of Director of Madrigal Pharmaceuticals, Inc., Aquinox Pharmaceuticals, Inc. and Kodiak Sciences, Inc. Dr. Levy is Board Certified in Internal Medicine and Gastroenterology and holds an A.B. in Biology from Brown University and an M.D. from the University of Pennsylvania School of Medicine, and completed his training in Internal Medicine at the Hospital of the University of Pennsylvania and a fellowship in Gastroenterology and Hepatology at UCLA.

*Thomas R. Malley* has served as a member of our Board of Directors since December 2016. Since May 2007, Mr. Malley has served as the President of Mossrock Capital, LLC, a private investment firm. Mr. Malley serves on the boards of directors of BeiGene, Ltd. and Kura Oncology, Inc., and previously served on the boards of directors of OvaScience, Inc., Cougar Biotechnology, Puma Biotechnology and Synageva. Mr. Malley holds a B.S. degree in Biology from Stanford University.

*Tracey L. McCain* has served as a member of our Board of Directors since February 2018. Since September 2016, Ms. McCain has served as Executive Vice President and Chief Legal and Compliance Officer of Blueprint Medicine Corporation, or Blueprint, a biotechnology company. Prior to Blueprint, from January 2016 to September 2016, Ms. McCain was Senior Vice President and Head of Legal for Sanofi Genzyme, a global business unit of Sanofi S.A., or Sanofi. From May 1997 to September 2016, Ms. McCain held various roles at Genzyme, including as General Counsel following Genzyme's acquisition by Sanofi in 2011. Ms. McCain holds a J.D. from Columbia University School of Law and a B.A. from the University of Pennsylvania.

*Kimberly J. Popovits* has served as a member of our Board of Directors since February 2018. Since 2009, Ms. Popovits has served as the Chief Executive Officer of Genomic Health, Inc., and since 2012, has served as the Chairman of the board of directors. Ms. Popovits also serves on the board of directors of MyoKardia, Inc., and previously served on the board of directors of ZS Pharma Inc. Ms. Popovits holds a B.A in Business from Michigan State University.

*Barry D. Quart, Pharm.D.*, has served as a member of our Board of Directors since October 2015. Since 2013, Dr. Quart has served as the Chief Executive Officer and on the board of directors of Heron Therapeutics, Inc., a biotechnology company. In 2006, Dr. Quart co-founded Ardea Biosciences, Inc., a biotechnology company, and served as its President and Chief Executive Officer, and on its board of directors, from its inception through May 2013. Dr. Quart previously served on the board of directors of Synageva. Dr. Quart holds a Pharm.D. degree from the University of California, San Francisco.

***Executive Officers of the Registrant***

*Sanj K. Patel* has served as our Chief Executive Officer and Chairman of our Board of Directors since our formation in July 2015. See "—Directors of the Registrant" for Mr. Patel's biography.

*Thomas Beetham* has served as our Chief Legal Officer since our formation in July 2015 and is also responsible for corporate development. Prior to serving as our Chief Legal Officer, Mr. Beetham was the Chief Legal Officer and Senior Vice President of Corporate Development for Synageva from October 2013 to June 2015. At Synageva, in addition to leading the legal department, Mr. Beetham was responsible for business development activities. Prior to joining Synageva, from October 2011 to October 2013, Mr. Beetham was the General Legal Counsel for New England Biolabs, Inc., or Biolabs, a reagent supplier for genomic research, where he was responsible for legal matters and a member of Biolabs' global business development team. Before Synageva, Mr. Beetham was at Genzyme from September 2004 to October 2011, most recently as the lead corporate attorney responsible for Genzyme's hematology/oncology and multiple sclerosis products, and from September 1999 to September 2004 was a corporate and transactional attorney with the law firm of Palmer & Dodge, LLP. Mr. Beetham holds an M.B.A. from Boston College's Carroll School of Management, a J.D. from Boston College Law School and a B.A. from the University of Rochester.

*Chris Heberlig* has served as our Chief Financial Officer since our formation in July 2015. Prior to serving as our Chief Financial Officer, Mr. Heberlig held various roles at Synageva from 2008 to 2015, most recently serving as Senior Vice President of Finance and Business Operations. At Synageva, he led strategic tax planning, including overseeing the transfer of tax and intellectual property assets to Europe, and was responsible for global financial operations, facilities, as well as program management. Mr. Heberlig holds an M.B.A. from Boston University School of Management and a B.A. from St. Lawrence University. Mr. Heberlig is also a Certified Public Accountant.

*Stephen Mahoney* has served as our Chief Operating Officer since our formation in July 2015 and as our President since June 2017. Prior to serving as our Chief Operating Officer, Mr. Mahoney held various roles at Synageva from 2012 to 2015, most recently as Chief Commercial Officer, where he was responsible for Synageva's global commercial operations. Mr. Mahoney was also responsible for areas such as Global Sales Operations & Business Analytics, Commercial Supply Chain and Logistics, Global Procurement, Patient Services, Sales Training and Legal and Corporate Development. Prior to Synageva, Mr. Mahoney held various roles at Genzyme from 2003 to 2012, most recently as the Regional Legal Director for the Asia Pacific region, where he was responsible for legal and healthcare compliance issues for multiple business units. Mr. Mahoney holds an M.B.A. from Boston College's Carroll School of Management, a J.D. from Boston College Law School and a B.A. from Colorado College.

*John F. Paolini, M.D., Ph.D.*, has served as our Chief Medical Officer since August 2016. From August 2015 to August 2016, Dr. Paolini was Clinical Research Head of the Cardiovascular and Metabolic Diseases Research Unit at Pfizer Inc., a pharmaceutical company, where he was responsible for bringing forward programs from pre-clinical through early clinical development and proof of concept. Prior to Pfizer, from August 2011 to July 2015, Dr. Paolini served as Chief Medical Officer of Cerenis Therapeutics, a biotechnology company focused on cardiovascular and metabolic diseases, where he was responsible for designing and executing clinical trials and regulatory strategy for a portfolio of products. Dr. Paolini holds an M.D., Ph.D. from Duke University School of Medicine, a B.A. and a B.S. from Tulane University, and completed his internship, residency and fellowship in Internal Medicine and Cardiology at Brigham and Women's Hospital, Boston.

## PART II

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

### *Principal Market*

Our Class A common shares are listed on The Nasdaq Global Select Market under the symbol "KNSA."

### *Holders*

As of March 1, 2019, there were 26 holders of record of our Class A common shares, 12 holders of record of our Class B common shares, four holders of record of our Class A1 common shares and two holders of record of our Class B1 common shares. The actual number of shareholders is greater than this number of record holders, and includes shareholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. The number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

### *Dividends Policy*

We have never declared or paid any cash dividends on our common shares. We intend to retain all of our future earnings, if any, to finance the operation and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future. Any future determination to declare and pay dividends to holders of our common shares will be made at the discretion of our board of directors, which may take into account several factors, including general economic conditions, our financial condition and results of operations, available cash and current and anticipated cash needs, capital requirements, contractual, legal, tax and regulatory restrictions, the implications of the payment of dividends by us to our shareholders and any other factors that our board of directors may deem relevant. In addition, pursuant to the Bermuda Companies Act 1981, as amended, a company may not declare or pay dividends if there are reasonable grounds for believing that (1) the company is, or would after the payment be, unable to pay its liabilities as they become due or (2) that the realizable value of its assets would thereby be less than its liabilities. Under our amended and restated bye-laws, each of our common shares is entitled to dividends if, as and when dividends are declared by our board of directors, subject to any preferred dividend right of the holders of any preferred shares.

### *Recent Sales of Unregistered Securities*

In February 2018, we issued and sold an aggregate of 12,784,601 Series C preferred shares to new investors, existing shareholders and certain executive officers at a price of $15.6438 per share, resulting in aggregate gross proceeds to us of approximately $200.0 million. Each Series C preferred share converted into one Class A common share or, at the holder's election, one Class A1 common share upon the closing of the initial public offering of our Class A common shares, or the IPO. These securities were issued under Section 4(a)(2) of the Securities Act in a transaction not involving a public offering.

On February 4, 2019, we issued and sold an aggregate of 2,000,000 Class A1 common shares to existing investors at price of $18.26 per share, resulting in aggregate gross proceeds to us of approximately $36.5 million. These securities were issued under Section 4(a)(2) of the Securities Act in a transaction not involving a public offering.

On March 8, 2019, in connection with our acquisition of the securities, or Securities, of Primatope Therapeutics, Inc., or Primatope, we issued an aggregate of 337,008 Class A common shares to the holders of all of the issued and outstanding Securities, having an aggregate value of approximately $5.9 million, as payment, in part, for (a) the Securities and (b) the achievement of certain milestones at or before the closing of the acquisition. These securities were issued under Section 4(a)(2) of the Securities Act in a transaction not involving a public offering.

*Use of Proceeds from Registered Securities*

On May 29, 2018, we issued and sold 8,477,777 Class A common shares to the underwriters of our IPO and on June 22, 2018, we issued and sold an additional 1,006,425 Class A common shares pursuant to the exercise by the underwriters of their over-allotment option to purchase additional shares. Our Class A common shares were sold at a price to the public of $18.00 per share. We received aggregate gross proceeds from the IPO inclusive of the underwriters' over-allotment option of approximately $170.7 million and aggregate net proceeds of approximately $155.5 million after deducting underwriting discounts and commissions of approximately $12.0 million and other offering expenses. The offer and sale of all of the shares in our IPO inclusive of the underwriters' over-allotment option were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-224488), which was declared effective by the Securities and Exchange Commission on May 23, 2018, and a registration statement on Form S-1 to register additional shares (File No. 333-225159), which was automatically effective upon filing with the Securities and Exchange Commission on May 23, 2018. There has been no material change in our planned use of the net proceeds from the IPO as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the Securities and Exchange Commission on May 24, 2018.

## ITEM 6. SELECTED FINANCIAL DATA.

### SELECTED CONSOLIDATED FINANCIAL DATA

You should read the following selected consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Annual Report on Form 10-K. We have derived the consolidated statement of operations data for the years ended December 31, 2018 and 2017 and the consolidated balance sheet data as of December 31, 2018 and 2017 from our audited consolidated financial statements appearing at the end of this Annual Report on Form 10-K. Our historical results are not necessarily indicative of results that may be expected in any future period.

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| | (in thousands, except share and per share data) | |
| **Consolidated Statement of Operations Data:** | | |
| Operating expenses: | | |
| Research and development | $ 86,597 | $ 56,357 |
| General and administrative | 21,563 | 9,043 |
| Total operating expenses | 108,160 | 65,400 |
| Loss from operations | (108,160) | (65,400) |
| Interest income | 4,719 | 529 |
| Loss before benefit (provision) for income taxes | (103,441) | (64,871) |
| Benefit (provision) for income taxes | 214 | (2) |
| Net loss | $ (103,227) | $ (64,873) |
| Net loss per share attributable to common shareholders—basic and diluted[(1)] | $ (3.49) | $ (35.85) |
| Weighted average common shares outstanding—basic and diluted[(1)] | 29,547,427 | 1,809,751 |

(1) See Note 11 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K for further details on the calculation of basic and diluted net loss per share attributable to common shareholders.

| | As of December 31, | |
|---|---|---|
| | 2018 | 2017 |
| **Consolidated Balance Sheet Data:** | (in thousands) | |
| Cash , cash equivalents and short term investments | $ 307,304 | $ 45,555 |
| Working capital[1] | 271,196 | 29,674 |
| Total assets | 321,965 | 47,492 |
| Convertible preferred shares | — | 119,770 |
| Accumulated deficit | (194,225) | (90,998) |
| Total shareholders' equity (deficit) | 279,267 | (89,708) |

(1) We define working capital as current assets less current liabilities.

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

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, that involve risks and uncertainties. As a result of many factors, including those factors set forth in the risks identified in Part I-Item 1A "Risk Factors'' section of this Annual Report on Form 10-K and our other filings with the Securities and Exchange Commission, or SEC, our actual results could differ materially from the results, performance or achievements expressed in or implied by these forward-looking statements.*

### Overview

We are a biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients suffering from debilitating diseases with significant unmet medical need. We have a pipeline of product candidates across various stages of development, focused on autoinflammatory and autoimmune conditions. We believe that our product candidates are grounded in strong biologic rationales or validated mechanisms of action and have the potential to address multiple indications.

Our product candidates include rilonacept, mavrilimumab, KPL-716, KPL-404 and KPL-045.

Our lead candidate is rilonacept, an interleukin-1α, and interleukin-1β, cytokine trap. We are developing rilonacept for the potential treatment of recurrent pericarditis, an inflammatory cardiovascular disease and are enrolling a single, pivotal, placebo-controlled, randomized-withdrawal design, global Phase 3 clinical trial of rilonacept in subjects with recurrent pericarditis, named RHAPSODY. We also have an ongoing open-label Phase 2 proof-of-concept clinical trial in subjects with both symptomatic recurrent pericarditis as well as other patient subsets within pericarditis, including asymptomatic steroid-dependent subjects with recurrent pericarditis and subjects with post-pericardiotomy syndrome. We completed enrollment in this trial and presented interim data from this trial in December 2018. We expect to present additional data at the American College of Cardiology 68th Annual Scientific Session & EXPO 2019 in March 2019.

Mavrilimumab is a monoclonal antibody that antagonizes colony stimulating factor. We are evaluating mavrilimumab for the potential treatment of giant cell arteritis, or GCA, an inflammatory disease of the blood vessels. We have commenced dosing in multiple countries in a randomized, double-blind, placebo-controlled, Phase 2 proof-of-concept trial for the study of mavrilimumab in GCA. In the United States, the FDA initially placed our IND for the trial on clinical hold due to its request for additional information regarding the 510(k)-cleared delivery device to be used in our trial. We have since provided the FDA with the requested information and our IND is now active. We plan for U.S. subjects to be included in our ongoing, global Phase 2 proof-of-concept clinical trial.

KPL-716 is a monoclonal antibody that simultaneously inhibits the signaling of the cytokines interleukin 31, or IL 31, and oncostatin M, or OSM, by targeting their common receptor subunit, oncostatin M receptor beta. We plan to study KPL-716 in a variety of pruritic, inflammatory, and fibrotic indications driven by these cytokines. In September 2018, we announced interim results from the randomized, double-blind, placebo-controlled, single-ascending-dose, sequential-group portion of the Phase 1a/1b clinical trial in healthy volunteers and in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus. We are enrolling a 12-week, repeated single-dose cohort as an additional part of the Phase 1b portion of the Phase 1a/1b clinical trial in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus. We expect to report top-line data from this cohort in the second half of 2019. We plan to initiate an adaptive design Phase 2a/2b clinical trial in prurigo nodularis in the first half of 2019 and expect to report top-line data from the first part of this trial in the first half of 2020. We also plan to initiate an exploratory, pilot Phase 2 clinical trial in the first half of 2019 designed to explore the role of IL-31 and OSM in a number of diseases characterized by chronic pruritus and plan to report top-line data from this trial in the second half of 2020.

KPL-404 is a monoclonal antibody inhibitor of the CD40 co-stimulatory molecule. We are continuing our pre-clinical activities with KPL-404 in T-cell dependent, B-cell mediated diseases, and expect to file an IND with the FDA for this program in the second half of 2019 and initiate a Phase 1 clinical trial in the first half of 2020. In September 2017, we licensed the right to conduct research and development on KPL-404 from Primatope Therapeutics, Inc., or Primatope, the company that owned or controlled the intellectual property related to KPL-404. In March 2019, we acquired all of the issued and outstanding equity securities of Primatope in exchange for upfront consideration of $10.0 million at closing as well as milestone payments of up to $8.0 million ($5.0 million of which had been achieved as of the closing date and was paid at closing), each paid or payable in a combination of cash and our Class A common shares (inclusive of escrow and holdback amounts) in accordance with the terms and conditions of our stock purchase option agreement with Primatope.

KPL-045, is a monoclonal antibody inhibitor of the CD30L co-stimulatory molecule. We are evaluating the progression of KPL-045 pending preclinical data from the program in the context of our portfolio.

Since our inception in 2015, we have devoted substantially all of our efforts and financial resources to organizing and staffing our company, business planning, raising capital, acquiring, in-licensing or discovering product candidates and securing related intellectual property rights and conducting research and development activities for our programs. We do not have any products approved for sale and have not generated any revenue from product sales. We may never be able to develop or commercialize a marketable product. We have not yet successfully completed any Phase 3 or other pivotal clinical trials, obtained any regulatory approvals, manufactured a commercial-scale drug, or conducted sales and marketing activities. Prior to the completion of our initial public offering, or IPO, in May 2018, we had funded our operations primarily with proceeds from the sale of preferred shares, from which we received net proceeds of $310.6 million.

On May 23, 2018, our registration statement on Form S-1 relating to our IPO was declared effective by the SEC. On May 29, 2018, we completed our IPO of 8,477,777 Class A common shares at $18.00 per share for gross proceeds of $152.6 million. In addition, on June 22, 2018, we completed the sale of 1,006,425 Class A common shares to the underwriters of the IPO following the exercise of their over-allotment option to purchase additional shares at $18.00 per share for gross proceeds of $18.1 million. The aggregate net proceeds to us from the IPO, inclusive of the over-allotment option exercise, was $155.5 million after deducting underwriting discounts and commissions and other offering costs.

Upon the closing of the IPO, all convertible preferred shares then outstanding automatically converted into 5,546,019 Class A common shares, 1,070,502 Class B common shares, 12,995,954 Class A1 common shares and 16,057,618 Class B1 common shares.

On February 4, 2019, we completed a follow-on offering of 2,654,984 Class A common shares and concurrent private placement of 2,000,000 Class A1 common shares, both at $18.26 per share for aggregate gross proceeds of $85.0 million. In addition, on March 1, 2019, we completed the sale of 161,126 Class A common shares to the underwriters of the follow-on offering following the exercise in part of their over-allotment option to purchase additional shares at $18.26 per share for gross proceeds of $2.9 million. The aggregate net proceeds to us from the follow-on offering and concurrent private placement, inclusive of the over-allotment option exercise, was $83.0 million after deducting underwriting discounts and commissions and other offering costs.

We have incurred significant operating losses since inception. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our current or future product candidates. Our net losses were $103.2 million and $64.9 million for the years ended December 31, 2018 and 2017, respectively. As of December 31, 2018 and 2017, we had an accumulated deficit of $194.2 million and $91.0 million, respectively. We expect to continue to incur significant operating losses for at least the next several years as we advance our product candidates through all stages of development and clinical trials and, ultimately, seek regulatory approval. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. We may also incur expenses in connection with the in-licensing or acquisition of additional product

candidates. We expect to continue to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the sale of equity, debt financings or other capital sources, which may include collaborations with other companies or other strategic transactions. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as and when needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions.

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

As of December 31, 2018 and 2017, we had cash, cash equivalents and short-term investments of $307.3 million and $45.6 million, respectively. We believe that our existing cash, cash equivalents and short-term investments, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See "Liquidity and Capital Resources." Our future viability beyond that point is dependent on our ability to raise additional capital to finance our operations.

**Components of Our Results of Operations**

***Revenue***

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales.

***Operating Expenses***

*Research and Development Expenses*

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. We expense research and development costs as incurred. These expenses may include:

- expenses incurred to conduct the necessary pre-clinical studies and clinical trials required to obtain regulatory approval;
- expenses incurred under agreements with contract research organizations, or CROs, that are primarily engaged in the oversight and conduct of our clinical trials and contract manufacturing organizations, or CMOs, that are primarily engaged to provide pre-clinical and clinical drug substance and product for our research and development programs;
- other costs related to acquiring and manufacturing pre-clinical studies and clinical trial materials, including manufacturing validation batches, as well as investigative sites and consultants that conduct our clinical trials, pre-clinical studies and other scientific development services;
- payments made in cash or equity securities under third-party licensing, acquisition and option agreements;

- employee-related expenses, including salaries and benefits, travel and share-based compensation expense for employees engaged in research and development functions;

- costs related to compliance with regulatory requirements; and

- allocated facilities-related costs, depreciation and other expenses, which include rent and utilities.

We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

Our direct research and development expenses are tracked on a program-by-program basis for our product candidates and consist primarily of external costs, such as fees paid to outside consultants, CROs, CMOs and research laboratories in connection with our pre-clinical development, process development, manufacturing and clinical development activities. Our direct research and development expenses by program also include fees incurred under license, acquisition and option agreements. We do not allocate employee costs or facility expenses, including depreciation or other indirect costs, to specific programs because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to conduct our research and discovery as well as for managing our pre-clinical development, process development, manufacturing and clinical development activities.

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

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| | (in thousands) | |
| Rilonacept [1] | $ 13,446 | $ 6,301 |
| Mavrilimumab [2] | 15,260 | 18,000 |
| KPL-716 [3] | 25,562 | 24,164 |
| KPL-404 [4] | 5,967 | 549 |
| KPL-045 [5] | 5,707 | 1,654 |
| Unallocated research and development expenses | 20,655 | 5,689 |
| Total research and development expenses | $ 86,597 | $ 56,357 |

[1] The amount for the year ended December 31, 2017 includes expense of $5.0 million related to an upfront payment under our license agreement with Regeneron.

[2] The amount for the year ended December 31, 2018 consists of $5.0 million related to a pass-through payment due upon the achievement of a specified clinical milestone event due under our license agreement with MedImmune. The amount for the year ended December 31, 2017 consists of $18.0 million related to an upfront payment and an accrued milestone under our license agreement with MedImmune.

[3] The amount for the year ended December 31, 2017 includes expense of $4.0 million related to a milestone payment under our asset purchase agreement with Biogen associated with the achievement of a specified clinical milestone event.

[4] The amount for the year ended December 31, 2018 includes expense of $0.8 million related to the extension of the option period under our stock purchase option agreement with Primatope. The amount for the year ended December 31, 2017 includes expense of $0.5 million related to upfront payments for the initial option period under our stock purchase option agreement with Primatope.

[5] The amount for the year ended December 31, 2017 includes expense of $1.5 million related to an upfront payment under our license agreement with Novo Nordisk.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect that our research and

development expenses will increase substantially over the next several years as we complete our ongoing and planned clinical trials for rilonacept, mavrilimumab and KPL-716, as well as conduct other pre-clinical and clinical development including regulatory filings for our other product candidates and our discovery research efforts and our related personnel costs will increase, including costs associated with share-based compensation. We also expect to incur additional expenses related to milestone and royalty payments payable to third parties with whom we have entered into license, acquisition and option agreements to acquire the rights to our product candidates.

The successful development and commercialization of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the pre-clinical and clinical development of any of our product candidates or when, if ever, material net cash inflows may commence from any of our product candidates. This uncertainty is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the scope, progress, outcome and costs of our pre-clinical development activities, clinical trials and other research and development activities;
- establishing an appropriate safety and efficacy profile with Investigational New Drug, or IND, enabling and clinical studies;
- successful patient enrollment in and the initiation and completion of clinical trials;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities including the U.S. Federal Drug Administration, or FDA;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishing clinical and commercial manufacturing capabilities or making arrangements with third-party manufacturers in order to ensure that we or our third-party manufacturers are able to make product successfully;
- development and timely delivery of clinical-grade and commercial-grade drug formulations that can be used in our clinical trials and for commercial launch;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- significant and changing government regulation;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of our product candidates following approval, if any, of our product candidates.

*General and administrative expenses*

General and administrative expenses consist primarily of salaries and benefits, travel and share-based compensation expense for personnel in executive, business development, finance, human resources, legal, information technology, pre-commercial and support personnel functions. General and administrative expenses also include insurance and professional fees for legal, patent, consulting, accounting and audit services.

We expect that our general and administrative expenses will continue to increase in the future as we increase our headcount to support our continued research activities and development of our product candidates and prepare for potential commercialization activities. We also anticipate that we will incur increased accounting, audit, legal,

regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses associated with being a public company.

***Interest income***

Interest income consists of income recognized from investments in money market funds and U.S. Treasury notes.

***Income taxes***

As an exempted company incorporated under the laws of Bermuda, we are principally subject to taxation in Bermuda. Under the current laws of Bermuda, tax on a company's income is assessed at a zero percent tax rate. As a result, we have not recorded any income tax benefits from our losses incurred in Bermuda during each reporting period, and no net operating loss carryforwards will be available to us for those losses. Our wholly owned U.S. subsidiary, Kiniksa Pharmaceuticals Corp., or Kiniksa US, is subject to federal and state income taxes in the United States. Our provision for income taxes relates to taxable income generated by Kiniksa US under a cost-plus arrangement that it has with us.

As of December 31, 2018, we had state research and development tax credit carryforwards of approximately $0.1 million available to reduce future tax liabilities, which begin to expire in 2033.

**Results of Operations**

***Comparison of the Years Ended December 31, 2018 and 2017***

The following table summarizes our results of operations for the years ended December 31, 2018 and 2017:

| | Years Ended December 31, | | |
|---|---|---|---|
| | 2018 | 2017 | Change |
| | | (in thousands) | |
| Operating expenses: | | | |
| Research and development | $ 86,597 | $ 56,357 | $ 30,240 |
| General and administrative | 21,563 | 9,043 | 12,520 |
| Total operating expenses | 108,160 | 65,400 | 42,760 |
| Loss from operations | (108,160) | (65,400) | (42,760) |
| Interest income | 4,719 | 529 | 4,190 |
| Loss before provision for income taxes | (103,441) | (64,871) | (38,570) |
| Benefit (provision) for income taxes | 214 | (2) | 216 |
| Net loss | $ (103,227) | $ (64,873) | $ (38,354) |

*Research and Development Expenses*

| | Years Ended December 31, | | |
|---|---|---|---|
| | 2018 | 2017 | Change |
| | | (in thousands) | |
| Direct research and development expenses by program: | | | |
| Rilonacept | $ 13,446 | $ 6,301 | $ 7,145 |
| Mavrilimumab | 15,260 | 18,000 | (2,740) |
| KPL-716 | 25,562 | 24,164 | 1,398 |
| KPL-404 | 5,967 | 549 | 5,418 |
| KPL-045 | 5,707 | 1,654 | 4,053 |
| Unallocated research and development expenses: | | | |
| Personnel related (including share-based compensation) | 15,032 | 4,576 | 10,456 |
| Other | 5,623 | 1,113 | 4,510 |
| Total research and development expenses | $ 86,597 | $ 56,357 | $ 30,240 |

Research and development expenses were $86.6 million for the year ended December 31, 2018, compared to $56.4 million for the year ended December 31, 2017. The increase of $30.2 million was primarily due to a $15.2 million increase in external fees related to our development programs, as well as an increase of $15.0 million in unallocated research and development expenses.

The direct costs for our rilonacept program were $13.5 million for the year ended December 31, 2018, compared to $6.3 million during the year ended December 31, 2017, or an increase of $7.2 million. During the year ended December 31, 2018, expenses incurred related to clinical research and development for our Phase 2 open label clinical trial and initiation of our Phase 3 clinical trial compared to the year ended December 31, 2017, in which expenses incurred were primarily due to the $5.0 million upfront payment made under our license agreement with Regeneron.

The direct costs of $15.3 million for our mavrilimumab program during the year ended December 31, 2018 were due to $5.0 million related to a pass-through payment due upon the achievement of a specified clinical milestone event due under our license agreement with MedImmune as well as expenses related to preparation for our planned clinical trials, including a Phase 2 trial in GCA and manufacturing process development related expenses compared to the year ended December 31, 2017 in which the expenses incurred related primarily to the $8.0 million upfront payment and $10.0 million accrued milestone under our license agreement with MedImmune.

The direct costs for our KPL-716 program were $25.6 million for the year ended December 31, 2018, compared to $24.2 million during the year ended December 31, 2017, or an increase of $1.4 million. During the year ended December 31, 2018, expenses incurred related to manufacturing and development costs for our clinical drug supply and our Phase 1a/1b clinical trial, compared to the year ended December 31, 2017, in which expenses incurred also included a $4.0 million milestone payment made upon the achievement of a specified clinical milestone event under our agreement with Biogen.

The direct costs for our KPL-404 program were $6.0 million during the year ended December 31, 2018, compared to $0.5 million during the year ended December 31, 2017, or an increase of $5.4 million. During the year ended December 31, 2018, expenses incurred primarily related to clinical research and development, including manufacturing development costs as well as $0.8 million related to the extension of the option period under our stock purchase option agreement with Primatope, compared to the year ended December 31, 2017, in which expenses incurred related to the $0.5 million initial option period payment under our stock purchase option agreement with Primatope.

The direct costs for our KPL-045 program were $5.7 million during the year ended December 31, 2018, compared to $1.7 million during the year ended December 31, 2017, or an increase of $4.0 million. During the year ended December 31, 2018, expenses incurred related to clinical research and development, including manufacturing

development costs, compared to the year ended December 31, 2017, in which expenses incurred related primarily to the upfront payment of $1.5 million made under our license agreement with Novo Nordisk.

Unallocated research and development expenses were $20.7 million for the year ended December 31, 2018 compared to $5.7 million for the year ended December 31, 2017. The increase of $15.0 million in unallocated research and development expenses was due to an increase of $10.5 million in personnel-related costs, including share-based compensation, and an increase of $4.5 million in other costs, including research costs related to potential future programs. The increase in personnel-related costs was primarily due to the hiring of additional personnel in our research and development functions, particularly those responsible for coordinating with CMOs on process development and manufacturing of drug supply and coordinating with CROs on the conduct and oversight of our current and planned clinical trials as well as research studies and development programs for our product candidates. Personnel-related costs for the year ended December 31, 2018 and 2017 included share-based compensation of $2.3 million and $0.3 million, respectively.

*General and Administrative Expenses*

General and administrative expenses were $21.6 million for the year ended December 31, 2018 compared to $9.0 million for the year ended December 31, 2017. The increase of $12.6 million was primarily due to increases of $7.7 million in personnel-related costs and $3.4 million in professional fees. The increase in personnel-related costs was due to the hiring of additional personnel in our general and administrative functions, primarily in our corporate departments, including legal, finance and human resources, as we became a public company and continued to expand our operations to support the organization. Personnel-related costs for the year ended December 31, 2018 and 2017 included share-based compensation of $3.4 million and $0.6 million, respectively. Professional fees increased due to legal costs incurred in connection with maintaining and registering worldwide patents and costs associated with our ongoing business operations, as well as higher recruiting, market research expenses, accounting and other costs incurred due to becoming a public company.

*Interest Income*

Interest income was $4.7 million for the year ended December 31, 2018 compared to $0.5 million for the year ended December 31, 2017. The increase was due to both higher average invested balances and higher interest rates on U.S. Treasury notes in 2018.

*Benefit (Provision) for Income Taxes*

We recorded an insignificant benefit (provision) for income taxes for the year ended December 31, 2018 and 2017.

**Liquidity and Capital Resources**

Since our inception, we have not generated any revenue from any sources, including from product sales, and have incurred significant operating losses and negative cash flows from our operations. Prior to the completion of our IPO in May 2018, we funded our operations primarily with proceeds from the sale of preferred shares, from which we had received net proceeds of $310.6 million.

On May 23, 2018, our registration statement on Form S-1 relating to our IPO was declared effective by the SEC. On May 29, 2018, we completed the IPO of 8,477,777 Class A common shares at $18.00 per share for gross proceeds of $152.6 million. In addition, on June 22, 2018, we completed the sale of 1,006,425 Class A common shares to the underwriters of the IPO following the exercise of their over-allotment option to purchase additional shares at $18.00 per share, for gross proceeds of $18.1 million. The aggregate net proceeds to us from the IPO, inclusive of the over-allotment option exercise, was $155.5 million after deducting underwriting discounts and commissions and other offering costs.

On February 4, 2019, we completed a follow-on offering of 2,654,984 Class A common shares and concurrent private placement of 2,000,000 Class A1 common shares, both at $18.26 per share for aggregate gross proceeds of $85.0 million. In addition, on March 1, 2019, we completed the sale of 161,126 Class A common shares to the underwriters of the follow-on offering following the exercise in part of their over-allotment option to purchase additional shares at $18.26 per share for gross proceeds of $2.9 million. The aggregate net proceeds to us from the follow-on offering and concurrent private placement, inclusive of the over-allotment option exercise, was $83.0 million after deducting underwriting discounts and commissions and other offering costs.

As of December 31, 2018, we had cash, cash equivalents and short-term investments of $307.3 million.

***Cash Flows***

The following table summarizes our cash flows for each of the periods presented:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| | (in thousands) | |
| Net cash used in operating activities | $ (81,012) | $ (50,219) |
| Net cash used in investing activities | (239,198) | (69) |
| Net cash provided by financing activities | 346,736 | 39,873 |
| Net increase (decrease) in cash and cash equivalents and restricted cash | $ 26,526 | $ (10,415) |

*Operating Activities*

During the year ended December 31, 2018, operating activities used $81.0 million of cash, primarily resulting from our net loss of $103.2 million, partially offset by net non-cash charges of $3.9 million and net cash provided by changes in our operating assets and liabilities of $18.3 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2018 consisted of a $14.3 million increase in accrued expenses and other liabilities and a $8.8 million increase in accounts payable, partially offset by a $4.8 million increase in prepaid expenses and other current assets. The increase in accrued expenses was primarily due to our increased level of operating activities and the timing of vendor invoicing and payments, an increase in accrued milestones as well as an increase in accrued employee compensation expense. The increase in accounts payable was primarily due to increased operating activities as well as the timing of vendor invoicing and payments. The increase in prepaid expenses and other current assets was due to increases in prepaid insurance expenses, interest receivable and prepaid expenses to CMOs related to manufacturing development and CROs related to our clinical trials.

During the year ended December 31, 2017, operating activities used $50.2 million of cash, primarily resulting from our net loss of $64.9 million, partially offset by non-cash charges of $0.7 million and net cash provided by changes in our operating assets and liabilities of $13.9 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2017 consisted of a $14.1 million increase in accrued expenses and a $1.0 million increase in accounts payable, both partially offset by a $1.2 million increase in prepaid expenses and other current assets. The increase in accrued expenses was primarily due to an accrued milestone of $10.0 million related to our mavrilimumab program, increased clinical trial and manufacturing activities as well as increased accrued legal and professional fees and accrued employee compensation-related expenses. The increase in accounts payable was due to our increased level of operating activities and the timing of vendor invoicing and payments. The increase in prepaid expenses and other current assets was primarily due to prepaid clinical trial and manufacturing costs associated with our research and development programs.

*Investing Activities*

During the year ended December 31, 2018, investing activities used $239.2 million of cash, consisting of $5.3 million of purchases of property and equipment and $402.0 million of purchases of short-term investments partially offset by $168.1 million from proceeds of maturities of short-term investments.

During the year ended December 31, 2017, investing activities used $0.1 million of cash, consisting of purchases of property and equipment.

*Financing Activities*

During the year ended December 31, 2018, net cash provided by financing activities was $346.7 million, primarily consisting of proceeds of $159.2 million from our issuance and sale of Class A common shares, net of underwriting commissions and discounts upon completion of our IPO, inclusive of the over-allotment option exercise, $190.8 million in net proceeds from our issuance and sale of Series C preferred shares, and $0.4 million in proceeds from the exercise of share options and our employee share purchase plan, partially offset by $3.7 million of payments of other offering costs associated with our IPO, inclusive of the over-allotment option exercise.

During the year ended December 31, 2017, net cash provided by financing activities was $39.9 million, consisting of net proceeds from our issuance and sale of Series B preferred shares.

***Funding Requirements***

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the clinical trials and pre-clinical activities of our product candidates. Additionally, we expect to continue to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Our expenses will also increase as we:

- continue to conduct our current clinical trials and initiate our planned clinical trials, for rilonacept, mavrilimumab and KPL-716;
- advance pre-clinical development of our early-stage programs;
- manufacture, or have manufactured on our behalf, our pre-clinical and clinical drug material and develop processes for late stage and commercial manufacturing;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- establish a sales, marketing, medical affairs and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval and intend to commercialize on our own;
- hire additional clinical, quality control and scientific personnel;
- expand our operational, financial and management systems and increase personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- maintain, expand and protect our intellectual property portfolio; and
- in-license or acquire other product candidates and technologies or their related businesses.

We believe that our existing cash, cash equivalents and short-term investments will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. We anticipate that we may require additional capital if we choose to pursue in-licenses or acquisitions of other product candidates. If we receive regulatory approval for rilonacept or our other product candidates, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution, depending on where we choose to commercialize.

Because of the numerous risks and uncertainties associated with research, development and commercialization of biologic product candidates, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, progress, results and costs of researching and developing our product candidates, and conducting pre-clinical and clinical trials;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs, timing and ability to manufacture our product candidates to supply our clinical and pre-clinical development efforts and our clinical trials;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of manufacturing commercial-grade product and necessary inventory to support commercial launch;
- the ability to receive additional non-dilutive funding, including grants from organizations and foundations;
- the revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings, governmental funding, collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interest may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect our shareholders' rights as a common shareholder. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. In addition, debt financing would result in fixed payment obligations.

If we raise funds through governmental funding, collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, reduce or eliminate our product development or future commercialization efforts.

**Contractual Obligations and Commitments**

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

| | Payments Due by Period | | | | |
|---|---|---|---|---|---|
| | Less than 1 year | 1 to 3 years | 4 to 5 years | More than 5 years | Total |
| | | | (in thousands) | | |
| Accrued milestone [(1)] | $ 15,000 | $ — | $ — | $ — | $ 15,000 |
| Manufacturing commitments [(2)] | 12,012 | — | — | — | 12,012 |
| Operating lease commitments [(3)] | 1,394 | 2,793 | — | — | 4,187 |
| Total | $ 28,406 | $ 2,793 | $ — | $ — | $ 31,199 |

(1) Represents a payment of $10.0 million we are obligated to make under our license agreement with MedImmune upon the earlier to occur of (a) the first achievement of a specified regulatory milestone for a product licensed under the agreement and (b) December 31, 2018 and a $5.0 million related to a pass-through payment due upon the achievement of a specified clinical milestone event under our license agreement with MedImmune.

(2) Amounts in the table reflect commitments for costs associated with our external CMOs, which we have engaged to manufacture pre-clinical and clinical trial materials. Manufacturing commitments include agreements that are enforceable and legally binding on us and that specify all significant terms, including fixed or minimum quantities to be purchased; fixed, minimum or variable price provisions; and the approximate timing of the transaction. For obligations with cancellation provisions, the amounts included in the preceding table are limited to the non-cancelable portion of the agreement terms or the minimum cancellation fee.

(3) Amounts in the table reflect minimum payments due under operating lease agreements entered into by our wholly owned U.S. subsidiary Kiniksa US for office and laboratory space in Lexington, Massachusetts which expires in 2021 and office space in San Diego, California which expires in 2020.

Our contracts with CMOs, CROs and other third parties for the manufacture of our product candidates and to support clinical trials and pre-clinical research studies and testing are generally cancelable by us upon prior notice. Payments due upon cancellation consisting only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation are not included in the preceding table as the amount and timing of such payments are not known.

Under various agreements with third parties, we have agreed to make milestone payments, pay royalties, annual maintenance fees and to meet due diligence requirements based upon specified milestones. We generally have not included any contingent payment obligations, such as milestones, royalties or due diligence, in the table above as the amount, timing and likelihood of such payments are not known. We have not included any of the annual maintenance fee payments in the above table, as although the amount and timing are known, we cannot currently determine the final termination dates of the agreements and, as a result, we cannot determine the total amounts of such payments we will be required to make under the agreements.

Under our license agreement with Regeneron, we are obligated to make future regulatory milestone payments of $27.5 million in the aggregate. Thereafter, we have agreed to evenly split profits on our sales of rilonacept with Regeneron after deducting certain commercialization expenses subject to specified limits.

Under our license agreement with MedImmune, we are obligated to make future clinical, regulatory and initial sales milestone payments of up to $72.5 million in aggregate for the first two indications we develop, including, a $5.0 million pass-through payment due upon the achievement of a specified clinical milestone event which was met in the fourth quarter of 2018. Also included is a milestone payment of $10.0 million due upon the earlier to occur of a

specified regulatory milestone and December 31, 2018, and clinical and regulatory milestone payments of up to $15.0 million in the aggregate for each subsequent indication. The $10.0 million milestone payment was accrued on our consolidated balance sheet as of December 31, 2017 and recognized as research and development expense during the year ended December 31, 2017. The $5.0 million pass-through payment was accrued on our consolidated balance sheet as of December 31, 2018 and recognized as research and development expense during the year ended December 31, 2018. Such payments are included in the table above. We are also obligated to make milestone payments to MedImmune of up to $85.0 million upon the achievement of annual net sales thresholds of up to, but excluding, $1.0 billion in annual net sales as well as additional milestone payments aggregating up to $1.1 billion upon the achievement of additional specified annual net sales thresholds starting at $1.0 billion and higher. Commencing on the first commercial sale of licensed products, we are obligated to pay tiered royalties on escalating tiers of annual net sales of licensed products starting in the low double-digit percentages and ending at twenty percent. We must pay such royalties on a product-by-product and country-by-country basis until the latest to occur of the expiration of licensed patents, the expiration of regulatory exclusivity or the tenth anniversary of the first commercial sale of such product in such country.

Under our asset purchase agreement with Biogen, we are obligated to make future milestone payments of up to $325.0 million upon the achievement of specified clinical and regulatory milestones as well as upon the achievement of annual net sales thresholds, including a $10.0 million payment due upon the achievement of a specified clinical milestone event which may be met in the first half of 2019. We have also agreed to pay certain obligations under third-party contracts retained by Biogen that relate to KPL-716. Additionally, we are obligated to pay tiered royalties on escalating tiers of annual net sales of licensed products starting in the high single-digit percentages and ending below the teens.

Under our license agreement with Novo Nordisk, we are obligated to make future milestone payments upon the achievement of specified clinical, regulatory, initial sales milestones as well as upon the achievement of annual net sales thresholds, including a payment of $1.0 million upon the earlier to occur of a specified regulatory milestone and January 2020. We are also obligated to pay royalties on annual net sales of products licensed under the agreement. In addition, we are obligated to make a payment upon the completion of technology transfer.

In March 2019, we acquired all of the issued and outstanding equity securities of Primatope in exchange for upfront consideration of $10.0 million at closing as well as milestone payments of up to $8.0 million ($5.0 million of which had been achieved as of the closing date and was paid at closing) each paid or payable in a combination of cash and our Class A common shares (inclusive of escrow and holdback amounts) in accordance with the terms and conditions of our stock purchase option agreement with Primatope.

**Critical Accounting Policies and Significant Judgments and Estimates**

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

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

***Accrued Research and Development Expenses***

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

communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors, including research laboratories, in connection with pre-clinical development activities;
- CROs and investigative sites in connection with pre-clinical studies and clinical trials; and
- CMOs in connection with drug substance and drug product formulation of pre-clinical studies and clinical trial materials.

We base our expenses related to pre-clinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that conduct and manage pre-clinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. 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 service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

***Share-Based Compensation***

We measure options and other share-based awards granted to employees and directors based on the fair value on the date of the grant and recognize the corresponding compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. We have only issued options and restricted share awards with service-based vesting conditions and record the expense for these awards using the straight-line method.

For share-based awards granted to consultants and non-employees, we recognize compensation expense over the period during which services are rendered by such consultants and non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of our Class A common shares and updated assumption inputs in the Black-Scholes option-pricing model.

We estimate the fair value of each option grant using the Black-Scholes option-pricing model, which uses as inputs the fair value of our Class A common shares and assumptions we make for the volatility of our Class A common shares, the expected term of our options, the risk-free interest rate for a period that approximates the expected term of our options and our expected dividend yield. Until our IPO, we were a private company and we lacked company-specific historical and implied volatility information. Accordingly, we estimate our expected volatility based on the historical volatility of publicly traded peer companies and expect to continue to do so until such time as we have adequate historical data regarding the volatility of our traded share price. The expected term of our options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options, while the expected term of our options granted to consultants and nonemployees has been determined based on the contractual term of the options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the

award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future.

**Emerging Growth Company Status**

The Jumpstart Our Business Act permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

**Off-Balance Sheet Arrangements**

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

**Recently Issued Accounting Pronouncements**

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our annual consolidated financial statements appearing at the end of this Annual Report on Form 10-K.

## ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.

***Interest Rate Risk***

We are exposed to market risk related to changes in interest rates. As of December 31, 2018, our cash, cash equivalents and short-term investments consisted of money market funds and U.S. Treasury notes. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

## ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found in Item 15.

## ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE.

None.

## ITEM 9A. CONTROLS AND PROCEDURES.

***Evaluation of Disclosure Controls and Procedures***

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated, as of the end of the period covered by this Annual Report, 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, or the Exchange Act). Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2018.

In designing and evaluating 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, or the Exchange Act), management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives.

***Exemption from Management's Report on Internal Control over Financial Reporting for the Fiscal Year Ended December 31, 2018.***

This Annual Report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our independent registered public accounting firm due to a transition period established by the rules of the SEC for newly public companies.

***Changes in Internal Control over Financial Reporting***

There was no change in our internal control over financial reporting, (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the fourth quarter of the year ended December 31, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## ITEM 9B. OTHER INFORMATION.

None.

# PART III

## ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE.

Except to the extent provided below, the information required to be disclosed by this Item will be set forth in our Proxy Statement for our 2019 Annual Meeting of Shareholders to be filed with the SEC within 120 days of December 31, 2018, and is incorporated into this Annual Report by reference.

We have adopted a written code of business conduct and ethics that applies to all of our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer and controller, or persons performing similar functions. Our code of business conduct and ethics is available in the "Investors & Media" section of our website at *www.kiniksa.com* under "Corporate Governance". We intend to post on our website all disclosures that are required by law or the Nasdaq rules concerning any amendments to, or waivers from, any provision of the code. The reference to our website address does not constitute incorporation by reference of the information contained at or available through our website, and you should not consider it to be a part of this Annual Report.

## ITEM 11. EXECUTIVE COMPENSATION.

The information required to be disclosed by this Item will be set forth in our Proxy Statement for our 2019 Annual Meeting of Shareholders to be filed with the SEC within 120 days of December 31, 2018, and is incorporated into this Annual Report by reference.

## ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS.

The information required to be disclosed by this Item will be set forth in our Proxy Statement for our 2019 Annual Meeting of Shareholders to be filed with the SEC within 120 days of December 31, 2018, and is incorporated into this Annual Report by reference.

## ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE.

The information required to be disclosed by this Item will be set forth in our Proxy Statement for our 2019 Annual Meeting of Shareholders to be filed with the SEC within 120 days of December 31, 2019, and is incorporated into this Annual Report by reference.

## ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES.

The information required to be disclosed by this Item will be set forth in our Proxy Statement for our 2019 Annual Meeting of Shareholders to be filed with the SEC within 120 days of December 31, 2019, and is incorporated into this Annual Report by reference.

## PART IV

## ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES.

(a)(1) Financial Statements.

See the "Index to Consolidated Financial Statements" on page F-1 below for the list of financial statements filed as part of this report.

(a)(2) Financial Statement Schedules.

All schedules have been omitted because they are not required or because the required information is given in the Consolidated Financial Statements or Notes thereto set forth below beginning on page F-1.

(a)(3) Exhibits. See Exhibit Index.

EXHIBIT INDEX

| Exhibit Number | Exhibit Description | Incorporated by Reference | | | | Filed/ Furnished Herewith |
|---|---|---|---|---|---|---|
| | | Form | File No. | Exhibit | Filing Date | |
| 3.1 | Memorandum of Association of Kiniksa Pharmaceuticals, Ltd. | S-1 | 333-224488 | 3.1 | 4/27/18 | |
| 3.2 | Amended and Restated Bye-Laws of Kiniksa Pharmaceuticals, Ltd. | 8-K | 001-38492 | 3.1 | 5/29/18 | |
| 4.1 | Specimen Share Certificate evidencing the Class A common shares | S-1/A | 333-224488 | 4.1 | 5/14/18 | |
| 4.2 | Second Amended and Restated Investors' Rights Agreement, dated as of February 9, 2018 | S-1 | 333-224488 | 4.2 | 4/27/18 | |
| 10.1 | Amended and Restated Employment Agreement, dated as of May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and Sanj K. Patel | 10-Q | 001-38492 | 10.7 | 8/6/18 | |
| 10.2 | Amended and Restated Employment Agreement, dated as of May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and Stephen Mahoney | 10-Q | 001-38492 | 10.8 | 8/6/18 | |
| 10.3 | Amended and Restated Employment Agreement, dated as of May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and John F. Paolini | 10-Q | 001-38492 | 10.9 | 8/6/18 | |
| 10.4 | Amended and Restated Employment Agreement, dated as of May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and Thomas Beetham | S-1 | 333-229394 | 10.4 | 1/28/19 | |
| 10.5 | Amended and Restated Employment Agreement, dated as May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and Chris Heberlig | S-1 | 333-229394 | 10.5 | 1/28/19 | |
| 10.6 | Amended and Restated Employment Agreement, dated as of May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and Carsten Boess | S-1 | 333-229394 | 10.6 | 1/28/19 | |
| 10.7 | Amended and Restated Employment Agreement, dated as May 29, 2018, by and between Kiniksa Pharmaceuticals Corp. and Rasmus Holm-Jorgensen | S-1 | 333-229394 | 10.7 | 1/28/19 | |
| 10.8† | Asset Purchase Agreement, dated September 7, 2016, by and between the Registrant and Biogen MA Inc., as amended | S-1 | 333-224488 | 10.6 | 4/27/18 | |
| 10.9† | License Agreement, dated September 25, 2017, by and between the Registrant and Regeneron Pharmaceuticals, Inc. | S-1 | 333-224488 | 10.7 | 4/27/18 | |

| Exhibit Number | Exhibit Description | Incorporated by Reference | | | | Filed/ Furnished Herewith |
|---|---|---|---|---|---|---|
| | | Form | File No. | Exhibit | Filing Date | |
| 10.10† | License Agreement, dated as of December 21, 2017, by and between the Registrant and MedImmune, Limited | S-1 | 333-224488 | 10.8 | 4/27/18 | |
| 10.11 | Clinical Supply Agreement, dated as of September 27, 2017, by and between the Registrant and Regeneron Pharmaceuticals, Inc. | S-1 | 333-224488 | 10.9 | 4/27/18 | |
| 10.12 | Sublease Agreement, dated as of March 13, 2018, by and between Kiniksa Pharmaceuticals Corp. and Shire Human Genetic Therapies, Inc. | S-1 | 333-224488 | 10.10 | 4/27/18 | |
| 10.13 | First and Second Amendment to Sublease Agreement, dated as of June 26, 2018 and July 17, 2018, respectively, by and between Kiniksa Pharmaceuticals Corp. and Shire Human Genetic Therapies, Inc. | 10-Q | 001-38492 | 10.10 | 8/6/18 | |
| 10.14 | Third Amendment to Sublease Agreement, dated as of November 7, 2018, by and between Kiniksa Pharmaceuticals Corp. and Shire Human Genetic Therapies, Inc. | 8-K | 001-38492 | 10.1 | 11/13/18 | |
| 10.15 | Form of Indemnification Agreement for Non-Fund-Designated Directors | S-1 | 333-224488 | 10.11 | 4/27/18 | |
| 10.16 | Form of Indemnification Agreement for Fund-Designated Directors | S-1 | 333-224488 | 10.12 | 4/27/18 | |
| 10.17 | Form of Indemnification Agreement for Officers | S-1 | 333-224488 | 10.13 | 4/27/18 | |
| 10.18 | 2015 Equity Incentive Plan, as amended, and form of share option grant notice and option agreement thereunder | S-1 | 333-224488 | 10.1 | 4/27/18 | |
| 10.19 | 2018 Incentive Award Plan, and the form of share option grant notice and option agreement, form of restricted share grant notice and restricted share agreement, and form of restricted share unit grant notice and restricted share unit agreement thereunder | S-1 | 333-229394 | 10.19 | 1/28/19 | |
| 10.20 | 2018 Employee Share Purchase Plan | S-1/A | 333-224488 | 10.14 | 5/14/18 | |
| 10.21 | Offering Document under the 2018 Employee Share Purchase Plan | 10-Q | 001-38492 | 10.6 | 8/6/18 | |
| 10.22 | Offering Document under the 2018 Employee Share Purchase Plan | S-1 | 333-229394 | 10.22 | 1/28/19 | |
| 10.23 | 2018 Incentive Award Plan; Sub-Plan for UK Employees, and the form of share option grant notice for UK participants | S-1 | 333-229394 | 10.23 | 1/28/19 | |

| Exhibit Number | Exhibit Description | Incorporated by Reference | | | | Filed/ Furnished Herewith |
|---|---|---|---|---|---|---|
| | | Form | File No. | Exhibit | Filing Date | |
| 10.24 | Non-Employee Director Compensation Program | S-1/A | 333-224488 | 10.15 | 5/14/18 | |
| 10.25 | Restricted Share Agreement, dated as of September 16, 2015, by and between the Registrant and Sanj K. Patel | S-1 | 333-229394 | 10.25 | 1/28/19 | |
| 10.26 | Restricted Share Agreement, dated as of September 18, 2015, by and between the Registrant and Stephen Mahoney | S-1 | 333-229394 | 10.26 | 1/28/19 | |
| 10.27 | 2018 Incentive Award Plan forms of share option grant notice and share option agreement for German participants, restricted share grant notice and restricted share agreement for German participants, and restricted share unit grant notice and restricted share unit agreement for German participants | | | | | * |
| 21.1 | Subsidiaries of the Registrant | S-1 | 333-229394 | 21.1 | 1/28/19 | |
| 23.1 | Consent of PricewaterhouseCoopers LLP, independent registered public accounting firm | | | | | * |
| 31.1 | Rule 13a-14(a) / 15d-14(a) Certification of Chief Executive Officer | | | | | * |
| 31.2 | Rule 13a-14(a) / 15d-14(a) Certification of Chief Financial Officer | | | | | * |
| 32.1 | Section 1350 Certification of Chief Executive Officer | | | | | ** |
| 32.2 | Section 1350 Certification of Chief Financial Officer | | | | | ** |
| 101.INS | XBRL Instance Document | | | | | *** |
| 101.SCH | XBRL Taxonomy Extension Schema Document | | | | | *** |
| 101.CAL | XBRL Taxonomy Extension Calculation Linkbase Document | | | | | *** |
| 101.DEF | XBRL Extension Definition Linkbase Document | | | | | *** |
| 101.LAB | XBRL Taxonomy Label Linkbase Document | | | | | *** |
| 101.PRE | XBRL Taxonomy Extension Presentation Linkbase Document | | | | | *** |

* Filed herewith
** Furnished herewith
*** Submitted electronically herewith
† Confidential treatments of certain provisions has been granted by the SEC pursuant to Rule 406 under the Securities Act of 1933, as amended

## ITEM 16. FORM 10-K SUMMARY

None.

## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

**KINIKSA PHARMACEUTICALS, LTD.**

Date: March 12, 2019

By:/s/ Sanj K. Patel
Sanj K. Patel
Chief Executive Officer and Chairman of the Board of Directors

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

| Signature | Title | Date |
|---|---|---|
| /s/ Sanj K. Patel<br>Sanj K. Patel | Chief Executive Officer and Chairman of the Board of Directors (principal executive officer) | March 12, 2019 |
| /s/ Chris Heberlig<br>Chris Heberlig | Chief Financial Officer (principal financial and accounting officer) | March 12, 2019 |
| /s/ Felix J. Baker<br>Felix J. Baker | Lead Independent Director | March 12, 2019 |
| /s/ Stephen R. Biggar<br>Stephen R. Biggar | Director | March 12, 2019 |
| /s/ Richard S. Levy<br>Richard S. Levy | Director | March 12, 2019 |
| /s/ Thomas R. Malley<br>Thomas R. Malley | Director | March 12, 2019 |
| /s/ Tracey L. McCain<br>Tracey L. McCain | Director | March 12, 2019 |
| /s/ Kimberly J. Popovits<br>Kimberly J. Popovits | Director | March 12, 2019 |
| /s/ Barry D. Quart<br>Barry D. Quart | Director | March 12, 2019 |

## INDEX TO CONSOLIDATED FINANCIAL STATEMENTS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| Consolidated Statements of Cash Flows | F-6 |
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## Report of Independent Registered Public Accounting Firm

To the Board of Directors and Shareholders of Kiniksa Pharmaceuticals, Ltd.

***Opinion on the Financial Statements***

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

***Basis for Opinion***

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

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

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

/s/ PricewaterhouseCoopers LLP
Boston, Massachusetts
March 12, 2019

We have served as the Company's auditor since 2016.

**KINIKSA PHARMACEUTICALS, LTD.**
**CONSOLIDATED BALANCE SHEETS**
**(In thousands, except share and per share amounts)**

| | December 31, 2018 | December 31, 2017 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 71,976 | $ 45,555 |
| Restricted cash | — | 105 |
| Short-term investments | 235,328 | — |
| Prepaid expenses and other current assets | 6,446 | 1,444 |
| Total current assets | 313,750 | 47,104 |
| Property and equipment, net | 6,356 | 125 |
| Restricted cash | 210 | — |
| Deferred offering costs | 433 | 25 |
| Deferred tax assets | 1,216 | 238 |
| Total assets | $ 321,965 | $ 47,492 |
| **Liabilities, Convertible Preferred Shares and Shareholders' Equity (Deficit)** | | |
| Current liabilities: | | |
| Accounts payable | $ 10,918 | $ 1,218 |
| Accrued expenses | 16,418 | 6,212 |
| Accrued milestones | 15,000 | 10,000 |
| Other current liabilities | 218 | — |
| Total current liabilities | 42,554 | 17,430 |
| Deferred rent | 144 | — |
| Total liabilities | 42,698 | 17,430 |
| Commitments and contingencies (Note 12) | | |
| Convertible preferred shares (Series A, B and C), $0.000273235 par value; 0 shares and 22,885,492 shares issued and outstanding as of December 31, 2018 and December 31, 2017, respectively; aggregate liquidation preference of $0 and $120,000 as of December 31, 2018 and December 31, 2017, respectively; | — | 119,770 |
| Shareholders' equity (deficit): | | |
| Class A common shares, par value of $0.000273235 per share; 15,797,220 shares and 719,976 shares issued and outstanding as of December 31, 2018 and December 31, 2017; respectively | 4 | — |
| Class B common shares, par value of $0.000273235 per share; 4,638,855 shares and 3,568,353 shares issued and outstanding as of December 31, 2018 and December 31, 2017, respectively | 1 | 1 |
| Class A1 common shares, $0.000273235 par value; 12,995,954 shares and 0 shares issued and outstanding as of December 31, 2018 and December 31, 2017, respectively | 4 | — |
| Class B1 common shares, $0.000273235 par value; 16,057,618 shares and 0 shares issued and outstanding as of December 31, 2018 and December 31, 2017, respectively | 4 | — |
| Additional paid-in capital | 473,483 | 1,289 |
| Accumulated other comprehensive loss | (4) | — |
| Accumulated deficit | (194,225) | (90,998) |
| Total shareholders' equity (deficit) | 279,267 | (89,708) |
| Total liabilities, convertible preferred shares and shareholders' equity (deficit) | $ 321,965 | $ 47,492 |

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

**KINIKSA PHARMACEUTICALS, LTD.**
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**
**(In thousands, except share and per share amounts)**

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Operating expenses: | | |
| Research and development | $ 86,597 | $ 56,357 |
| General and administrative | 21,563 | 9,043 |
| Total operating expenses | 108,160 | 65,400 |
| Loss from operations | (108,160) | (65,400) |
| Interest income | 4,719 | 529 |
| Loss before benefit (provision) for income taxes | (103,441) | (64,871) |
| Benefit (provision) for income taxes | 214 | (2) |
| Net loss | $ (103,227) | $ (64,873) |
| Net loss per share attributable to common shareholders—basic and diluted | $ (3.49) | $ (35.85) |
| Weighted average common shares outstanding—basic and diluted | 29,547,427 | 1,809,751 |
| Comprehensive loss: | | |
| Net loss | $ (103,227) | $ (64,873) |
| Other comprehensive loss: | | |
| Unrealized loss on short-term investments | (4) | — |
| Total other comprehensive loss | (4) | — |
| Total comprehensive loss | $ (103,231) | $ (64,873) |

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

**KINIKSA PHARMACEUTICALS, LTD.**
**CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED SHARES AND SHAREHOLDERS' EQUITY (DEFICIT)**
**(In thousands, except share amounts)**

| | Convertible Preferred Shares (Series A, B and C) | | Common Shares (Class A, B, A1 and B1) | | Additional Paid-In Capital | Accumulated OCL | Accumulated Deficit | Total Shareholders' Equity (Deficit) |
|---|---|---|---|---|---|---|---|---|
| | Shares | Amount | Shares | Amount | | | | |
| **Balances at December 31, 2016** | 17,128,120 | $ 79,897 | 4,288,329 | $ 1 | $ 392 | $ — | $ (26,125) | $ (25,732) |
| Issuance of Series B convertible preferred shares, net of issuance costs of $127 | 5,757,372 | 39,873 | — | — | — | — | — | — |
| Share-based compensation expense | — | — | — | — | 897 | — | — | 897 |
| Net loss | — | — | — | — | — | — | (64,873) | (64,873) |
| **Balances at December 31, 2017** | 22,885,492 | $ 119,770 | 4,288,329 | $ 1 | $ 1,289 | $ — | $ (90,998) | $ (89,708) |
| Issuance of Series C convertible preferred shares, net of issuance costs of $9,178 | 12,784,601 | 190,822 | — | — | — | — | — | — |
| Conversion of convertible preferred shares to common shares | (35,670,093) | (310,592) | 35,670,093 | 8 | 310,584 | — | — | 310,592 |
| Issuance of Class A common shares upon completion of initial public offering, net of underwriting discounts and commissions and offering costs | — | — | 9,484,202 | 4 | 155,532 | — | — | 155,536 |
| Exercise of options and issuance of shares under the employee share purchase plan | — | — | 47,023 | — | 377 | — | — | 377 |
| Share-based compensation expense | — | — | — | — | 5,701 | — | — | 5,701 |
| Unrealized loss on short-term investments | — | — | — | — | — | (4) | — | (4) |
| Net loss | — | — | — | — | — | — | (103,227) | (103,227) |
| **Balances at December 31, 2018** | — | $ — | 49,489,647 | $ 13 | $ 473,483 | $ (4) | $ (194,225) | $ 279,267 |

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

**KINIKSA PHARMACEUTICALS, LTD.**
**CONSOLIDATED STATEMENTS OF CASH FLOWS**
**(In thousands)**

| | Years Ended December 31, | |
|---|---|---|
| | **2018** | **2017** |
| **Cash flows from operating activities:** | | |
| Net loss | $ (103,227) | $ (64,873) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation expense | 286 | 28 |
| Share-based compensation expense | 5,701 | 897 |
| Loss on disposal of property and equipment | 66 | — |
| Non-cash rent expense | 235 | — |
| Accretion of discounts on short-term investments | (1,423) | — |
| Deferred income taxes | (978) | (197) |
| Changes in operating assets and liabilities: | | |
| Prepaid expenses and other assets | (4,791) | (1,185) |
| Accounts payable | 8,823 | 1,006 |
| Accrued expenses and other liabilities | 9,296 | 4,105 |
| Accrued milestones | 5,000 | 10,000 |
| Net cash used in operating activities | (81,012) | (50,219) |
| **Cash flows from investing activities:** | | |
| Purchases of property and equipment | (5,290) | (69) |
| Purchases of short-term investments | (402,008) | — |
| Proceeds from the maturities of short-term investments | 168,100 | — |
| Net cash used in investing activities | (239,198) | (69) |
| **Cash flows from financing activities:** | | |
| Proceeds from issuance of Series B convertible preferred shares, net of issuance costs | — | 39,873 |
| Proceeds from issuance of Series C convertible preferred shares, net of issuance costs | 190,822 | — |
| Proceeds from issuance of Class A common shares upon completion of initial public offering, net of underwriting commissions and discounts, inclusive of the over-allotment option exercise | 159,194 | — |
| Payments of offering costs | (3,657) | — |
| Proceeds from exercise of options and employee share purchase plan | 377 | — |
| Net cash provided by financing activities | 346,736 | 39,873 |
| **Net increase (decrease) in cash and cash equivalents and restricted cash** | 26,526 | (10,415) |
| Cash and cash equivalents and restricted cash at beginning of period | 45,660 | 56,075 |
| Cash and cash equivalents and restricted cash at end of period | $ 72,186 | $ 45,660 |
| | | |
| **Supplemental information:** | | |
| Cash paid for income taxes | $ 383 | $ 290 |
| | | |
| **Supplemental disclosure of non-cash investing and financing activities:** | | |
| Deferred offering costs included in accrued expenses and accounts payable | $ 404 | $ 25 |
| Property and equipment included in accrued expenses and accounts payable | $ 1,292 | $ — |

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

## 1. Nature of the Business and Basis of Presentation

Kiniksa Pharmaceuticals, Ltd. (the "Company") is a clinical-stage biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients suffering from debilitating diseases with significant unmet medical need. The Company was incorporated in July 2015 as a Bermuda exempted company. The Company has a pipeline of product candidates across various stages of development, currently focused on autoinflammatory and autoimmune conditions.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's technology will be obtained, that any products developed will obtain necessary government regulatory approval or that any products, if approved, will be commercially viable. The Company does not currently generate revenue from sales of any products, and it may never be able to develop or commercialize a marketable product. The Company has not yet successfully completed any Phase 3 or other pivotal clinical trials, obtained any regulatory approvals, manufactured a commercial-scale drug, or conducted sales and marketing activities. The Company operates in an environment of rapid technological innovation and substantial competition from pharmaceutical and biotechnological companies. In addition, the Company is dependent upon the services of its employees, consultants and service providers. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

### *Principles of Consolidation*

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") and include the accounts of the Company and its wholly owned subsidiaries, Kiniksa Pharmaceuticals, Corp. ("Kiniksa US") and Kiniksa Pharmaceuticals (UK), Ltd. ("Kiniksa UK"), after elimination of all significant intercompany accounts and transactions.

In assessing the consolidation requirement for variable interest entities ("VIEs"), the Company focuses on identifying whether it has both the power to direct the activities that most significantly impact the VIE's economic performance and the obligation to absorb losses or the right to receive benefits from the VIE. In the event that the Company is the primary beneficiary of a VIE, the assets, liabilities, and results of operations of the VIE would be included in the Company's consolidated financial statements. At December 31, 2018 and 2017 and during the years then ended, the Company was not the primary beneficiary of a VIE.

### *Use of Estimates*

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

### *Reporting and Functional Currency*

The Company's reporting currency is the U.S. dollar ("USD") and its operations utilize the USD or local currency as the functional currency, where applicable.

Transactions in other currencies are recorded in the functional currency at the rate of exchange prevailing when the transactions occur. Monetary assets and liabilities denominated in other currencies are re-measured into the functional currency at the rate of exchange in effect at the balance sheet date. Exchange gains and losses arising from re-measurement of foreign currency-denominated monetary assets and liabilities are included in income in the period in which they occur.

For our foreign subsidiary where the local currency is the functional currency, assets and liabilities denominated in local currencies are translated into USD at end-of-period exchange rates and the resulting translation adjustments are reported as a component of accumulated other comprehensive income (loss) within shareholders' equity (deficit).

***Reverse Share Split***

On May 11, 2018, the Company effected a 1-for-2.73235 reverse share split of its authorized, designated, issued and outstanding common shares and preferred shares. Accordingly, all share and per share amounts for all periods presented in the accompanying consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse share split.

***Initial Public Offering***

On May 23, 2018, the Company's registration statement on Form S-1 relating to its initial public offering of its Class A common shares (the "IPO") was declared effective by the Securities and Exchange Commission ("SEC"). On May 29, 2018, the Company completed the IPO of 8,477,777 Class A common shares at $18.00 per share for gross proceeds of $152,600. In addition, on June 22, 2018, the Company completed the sale of 1,006,425 Class A common shares to the underwriters of the IPO following the exercise of their over-allotment option to purchase additional shares at $18.00 per share for gross proceeds of $18,116. The aggregate net proceeds to the Company from the IPO, inclusive of the over-allotment option exercise, was $155,536 after deducting underwriting discounts and commissions and other offering costs.

Upon the closing of the IPO, all convertible preferred shares then outstanding automatically converted into 5,546,019 Class A common shares, 1,070,502 Class B common shares, 12,995,954 Class A1 common shares and 16,057,618 Class B1 common shares. In connection with the closing of the IPO, the Company amended and restated its bye-laws (the "Amended & Restated Bye-Laws").

***Liquidity***

In accordance with Accounting Standards Update ("ASU") 2014-15, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40), the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the consolidated financial statements are issued. As of December 31, 2018, the Company had an accumulated deficit of $194,225. During the year ended December 31, 2018, the Company incurred a net loss of $103,227 and used $81,012 of cash in operating activities. The Company expects to continue to generate operating losses and cash used in operations for the foreseeable future. As of December 31, 2018, the Company had cash, cash equivalents and short-term investments of $307,304. On February 4, 2019, the Company completed a follow-on offering of 2,654,984 Class A common shares and concurrent private placement of 2,000,000 class A1 common shares, both at $18.26 per share for aggregate gross proceeds of $85,000. In addition, on March 1, 2019, the Company completed the sale of 161,126 Class A common shares to the underwriters of the follow-on offering following the exercise in part of their over-allotment option to purchase additional shares at $18.26 per share for gross proceeds of $2,942. The aggregate net proceeds to the Company from the follow-on offering and concurrent private placement,

inclusive of the over-allotment exercise was $82,969 after deducting underwriting discounts and commissions and other offering costs.

Based on its current operating plan, the Company expects that its cash, cash equivalents and short term investments, including the proceeds from the February 2019 follow-on offering and concurrent private placement inclusive of the over-allotment exercise, will be sufficient to fund its operating expenses and capital expenditure requirements for at least twelve months from the issuance date of these consolidated financial statements. The future viability of the Company beyond that point is dependent on its ability to raise additional capital to finance its operations. Although the Company has been successful in raising capital in the past, there is no assurance that it will be successful in obtaining such additional financing on terms acceptable to the Company, if at all. If the Company is unable to obtain funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations.

## 2. Summary of Significant Accounting Policies

### *Cash and Cash Equivalents*

The Company classifies deposits in banks, money market funds and cash invested temporarily in various instruments with maturities of three months or less at the time of purchase as cash and cash equivalents. At December 31, 2018 and 2017, cash and cash equivalents consisted principally of U.S. Treasury notes, amounts held in money market accounts and cash on deposit at commercial banks.

### *Short-Term Investments*

The Company generally invests its excess cash in money market funds and short-term investments in U.S. Treasury notes. Such investments included in short-term investments on the Company's consolidated balance sheets are considered available-for-sale debt securities and are reported at fair value with unrealized gains and losses included as a component of shareholders' equity (deficit). Realized gains and losses, if any, on short-term investments are included in interest income (expense), net.

The Company evaluates its short-term investments with unrealized losses for other-than-temporary impairment. When assessing short-term investments for other-than-temporary declines in value, the Company considers such factors as, among other things, how significant the decline in value is as a percentage of the original cost, the Company's ability and intent to retain the investment for a period of time sufficient to allow for any anticipated recovery in fair value and market conditions in general. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the consolidated statement of operations and comprehensive loss. No such adjustments were necessary during the periods presented.

### *Concentrations of Credit Risk*

Financial instruments that potentially subject the Company to concentrations of credit risk consist principally of cash and cash equivalents. At December 31, 2018 and 2017, all of the Company's cash and cash equivalents were held at two financial institutions. The Company generally maintains balances in various operating accounts at financial institutions that management believes to be of high credit quality, in amounts that may exceed federally insured limits. The Company has not experienced any losses related to its cash and cash equivalents and does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

(Amounts in thousands, except share and per share amounts)

***Restricted Cash***

Restricted cash as of December 31, 2017 consisted of cash held in a money market fund in connection with the Company's corporate credit cards. This restricted cash amount has been classified as current assets based on the expected release date of the restrictions.

In conjunction with the Company's lease agreement entered into in March 2018 (see Note 12), the Company maintains a letter of credit for the benefit of the landlord. As of December 31, 2018, the underlying cash balance of $210 securing this letter of credit, was classified as non-current in its consolidated balance sheet.

***Property and Equipment***

Property and equipment are recorded at cost and depreciated over the estimated useful lives of the related assets using the straight-line method. Maintenance and repairs are charged to expense as incurred. When assets are retired or otherwise disposed of, the cost of these assets and related accumulated depreciation or amortization are eliminated from the consolidated balance sheet and any resulting gains or losses are included in the consolidated statement of operations and comprehensive loss in the period of disposal. The expected useful lives of the respective assets are as follows:

| | Estimated Useful Life |
|---|---|
| Computer hardware and software | 3 - 5 years |
| Laboratory and facility equipment | 5 years |
| Furniture, fixtures and vehicles | 5 - 7 years |
| Leasehold improvements | Shorter of estimated useful life or lease term |

***Impairment of Long-Lived Assets***

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

***Deferred Offering Costs***

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process preferred share or common equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction to the carrying value of convertible preferred shares or in shareholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering. Should an in-process equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the consolidated statements of operations and comprehensive loss. The Company recorded deferred offering costs related to the follow-on offering of its Class A common shares and concurrent private placement of $433 as of December 31, 2018 and deferred offering costs related to the sale of convertible preferred shares of $25 as of December 31, 2017.

***Fair Value Measurements***

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

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

The Company's restricted cash, which is held in a money market fund, is carried at fair value, determined based on Level 1 inputs in the fair value hierarchy described above (see Note 3). The Company's cash equivalents, consisting of money market accounts and U.S. Treasury notes, are carried at fair value, determined based on Level 1 and 2 inputs in the fair value hierarchy described above (see Note 3). The carrying values of the Company's prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

***Segment Information***

The Company manages its operations as a single operating segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on developing and delivering therapeutic medicines for patients suffering from debilitating diseases with significant unmet medical need.

***Research and Development Costs***

Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred to discover, research and develop drug candidates, including personnel expenses, share-based compensation expense, allocated facility-related and depreciation expenses, third-party license fees and external costs of outside vendors engaged to conduct clinical development activities and clinical trials as well as to manufacture clinical trial materials. Non-refundable prepayments for goods or services that will be used or rendered for future research and development activities are recorded as prepaid expenses. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

***Research Contract Costs and Accruals***

The Company has entered into various research and development-related contracts with companies both inside and outside of the United States. The related costs are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or clinical trials, including the phase or completion of events, invoices

received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ materially from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

***Patent Costs***

The Company charges patent-related costs in connection with filing and prosecuting patent applications to operations as incurred as their realization is uncertain. These costs are classified as general and administrative expenses.

***Share-Based Compensation***

The Company measures all share-based awards granted to employees and directors based on their fair value on the date of the grant and recognizes compensation expense for those awards over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company issues share-based awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company has not issued any share-based awards with performance-based vesting conditions.

For share-based awards granted to consultants and non-employees, compensation expense is recognized over the vesting period of the awards, which is generally the period during which services are rendered by such consultants and non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of the Company's Class A common shares and updated assumption inputs in the Black-Scholes option-pricing model.

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

The fair value of each restricted share award is estimated on the date of grant based on the fair value of the Company’s Class A or Class B common shares on that same date. The fair value of each option grant is estimated on the date of grant using the Black-Scholes option-pricing model, which requires inputs based on certain subjective assumptions, including the expected share price volatility, the expected term of the award, the risk-free interest rate, and expected dividends (see Note 8). The Company historically has been a private company and lacks company-specific historical and implied volatility information for its shares. Therefore, it estimates its expected share price volatility based on the historical volatility of publicly traded peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded share price. The expected term of the Company’s options has been determined utilizing the “simplified” method for awards that qualify as “plain-vanilla” options. The expected term of options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends on common shares and does not expect to pay any cash dividends in the foreseeable future.

***Comprehensive Loss***

Comprehensive loss includes net loss as well as other changes in shareholders’ equity (deficit) that result from transactions and economic events other than those with shareholders. For the year ended December 31, 2018, the Company’s only element of other comprehensive loss was unrealized loss on short-term investments. For the year ended December 31, 2017, there was no difference between net loss and comprehensive loss.

***Income Taxes***

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

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

***Net Income (Loss) per Share***

The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common shareholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net income (loss) per share attributable to common shareholders is computed by dividing the net income (loss) attributable to common shareholders by the weighted average number of common shares outstanding for the period. Diluted net income (loss) attributable to common shareholders is computed by adjusting net income (loss) attributable to common shareholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net income (loss) per share attributable to common shareholders is computed by dividing the diluted net income (loss) attributable to common shareholders by the weighted average number of common shares outstanding for the period, including potential dilutive common shares. For purpose of this calculation, outstanding options, unvested restricted common shares and convertible preferred shares are considered potential dilutive common shares.

Prior to the closing of its IPO, when the Company's convertible preferred shares converted to common shares, the Company's convertible preferred shares contractually entitled the holders of such shares to participate in dividends but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, for periods in which the Company reports a net loss attributable to common shareholders, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss attributable to common shareholders, diluted net loss per share attributable to common shareholders is the same as basic net loss per share attributable to common shareholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive. The Company reported a net loss attributable to common shareholders for the years ended December 31, 2018 and 2017.

***Recently Adopted Accounting Pronouncements***

In June 2018, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2018-07, "Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting." ASU 2018-07 aligns the accounting for share-based payment awards issued to employees and nonemployees as well as improves financial reporting for share-based payments to nonemployees. The ASU is effective for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years and applies to all new option awards granted after the date of adoption. Early adoption is permitted. The Company elected to early adopt ASU 2018-07 effective as of January 1, 2018 and applied it to share-based payment awards issued during year ended December 31, 2018. The adoption of this standard did not have a material impact on the Company's consolidated financial statements and related disclosures.

In May 2017, the FASB issued ASU No. 2017-09, Compensation — Stock Compensation (Topic 718): Scope of Modification Accounting ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. ASU 2017-09 is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted. The Company adopted ASU 2017-09 as of the required effective date of January 1, 2018. The adoption of ASU 2017-09 will have an impact on the modification of stock-based awards, if any, after the date of adoption. The adoption of ASU 2017-09 did not have an impact on the Company's financial position, results of operations or cash flows.

In August 2016, the FASB issued ASU No. 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments ("ASU 2016-15"). This guidance addresses diversity in practice in how certain cash receipts and cash payments are presented in the statement of cash flows. The standard is effective for fiscal years beginning after December 15, 2017, including interim periods in those fiscal years, and early adoption is permitted. The adoption of ASU 2016-15 is required to be applied retrospectively. The Company adopted ASU 2017-09 as of the required effective date of January 1, 2018, and the adoption did not have an impact on the Company's consolidated statement of cash flows.

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606) ("ASU 2014-09"), which supersedes existing revenue recognition guidance under GAAP. The standard's core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The standard defines a five-step process to achieve this principle, and will require companies to use more judgment and make more estimates than under the prior guidance. The Company expects that these judgments and estimates will include identifying performance obligations in the customer contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU 2014-09 also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts. In August 2015, the FASB issued ASU No. 2015-14, Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date, which delayed the effective date of ASU 2014-09 such that the standard is effective for public entities for annual periods beginning after December 15, 2017 and for interim periods within those fiscal years. The FASB subsequently issued amendments to ASU 2014-09 that had the same effective date and transition date. The Company adopted ASU 2014-09 as of the required effective date of January 1, 2018 and the adoption did not have an impact on the Company's consolidated financial statements as the Company does not currently have any revenue-generating arrangements.

***Recently Issued Accounting Pronouncements***

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842) ("ASU 2016-02"), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases today. The guidance is effective for annual periods beginning after December 15, 2018 and for interim periods within those fiscal years, and early adoption is permitted. This standard is effective for the Company on January 1, 2019 and the Company has elected a modified retrospective transition approach.

In July 2018, the FASB subsequently issued ASU No. 2018-11, Leases (Topic 842): Targeted Improvements ("ASU 2018-11"), which includes certain amendments to ASU 2016-02 intended to provide relief in implementing the new standard. Among these amendments is the option to not restate comparative periods presented in the financial statements. The Company has elected this transition approach, using a cumulative-effect adjustment on the effective date of the standard, with comparative periods presented in accordance with the existing guidance in ASC 840. The Company adopted the new standard on January 1, 2019 and used the effective date as its date of initial application. The Company is taking advantage of certain available expedients by electing the transition package of practical expedients permitted within ASU 2016-02, which allows the Company to not reassess previous accounting conclusions around whether arrangements are, or contain, leases, the classification of leases, and the treatment of initial direct costs. The Company also has made an accounting policy election to exclude leases with an initial term of 12 months or less from the balance sheet, as these will be accounted for similar to the prior guidance for operating leases.
While the Company is still assessing the impact of adopting the new standard, it currently expects a material impact to its consolidated balance sheet in recognizing additional lease liabilities and right-of-use assets as of January 1, 2019 related to the Company's operating leases. The Company further expects to provide enhanced disclosure about its leasing arrangements. The Company does not expect that the new standard will have a material impact on its consolidated statement of operations or cash flows.

## 3. Fair Value of Financial Assets and Liabilities

Short-term investments as of December 31, 2018 consisted of U.S. Treasury notes all of which are due within five months. As of December 31, 2018, the fair value of short-term investments was $235,328 of which the amortized cost was $235,332 and gross unrealized loss was $4. The Company did not have any short-term investments as of December 31, 2017.

(Amounts in thousands, except share and per share amounts)

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

| | Fair Value Measurements as of December 31, 2018 Using: | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Restricted cash — money market funds | $ 210 | $ — | $ — | $ 210 |
| Cash equivalents — money market funds | 29,721 | — | — | 29,721 |
| Cash equivalents — U.S. Treasury notes | — | 15,634 | — | 15,634 |
| Short-term investments - U.S. Treasury notes | — | 235,328 | — | 235,328 |
| | $ 29,931 | $ 250,962 | $ — | $ 280,893 |

| | Fair Value Measurements as of December 31, 2017 Using: | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Restricted cash — money market funds | $ 105 | $ — | $ — | $ 105 |
| Cash equivalents — money market funds | 5,487 | — | — | 5,487 |
| Cash equivalents — U.S. Treasury notes | — | 14,995 | — | 14,995 |
| | $ 5,592 | $ 14,995 | $ — | $ 20,587 |

During the years ended December 31, 2018 and 2017, there were no transfers between Level 1, Level 2 and Level 3.

The money market funds were valued using quoted prices in active markets, which represent a Level 1 measurement in the fair value hierarchy. The Company's cash equivalents as of December 31, 2018 and 2017 also consisted of U.S. Treasury notes, which are not traded on a daily basis and, therefore, represent a Level 2 measurement in the fair value hierarchy at each period end.

## 4. Property and Equipment, Net

Property and equipment, net consisted of the following:

| | December 31, 2018 | December 31, 2017 |
|---|---|---|
| Furniture, fixtures and vehicles | $ 91 | $ 168 |
| Computer hardware and software | 249 | 9 |
| Leasehold improvements | 2,676 | — |
| Lab equipment | 3,107 | — |
| Construction in progress | 552 | — |
| Total property and equipment | 6,675 | 177 |
| Less: Accumulated depreciation | (319) | (52) |
| Total property and equipment, net | $ 6,356 | $ 125 |

Depreciation expense for the years ended December 31, 2018 and 2017 was $286, and $28, respectively.

(Amounts in thousands, except share and per share amounts)

## 5. Accrued Expenses

Accrued expenses consisted of the following:

| | December 31, 2018 | December 31, 2017 |
|---|---|---|
| Accrued employee compensation and benefits | $ 5,678 | $ 1,570 |
| Accrued research and development expenses | 9,656 | 3,905 |
| Accrued legal and professional fees | 994 | 688 |
| Other | 90 | 49 |
| | $ 16,418 | $ 6,212 |

## 6. Convertible Preferred Shares

As of December 31, 2017, the Company's bye-laws, as amended and restated, designated 22,885,492 authorized shares to be issued as convertible preferred shares with a par value of $0.000273235 per share, of which 17,128,120 shares were further designated as Series A convertible preferred shares (the "Series A preferred shares") and 5,757,372 shares were further designated as Series B convertible preferred shares (the "Series B preferred shares"). In February 2018, the Company's bye-laws were further amended and restated to, among other things, effect an increase in the number of authorized convertible preferred shares with a par value of $0.000273235 per share to 35,670,093 shares, of which 12,784,601 shares were further designated as Series C convertible preferred shares (the "Series C preferred shares"). The holders of convertible preferred shares had liquidation rights in the event of a deemed liquidation that, in certain situations, was not solely within the control of the Company. Therefore, the Series A, Series B and Series C convertible preferred shares (collectively, the "Preferred Shares") were classified outside of shareholders' equity (deficit).

In October 2015, the Company issued and sold 8,028,809 Series A preferred shares at a price of $4.6707 per share (the "Series A Original Issue Price") for proceeds of $37,398, net of issuance costs of $102.

In September 2016, the Company issued and sold an additional 9,099,311 Series A preferred shares at a price of $4.6707 per share for proceeds of $42,499, net of issuance costs of $1.

In March 2017, the Company issued and sold 5,757,372 Series B preferred shares at a price of $6.9475 per share (the "Series B Original Issue Price") for proceeds of $39,873, net of issuance costs of $127.

In February 2018, the Company issued and sold 12,784,601 Series C preferred shares at a price of $15.6438 per share (the "Series C Original Issue Price") for proceeds of $190,822, net of issuance costs of $9,178.

In May 2018, upon the completion of the Company's IPO (which qualified as a "Qualified IPO" under the Company's bye-laws, as amended and restated), all of the outstanding Preferred Shares were converted into 5,546,019 Class A common shares, 1,070,502 Class B common shares, 12,995,954 Class A1 common shares and 16,057,618 Class B1 common shares in accordance with the Company's bye-laws, as amended and restated. In connection with the completion of its IPO in May 2018, the Company amended and restated its bye-laws to, among other things, authorize the issuance of undesignated preferred shares. As of December 31, 2018, no preferred shares were designated or issued.

(Amounts in thousands, except share and per share amounts)

As of December 31, 2017, the Preferred Shares consisted of the following:

| | December 31, 2017 | | | | |
|---|---|---|---|---|---|
| | Preferred Shares Designated | Preferred Shares Issued and Outstanding | Carrying Value | Liquidation Preference | Common Shares Issuable Upon Conversion |
| Series A preferred shares | 17,128,120 | 17,128,120 | $ 79,897 | $ 80,000 | 17,128,120 |
| Series B preferred shares | 5,757,372 | 5,757,372 | 39,873 | 40,000 | 5,757,372 |
| | 22,885,492 | 22,885,492 | $ 119,770 | $ 120,000 | 22,885,492 |

Prior to the conversion to common shares, the holders of the Preferred Shares had the following rights and preferences:

*Voting*

The holders of Preferred Shares were entitled to vote, together with the holders of common shares, on all matters submitted to shareholders for a vote. The holders of Series A preferred shares were entitled to the number of votes per Series A preferred share equal to the number of whole Class B common shares into which the Series A preferred shares were convertible at the time of such vote (which is ten votes for each Class B common share). The holders of Series B and Series C preferred shares were entitled to the number of votes per Series B or Series C preferred share equal to the number of whole Class A common shares into which the Series B and Series C preferred shares were convertible at the time of such vote (which is one vote for each Class A common share). Except as provided by law or by the other provisions of the Company's bye-laws, holders of Preferred Shares voted together with the holders of common shares as a single class.

The holders of Preferred Shares, voting together as a single class, were entitled to elect two directors of the Company. The holders of Preferred Shares, voting together with the holders of common shares as a single class, were entitled to elect the remaining directors of the Company, except for the one director that the holders of Class A common shares and Class B common shares, voting together as a single class were entitled to elect.

*Conversion*

Each Series A preferred share was convertible, at the option of the holder, at any time and from time to time, and without the payment of additional consideration by the holder thereof, in such manner as permitted by Bermuda law, into such number of fully paid and non-assessable Class B common shares determined by dividing the Series A Original Issue Price by the Series A Conversion Price (as defined below) in effect at the time of conversion. Each Series B preferred share was convertible, at the option of the holder, at any time and from time to time, and without the payment of additional consideration by the holder thereof, in such manner as permitted by Bermuda law, into such number of fully paid and non-assessable Class A common shares determined by dividing the Series B Original Issue Price by the Series B Conversion Price (as defined below) in effect at the time of conversion. Each Series C preferred share was convertible, at the option of the holder, at any time and from time to time, and without the payment of additional consideration by the holder thereof, in such manner as permitted by Bermuda law, into such number of fully paid and non-assessable Class A common shares determined by dividing the Series C Original Issue Price by the Series C Conversion Price (as defined below) in effect at the time of conversion.

At the time of the IPO, the Series A Original Issue Price and Series A Conversion Price were equal to $4.6707. The Series B Original Issue Price and Series B Conversion Price were equal to $6.9475, and the Series C Original Issue Price and Series C Conversion Price were equal to $15.6438. Therefore, each Series A preferred share was convertible

into one Class B common share, each Series B preferred share was convertible into one Class A common share and each Series C preferred share was convertible into one Class A common share.

Further, upon either (i) the closing of the sale of Class A common shares or Class B common shares to the public at a price of at least $15.6438 per share (subject to appropriate adjustment in the event of any share dividend, share split, combination or other similar recapitalization with respect to the applicable class of common shares) in an initial public offering resulting in at least $100,000 of gross proceeds to the Company (a "Qualified IPO") or (ii) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the outstanding Preferred Shares, voting together as a single class on an as if converted to Class A common shares basis, all outstanding Series A preferred shares would automatically be converted, in such manner as permitted pursuant to Bermuda law, into Class B common shares at the then effective conversion rate, and all outstanding Series B and Series C preferred shares would automatically be converted, in such manner as permitted pursuant to Bermuda law, into Class A common shares at the then effective conversion rate. Notwithstanding the foregoing, in the event of a mandatory conversion of preferred shares as a result of a Qualified IPO, (a) holders of Series A preferred shares could elect to receive Class B1 common shares in lieu of Class B common shares and (b) holders of Series B and Series C preferred shares could elect to receive Class A1 common shares in lieu of Class A common shares.

*Dividends*

The holders of the Preferred Shares were entitled to receive noncumulative dividends when and if declared by the Company's board of directors. The Company was not permitted to declare, pay or set aside any dividends on any other class or series of shares of the Company, other than dividends on common shares payable in common shares, unless the holders of the Preferred Share first received, or simultaneously received, a dividend on each outstanding Preferred Share equal to (i) in the case of a dividend on any class of common shares or any class or series convertible into common shares, that dividend per Preferred Share as would have equaled the product of (a) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into common shares and (b) the number of common shares issuable upon conversion of a share of the applicable series of Preferred Shares, or (ii) in the case of a dividend on any class or series that was not convertible into common shares, at a rate per Preferred Share determined by (a) dividing the amount of the dividend payable on each share of such class or series of shares by the original issue price of such class or series (subject to appropriate adjustment in the event of any bonus share, share dividend, share split, combination of or other similar recapitalization with respect to such class or series) and (b) multiplying such fraction by an amount equal to the applicable Series A, Series B or Series C Original Issue Price. Prior to the IPO, no cash dividends were declared or paid.

*Liquidation Preference*

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or deemed liquidation event (as defined below), the holders of Preferred Shares then outstanding were entitled to be paid out of the assets of the Company available for distribution to its shareholders, on a *pari passu* basis, before any payment was made to the holders of common shares by reason of their ownership thereof, an amount per share equal to the greater of (i) one times the applicable Original Issue Price, plus any dividends declared but unpaid thereon, and (ii) such amount per share as would have been payable had all Preferred Shares been converted into common shares immediately prior to such liquidation, dissolution, winding up or deemed liquidation event. Thereafter, the remaining assets of the Company available for distribution to its shareholders would have been distributed among the holders of common shares, pro rata based on the number of shares held by each such holder.

If upon any such liquidation, dissolution or winding up of the Company or deemed liquidation event, the assets of the Company available for distribution to its shareholders were insufficient to pay the holders of Preferred Shares the full amount to which they were entitled, the holders of Preferred Shares would have shared ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise have been payable in

respect of the shares held by such holders of Preferred Shares upon such distribution if all amounts payable on or with respect to such shares were paid in full.

Unless a majority of the holders of the then outstanding Preferred Shares elected otherwise, a deemed liquidation event would include a merger or consolidation (other than one in which shareholders of the Company own a majority by voting power of the outstanding shares of the surviving or acquiring company or corporation) or a sale, lease, transfer, exclusive license or other disposition of all or substantially all of the assets of the Company.

*Redemption*

The Company's bye-laws, as amended and restated, did not provide redemption rights to the holders of Preferred Shares.

## 7. Common Shares

As of December 31, 2017, the Company's bye-laws, as amended and restated, authorized the Company to issue 43,918,239 total shares with a par value of $0.000273235, of which 5,507,938 and 3,568,353 shares were designated as Class A and Class B common shares, respectively. In February 2018, the Company's bye-laws were further amended and restated to, among other things, effect an increase in the number of authorized common shares to 44,746,463 shares, of which 5,507,938 shares were designated as Class A common shares and 3,568,353 shares were designated as Class B common shares. The remaining 11,956,456 shares that were not designated as common shares or Preferred Shares as of December 31, 2017 could have been designated to any class at any time in the future by the Company's board of directors. No Class A1 common shares or Class B1 common shares were designated as of December 31, 2017.

On May 23, 2018, the Company's registration statement on Form S-1 relating to the IPO was declared effective by the SEC. On May 29, 2018, the Company completed the IPO of 8,477,777 Class A common shares at $18.00 per share for gross proceeds of $152,600. In addition, on June 22, 2018, the Company completed the sale of 1,006,425 Class A common shares to the underwriters of the IPO following the exercise of their over-allotment option to purchase additional shares at $18.00 per share for gross proceeds of $18,116. The aggregate net proceeds to the Company from the IPO, inclusive of the over-allotment option exercise, was $155,536 after deducting underwriting discounts and commissions and other offering costs.

In May 2018, upon completion of the IPO (which qualified as a "Qualified IPO" under the Company's bye-laws, as amended and restated), all outstanding Preferred Shares were converted into 5,546,019 Class A common shares, 1,070,502 Class B common shares, 12,995,954 Class A1 common shares and 16,057,618 Class B1 common shares in accordance with the Company's bye-laws, as amended and restated. In connection with the completion of the IPO in May 2018, the Company increased the authorized capital of the Company to $54,647 consisting of 200,000,000 shares of $0.000273235 par value per share and, among other things, amended the description of different classes of shares under the Amended & Restated Bye-Laws.

On February 4, 2019, the Company completed a follow-on offering of 2,654,984 Class A common shares and concurrent private placement of 2,000,000 Class A1 common shares, both at $18.26 per share for aggregate gross proceeds of $85,000. In addition, on March 1, 2019, the Company completed the sale of 161,126 Class A common shares to the underwriters of the follow-on offering following the exercise in part of their over-allotment option to purchase additional shares at $18.26 per share for gross proceeds of $2,942. The aggregate net proceeds to the Company from the follow-on offering and concurrent private placement, inclusive of the over-allotment option exercise, was $82,969 after deducting underwriting discounts and commissions and other offering costs.

The rights of the holders of the Company's Class A common shares, Class B common shares, Class A1 common shares and Class B1 common shares are identical, except with respect to voting and conversion, as described below. As of December 31, 2017, the voting, dividend and liquidation rights of the holders of the Company's common shares were subject to and qualified by the rights, powers and preferences of the holders of the Preferred Shares as set forth above. In May 2018, following the conversion of the Preferred Shares into common shares, the voting, dividend and liquidation rights of the holders of the Company's common shares were then subject to and qualified by the rights, powers and preferences of the holders of the preferred shares, if any. As of December 31, 2018, no preferred shares were designated or issued.

*Voting*

Each Class A common share entitles the holder to one vote on all matters submitted to the shareholders for a vote. Each Class B common share entitles the holder to ten votes on all matters submitted to the shareholders for a vote. Holders of Class A1 common shares and Class B1 common shares have no voting rights. As of December 31, 2017, the holders of the Class A and Class B common shares, voting together as a single class, were entitled to elect one director of the Company. The holders of the Class A and Class B common shares, voting together with the holders of the Preferred Shares, voting together as a single class, were entitled to elect the remaining directors of the Company, except for the two directors of the Company that the holders of the Preferred Shares, voting together as a single class, were entitled to elect. In May 2018, following the conversion of the Preferred Shares into common shares, the holders of Class A and Class B common shares, voting together as a single class, are entitled to elect the directors of the Company.

*Dividends*

Common shareholders are entitled to receive dividends, as may be declared by the Company's board of directors. As of December 31, 2017, any such dividends would have been subject to the preferential dividend rights of the holders of the Preferred Shares. In May 2018, following the conversion of the Preferred Shares into common shares, any such dividends would be subject to the rights, powers and preferences of the preferred shares, if any. As of December 31, 2018, no preferred shares were designated or issued. Through December 31, 2018 and 2017, no cash dividends have been declared or paid.

*Conversion*

Each Class B common share shall automatically convert into one Class A common share upon certain transfers of such shares by the holder thereof (subject to certain exceptions). Each Class B common share is convertible, at the holder's election into one Class A common share or one Class B1 common share. Each Class A1 common share is convertible into one Class A common share at the holder's election. Each Class B1 common share is convertible into one Class A common share or one Class B common share at the holder's election. There are no conversion rights associated with the Company's Class A common shares.

## 8. Share-Based Compensation

***2018 Incentive Award Plan***

In May 2018, the Company's board of directors and shareholders approved the 2018 Incentive Award Plan (the "2018 Plan"), which became effective on May 23, 2018. On the effectiveness of the 2018 Plan, the Company ceased granting awards under its 2015 Equity Incentive Plan (as amended, the "2015 Plan").

The 2018 Plan provides for the grant of incentive options, nonqualified options, share appreciation rights, restricted shares, dividend equivalents, restricted share units and other share- or cash-based awards. A total of 4,466,500 Class A common shares were initially reserved for issuance under the 2018 Plan. The number of Class A common shares

that may be issued under the 2018 Plan will automatically increase on each January 1, beginning in 2019 and continuing for each fiscal year until, and including, the fiscal year ending December 31, 2028, equal to the lesser of (i) 4% of the Class A common shares outstanding (on an as-converted basis) on the final day of the immediately preceding calendar year and (ii) a smaller number of Class A common shares determined by the Company's board of directors. No more than 27,915,000 Class A common shares may be issued under the 2018 Plan upon the exercise of incentive options. The Class A common shares underlying any awards issued under the 2018 Plan or the 2015 Plan that on or after the effective date of the 2018 Plan expire, lapse unexercised or are terminated, exchanged for cash, surrendered, repurchased, canceled without having been fully exercised or forfeited under the 2018 Plan or the 2015 Plan will be added back to the Class A common shares available for issuance under the 2018 Plan. As of December 31, 2018, 3,175,665 shares remained available for future grant.

***2015 Equity Incentive Plan***

Until May 23, 2018 (the effective date of the 2018 Plan), the 2015 Plan provided for the Company to grant qualified incentive options, nonqualified options, share grants and other share-based awards to employees and non-employees to purchase the Company's Class A common shares. On the effective date of the 2018 Plan, the Company ceased granting awards under the 2015 Plan. At that time, the 4,691,213 shares of Class A common shares subject to outstanding awards under the 2015 Plan remained reserved for issuance under the plan pursuant such awards and the 92,170 Class A common shares that had been available for future grant under the 2015 Plan were no longer authorized and reserved for issuance or available for future grant under the 2015 Plan.

As of December 31, 2017, the total number of Class A common shares authorized to be issued under the 2015 Plan was 4,794,266 shares and 1,644,893 shares were available for future grant. As of December 31, 2018, there were 4,516,621 shares of Class A common shares subject to outstanding awards under the 2015 Plan and reserved for issuance under the 2015 Plan pursuant such awards. On May 23, 2018, the effective date of the 2018 Plan, the Company ceased granting awards under the 2015 Plan and no Class A common shares were available for future grant under the 2015 Plan in connection with the 2018 Plan becoming effective.

The exercise price for incentive options was determined by the Company's board of directors. All incentive options granted to any person possessing 10% or less of the total combined voting power of all classes of shares could not have an exercise price of less than 100% of the fair market value of the Class A common shares on the grant date. All incentive options granted to any person possessing more than 10% of the total combined voting power of all classes of shares could not have an exercise price of less than 110% of the fair market value of the Class A common shares on the grant date. The option term for incentive awards could not be greater than 10 years. Incentive options granted to persons possessing more than 10% of the total combined voting power of all classes of shares could not have an option term of greater than five years. The vesting period for equity-based awards was determined by the board of directors, which was generally four to six years. For awards granted to employees and non-employees with four-year vesting terms, 25% of the option vests on the first anniversary of the grant date and the remaining shares vest equally each month for three years thereafter. For awards granted to employees with six-year vesting terms, 16% of the option vests on the first anniversary of the grant date and the remaining shares vest based on a predetermined vesting schedule for five years thereafter.

Shares that are expired, terminated, surrendered or canceled under the 2015 Plan without having been fully exercised become be available for future awards under the 2018 Plan.

***Share Option Grants During the Years Ended December 31, 2018 and 2017***

During the years ended December 31, 2018 and 2017, the Company granted options to purchase 3,114,139 and 1,545,045 Class A common shares, respectively, to employees and directors. The Company recorded share-based

(Amounts in thousands, except share and per share amounts)

compensation expense for options granted to employees and directors of $5,464 and $876 during the years ended December 31, 2018 and 2017, respectively.

During the years ended December 31, 2018 and 2017, the Company granted options to purchase 4,000 and 1,829 Class A common shares, respectively, to non-employees. The Company recorded share-based compensation expense for options granted to non-employees of $130 and $21 during the years ended December 31, 2018 and 2017, respectively.

***2018 Employee Share Purchase Plan***

In May 2018, the Company's board of directors and shareholders approved the 2018 Employee Share Purchase Plan (the "2018 ESPP"), which became effective on May 23, 2018. A total of 670,000 Class A common shares were initially reserved for issuance under the 2018 ESPP. The number of Class A common shares that may be issued under the 2018 ESPP will automatically increase on each January 1, beginning in 2019 and continuing for each fiscal year until, and including, the fiscal year ending December 31, 2028, equal to the lesser of (i) 1% of the Class A common shares outstanding (on an as-converted basis) on the final day of the immediately preceding calendar year and (ii) a smaller number of Class A common shares determined by the Company's board of directors, provided that no more than 6,420,000 Class A common shares may be issued under the 2018 ESPP. As of December 31, 2018, 648,660 Class A common shares were available for future issuance under the 2018 ESPP. In December 2018, the Company's board of directors determined that the January 1, 2019 automatic increase in shares available under the 2018 ESPP would be zero shares.

***Option Valuation***

The assumptions that the Company used to determine the grant-date fair value of options granted to employees and directors under the 2015 Plan and the 2018 Plan (collectively, the "Plans") during the years ended December 31, 2018 and 2017 were as follows, presented on a weighted-average basis:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Risk-free interest rate | 2.82 % | 1.99 % |
| Expected term (in years) | 6.40 | 6.25 |
| Expected volatility | 75.04 % | 74.18 % |
| Expected dividend yield | 0 % | 0 % |

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

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Risk-free interest rate | 2.91 % | 2.49 % |
| Expected term (in years) | 7.35 | 10.00 |
| Expected volatility | 74.18 % | 78.28 % |
| Expected dividend yield | 0 % | 0 % |

***Options***

Through December 31, 2017, all options granted by the Company under the 2015 Plan were for the purchase of Class A common shares. Until May 23, 2018 (the effective date of the 2018 Plan), the 2015 Plan provided for the

(Amounts in thousands, except share and per share amounts)

Company to grant qualified incentive share options, nonqualified share options, share grants and other share-based awards to employees, directors, and consultants to purchase the Company's Class A common shares. On May 23, 2018, the Company ceased granting awards under the 2015 Plan. At that time, shares of Class A common shares subject to outstanding awards under the 2015 Plan remained reserved for issuance under the plan pursuant such awards and shares of Class A common shares that had been available for future grant under the 2015 Plan were no longer authorized and reserved for issuance or available for future grant under the 2015 Plan. However, the 2015 Plan continues to govern the terms and conditions of the outstanding awards granted under it. Class A common shares subject to awards granted under the 2015 Plan that are forfeited, lapse unexercised or are settled in cash become available for issuance under the 2018 Plan.

The following table summarizes option activity for the year ended December 31, 2018:

| | Number of Shares | Weighted Average Exercise Price | Weighted Average Remaining Contractual Term (in years) | Aggregate Intrinsic Value |
|---|---|---|---|---|
| Outstanding as of December 31, 2017 | 3,123,064 | $ 2.75 | 8.82 | $ 6,010 |
| Granted | 3,118,139 | $ 17.49 | | |
| Exercised | (25,683) | $ 3.39 | | |
| Forfeited | (254,581) | $ 7.50 | | |
| Outstanding as of December 31, 2018 | 5,960,939 | $ 10.25 | 8.56 | $ 108,352 |
| Options exercisable as of December 31, 2018 | 1,798,223 | $ 2.62 | 7.41 | $ 45,794 |
| Options unvested as of December 31, 2018 | 4,162,716 | $ 13.55 | 9.06 | $ 62,558 |

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

During the year ended December 31, 2018, option holders exercised 25,683 options for Class A common shares with an intrinsic value of $411 for total cash proceeds to the Company of $87. There were no options exercised during the year ended December 31, 2017.

The weighted-average grant-date fair value per share of options granted during the years ended December 31, 2018 and 2017 was $11.96 and $2.57, respectively.

The total fair value of options vested during the years ended December 31, 2018 and 2017 was $2,255 and $445, respectively.

***Restricted Shares***

Under terms of the Class A and Class B restricted share agreements covering the Class A and Class B common shares, restricted common shares are subject to a vesting schedule. The restricted shares vest over a four-year period during which time the Company has the right to repurchase up to all unvested shares at the amount paid if the relationship between the recipient and the Company ceases. Subject to the continued employment (or other engagement of the recipient by the Company as described in the restricted share agreements), all of the restricted common shares become fully vested within four years of the date of issuance.

(Amounts in thousands, except share and per share amounts)

The following table summarizes restricted share activity for the year ended December 31, 2018:

| | Class A | | Class B | |
|---|---|---|---|---|
| | Number of Shares | Weighted Average Grant Date Fair Value | Number of Shares | Weighted Average Grant Date Fair Value |
| Unvested restricted shares outstanding as of December 31, 2017 | 312,229 | $ 0.000273235 | 1,635,495 | $ 0.000273235 |
| Granted | — | — | — | — |
| Vested | (178,417) | $ 0.000273235 | (892,088) | $ 0.000273235 |
| Unvested restricted shares outstanding as of December 31, 2018 | 133,812 | $ 0.000273235 | 743,407 | $ 0.000273235 |

The aggregate fair value of restricted shares that vested during the years ended December 31, 2018 and 2017 was $15,182 and $3,973, respectively.

***Share-Based Compensation***

Share-based compensation expense was classified in the consolidated statements of operations and comprehensive loss as follows:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Research and development expenses | $ 2,285 | $ 324 |
| General and administrative expenses | 3,416 | 573 |
| | $ 5,701 | $ 897 |

As of December 31, 2018, total unrecognized compensation cost related to the unvested share-based awards was $34,820, which is expected to be recognized over a weighted average remaining period of 3.44 years.

## 9. License and Acquisition Agreements

***Biogen Asset Purchase Agreement***

In September 2016, the Company entered into an asset purchase agreement (the "Biogen Agreement") with Biogen MA Inc. ("Biogen") to acquire all of Biogen's right, title and interest in and to certain assets used in or relating to KPL-716 and other antibodies covered by certain patent rights, including patents and other intellectual property rights, clinical data, know-how, and clinical drug supply. In addition, Biogen granted to the Company a non-exclusive, sublicensable, worldwide license to certain background patent rights related to the KPL-716 program. The Company is obligated to use commercially reasonable efforts to develop and commercialize such acquired products.

In exchange for these rights, the Company made an upfront payment to Biogen of $11,500 and a technology transfer payment of $500. The Company accounted for the acquisition of technology as an asset acquisition because it did not meet the definition of a business. The Company recorded the upfront payment and technology transfer payment as research and development expense in the consolidated statement of operations and comprehensive loss because the acquired technology represented in-process research and development and had no alternative future use.

Under the Biogen Agreement, the Company is obligated to make milestone payments to Biogen of up to $179,000 upon the achievement of specified clinical and regulatory milestones in multiple indications in various

territories, including a $10,000 payment due upon the achievement of a specified clinical milestone event which may be met in the first half of 2019. During the year ended December 31, 2017, the Company made a milestone payment of $4,000 associated with the achievement of a specified clinical milestone event. Additionally, the Company could be obligated to make up to an aggregate of up to $150,000 of payments upon the achievement of specified annual net sales milestones and to pay tiered royalties on escalating tiers of annual net sales of licensed products starting in the high single-digit percentages and ending below the teens.

The Company also agreed to pay certain obligations under third-party contracts retained by Biogen that relate to the KPL-716 program. Under these retained contracts, the Company paid a one-time upfront sublicense fee of $150 and is obligated to pay insignificant annual maintenance fees as well as clinical and regulatory milestone payments of up to an aggregate of $1,575. The Company made insignificant payments in connection with the retained contracts during the years ended December 31, 2018 and 2017.

The Biogen Agreement will terminate upon the expiration of all payment obligations with respect to the last product in all countries in the territory. The Company has the right to terminate the agreement with 90 days' prior written notice. Both parties may terminate by mutual written consent or in the event of material breach of the agreement by the other party that remains uncured for 90 days (or 30 days for payment-related breaches).

The Company did not incur any research and development expense, other than insignificant payments in connection with the retained contracts, under the Biogen Agreement during the year ended December 31, 2018. During the year ended December 31, 2017, the Company recorded research and development expense of $4,169, in connection with milestone and other payments due under the Biogen Agreement.

***Novo Nordisk License Agreement***

In August 2017, the Company entered into a license agreement (the "Novo Nordisk Agreement") with Novo Nordisk A/S ("Novo Nordisk"), pursuant to which the Company has been granted an exclusive, sublicensable, worldwide license under certain intellectual property rights controlled by Novo Nordisk to make, use, develop and commercialize KPL-045 for all indications. The Company is obligated to use commercially reasonable efforts to develop and commercialize such licensed products.

In consideration for the license, the Company made an upfront payment of $1,500 to Novo Nordisk. The Company accounted for the acquisition of technology as an asset acquisition because it did not meet the definition of a business. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations and comprehensive loss because the acquired technology represented in-process research and development and had no alternative future use.

Under the Novo Nordisk Agreement, the Company was also required to make a payment of $150 upon completion of the technology transfer by Novo Nordisk. The technology was transferred during the year ended December 31, 2018 and, as a result, this payment was made and is recorded in the Company's consolidated statement of operations for the year ended December 31, 2018. In addition, the Company is obligated to make milestone payments upon the achievement of specified clinical, regulatory and initial sales milestones and upon the achievement of annual net sales thresholds, including a payment of $1,000 upon the earlier to occur of a specified regulatory milestone and January 2020, unless the Novo Nordisk Agreement is earlier terminated by either party. As of December 31, 2018 and December 31, 2017, the Company determined that the payment related to the milestone was not probable and, therefore, no amount was recorded in the Company's consolidated statement of operations and comprehensive loss during years ended December 31, 2018 and 2017. The Company has also agreed to pay royalties on annual net sales of products licensed under the agreement.

Under the Novo Nordisk Agreement, the Company is solely responsible for all development, regulatory and commercial activities and costs. The Company is also responsible for costs related to filing, prosecuting and maintaining the licensed patent rights.

The Novo Nordisk Agreement will terminate upon expiration of the last-to-expire royalty term for any licensed product in the territories, as defined in the agreement. Either party may terminate the agreement upon the other party's insolvency or bankruptcy or for uncured material breach of the agreement by the other party. Novo Nordisk has the right to terminate the agreement if the Company challenges any of the licensed patent rights. The Company may also terminate the agreement for any reason upon prior written notice to Novo Nordisk.

During the year ended December 31, 2018, the Company recorded research and development expense of $154, in connection with milestone payments due under the Novo Nordisk Agreement. During the year ended December 31, 2017, the Company recorded research and development expense of $1,500, in connection with the upfront payment due under the Novo Nordisk Agreement.

***Primatope Stock Purchase Option Agreement***

In September 2017, the Company entered into a stock purchase option agreement (the "Primatope Agreement") with Primatope Therapeutics, Inc. ("Primatope"), pursuant to which the Company was granted a license to certain intellectual property rights owned or controlled by Primatope to research, develop, and manufacture the pre-clinical antibody, KPL-404.

The agreement provided the Company with an exclusive call option to purchase 100% of the equity securities of Primatope. Upon execution of the agreement, the Company made $500 in upfront payments for the initial option period through April 2018 (the "Initial Option Period"). The Primatope Agreement allowed for up to three extensions of the Initial Option Period through January 2019 (including the initial option period, the "Option Period") for total extension payments of up to $800. Through December 31, 2018, the Company made payments totaling $800 to extend the Option Period to January 15, 2019. During the Option Period, the Company could conduct research and pre-clinical work to assess the viability of the asset.

In January 2019, the Company exercised the call option. In March 2019, the Company acquired all of the issued and outstanding equity securities of Primatope in exchange for upfront consideration of $10,000 at closing as well as milestone payments of up to $8,000 ($5,000 of which had been achieved as of the closing date and was paid at closing) each paid or payable in a combination of cash and our Class A common shares (inclusive of escrow and holdback amounts) in accordance with the terms and conditions of our stock purchase option agreement with Primatope.

The Company determined that the call option represented a variable interest in Primatope and that Primatope is a VIE. However, as the Company had no ability to control the board of directors or direct the ongoing activities of Primatope during the Option Period, the Company did not have power over the activities that most significantly impact Primatope's economic performance and was not the primary beneficiary of Primatope. As a result, the Company did not consolidate the assets, liabilities, and results of operations of Primatope. At the closing of the acquisition, Primatope became a wholly owned subsidiary of the Company.

During the year ended December 31, 2018, the Company recorded research and development expense of $800, related to the extension of the option period under the Primatope Agreement. During the year ended December 31, 2017, the Company recorded research and development expense of $500, in connection with upfront payments related to the Initial Option Period under the Primatope Agreement.

***Regeneron License Agreement***

In September 2017, the Company entered into a license agreement (the "Regeneron Agreement") with Regeneron Pharmaceuticals, Inc. ("Regeneron"), pursuant to which the Company has been granted an exclusive, sublicensable license under certain intellectual property rights controlled by Regeneron to develop and commercialize rilonacept in certain fields and territories. The Company is obligated to use commercially reasonable efforts to develop and commercialize such licensed products.

In exchange for these rights, the Company made an upfront payment of $5,000. The Company accounted for the acquisition of technology as an asset acquisition because it did not meet the definition of a business. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations and comprehensive loss because the acquired technology represented in-process research and development and had no alternative future use.

Under the Regeneron Agreement, the Company is also obligated to make payments to Regeneron of up to an aggregate of $27,500 upon the achievement of specified regulatory milestones. Upon commercialization of the licensed products, the parties will share profits equally, after deducting certain commercialization expenses subject to specified limits.

Under the Regeneron Agreement, the Company is solely responsible for all development and commercialization activities and costs in its territories. The Company is also responsible for costs related to the filing, prosecution and maintenance of certain licensed patent rights.

The parties also entered into a clinical supply agreement under which Regeneron agreed to manufacture the developed product during the clinical phase. During the years ended December 31, 2018 and 2017, the Company recorded research and development expense of $1,835 and $208, respectively, related to the purchase of drug materials under this agreement. As of December 31, 2018, the Company has non-cancelable purchase commitments under the clinical supply agreement (see Note 12).

The Regeneron Agreement will expire when the Company is no longer developing or commercializing any licensed product under the Regeneron Agreement. Either party may terminate the agreement upon the other party's insolvency or bankruptcy or for material breach of the agreement by the other party that remains uncured for 90 days (or 30 days for payment-related breaches). Regeneron has the right to terminate the agreement if the Company suspends its development or commercialization activities for a consecutive 12-month period or does not grant a sublicense to a third-party to perform such activities, or if the Company challenges any of the licensed patent rights. The Company may terminate the agreement at any time that is 18 months after the effective date of the agreement with 180 days' written notice or with one years' written notice if the Company terminates the agreement following U.S. marketing approval of a rilonacept product developed by the Company. The Company may also terminate the agreement with three month's written notice if the products are determined to have certain safety concerns.

The Company did not incur any research and development expense directly related to milestone payments due under the Regeneron Agreement during the year ended December 31, 2018. During the year ended December 31, 2017, the Company recorded research and development expense of $5,208, in connection with the agreements with Regeneron.

***MedImmune License Agreement***

In December 2017, the Company entered into a license agreement (the "MedImmune Agreement") with MedImmune, Limited ("MedImmune"), pursuant to which MedImmune granted the Company an exclusive, sublicensable, worldwide license to certain intellectual property rights to make, use, develop and commercialize mavrilimumab. Under the MedImmune Agreement, the Company also acquired reference rights to relevant

manufacturing and regulatory documents and MedImmune's existing supply of mavrilimumab drug substance and product. The Company is obligated to use commercially reasonable efforts to develop and commercialize the licensed products.

In exchange for these rights, the Company made an upfront payment of $8,000. The Company accounted for the acquisition of technology as an asset acquisition because it did not meet the definition of a business. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations and comprehensive loss because the acquired technology represented in-process research and development and had no alternative future use. In addition, the Company is obligated to make clinical, regulatory and initial sales milestone payments of up to $72,500 in aggregate for the first two indications, including, a $5,000 pass-through payment due upon the achievement of a specified clinical milestone event which was met in the fourth quarter of 2018. Also included is a milestone payment of $10,000 due upon the earlier to occur of a specified regulatory milestone and December 31, 2018, unless the MedImmune Agreement is earlier terminated by either party. As of December 31, 2018 and 2017, the Company determined that the payment related to this milestone was probable and, therefore, recognized research and development expense and an accrued milestone of $10,000 during the year ended December 31, 2017. In addition, the Company is obligated to make clinical and regulatory milestone payments of up to $15,000 in the aggregate for each subsequent indication. The Company is obligated to make milestone payments to MedImmune of up to $85,000 upon the achievement of annual net sales thresholds up to, but excluding, $1,000,000 in annual net sales as well as additional milestone payments aggregating up to $1,100,000 upon the achievement of additional specified annual net sales thresholds starting at $1,000,000 and higher. The Company has also agreed to pay tiered royalties on escalating tiers of annual net sales of licensed products starting in the low double-digit percentages and ending at twenty percent. Royalty rates are subject to reductions upon certain events.

The Company is solely responsible for all development, manufacturing, and commercial activities and costs of the licensed products, including clinical studies or other tests necessary to support the use of a licensed product. The Company is also responsible for costs related to the filing, prosecution and maintenance of the licensed patent rights.

The MedImmune Agreement will expire upon the expiration of the royalty term in the last country for the last indication, as defined in the agreement. Either party may terminate the agreement upon the other party's insolvency or bankruptcy or for material breach of the agreement by the other party that remains uncured for 90 days. MedImmune has the right to terminate the agreement if the Company challenges any of the licensed patent rights. The Company may terminate the agreement at any time upon 90 days' prior written notice.

During the year ended December 31, 2018, the Company recorded research and development expense and an accrued milestone of $5,000 related to a pass-through payment due upon the achievement of a specified clinical milestone event due under the MedImmune Agreement. During the year ended December 31, 2017, the Company recorded research and development expense of $18,000, including $8,000 in connection with the upfront payment and $10,000 in connection with an accrued milestone payment that is due at the earlier of December 31, 2018 or specified regulatory milestone due under the MedImmune Agreement.

## 10. Income Taxes

As a company incorporated in Bermuda, the Company is principally subject to taxation in Bermuda. Under the current laws of Bermuda, tax on a company's income is assessed at a zero percent tax rate. As a result, the Company has not recorded any income tax benefits from its losses incurred in Bermuda during each reporting period, and no net operating loss carryforwards will be available to the Company for those losses.

In August 2015, the Company entered into agreements with its wholly owned subsidiary, Kiniksa US, under which Kiniksa US provides management and research and development services to the Company for which the Company pays costs plus a service fee. Kiniksa US is subject to tax for federal and state tax purposes. On December 22,

(Amounts in thousands, except share and per share amounts)

2017, the United States enacted new tax reform ("Tax Cuts and Jobs Act"). The Tax Cuts and Jobs Act contains provisions with separate effective dates but is generally effective for taxable years beginning after December 31, 2017. Beginning with the year ending December 31, 2018, the corporate statutory rates on U.S. earnings was reduced from a top marginal rate of 35% to a flat rate of 21%. On December 22, 2017, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118"), which allowed the Company to record provisional amounts for the impact of the Tax Cuts and Jobs Act during a measurement period which is similar to the measurement period used when accounting for business combinations. During the year ended December 31, 2018, the Company completed its accounting for the enactment date income tax effects of the Tax Cuts and Jobs Act in accordance with SAB 118 and recorded immaterial adjustments as a result. The impact of the future rate reduction resulted in a provision for income taxes of $69 for the year ended December 31, 2017 relating to the revaluation of the Company's net deferred tax assets.

In December 2018, the Company formed a wholly owned subsidiary in the United Kingdom, Kiniksa UK. Kiniksa UK is subject to taxation in the United Kingdom.

Income (loss) before provision for income taxes consisted of the following:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Bermuda | $ (105,562) | $ (65,391) |
| Foreign (U.S. and U.K.) | 2,121 | 520 |
| | $ (103,441) | $ (64,871) |

The components of the Company's income tax provision for the years ended December 31, 2018 and 2017 are as follows:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Current income tax (provision): | | |
| Bermuda | $ — | $ — |
| U.S. federal | (547) | (184) |
| U.S. state | (217) | (15) |
| Total current income tax (provision) | (764) | (199) |
| Deferred income tax benefit: | | |
| Bermuda | — | — |
| U.S. federal | 542 | 87 |
| U.S. state | 436 | 110 |
| Total deferred income tax benefit | 978 | 197 |
| Total benefit (provision) for income taxes | $ 214 | $ (2) |

(Amounts in thousands, except share and per share amounts)

A reconciliation of the Bermuda statutory income tax rate of 0% to the Company's effective income tax rate is as follows:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Bermuda statutory income tax rate | — % | — % |
| Foreign (U.S.) tax rate differential | (1.0) | (0.4) |
| Research and development tax credits | 1.5 | 0.5 |
| 2017 Tax Cuts and Jobs Act | — | (0.1) |
| Stock-based compensation | 0.1 | — |
| State Taxes, net of Federal | (0.4) | — |
| Effective income tax rate | 0.2 % | (0.0)% |

Net deferred tax assets consisted of the following:

| | December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Research and development tax credit carryforwards | $ 75 | $ 90 |
| Depreciation and amortization | (639) | (14) |
| Share-based compensation | 1,000 | — |
| Accrued expenses and other | 829 | 189 |
| Total deferred tax assets | 1,265 | 265 |
| Valuation allowance | (49) | (27) |
| Net deferred tax assets | $ 1,216 | $ 238 |

As of December 31, 2018 and 2017, the Company had state research and development tax credit carryforwards of approximately $95 and $113 respectively, available to reduce future tax liabilities, which begin to expire in 2033.

As required by ASC 740, the Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. In order to utilize state research and development tax credits, the Company will need taxable income in the jurisdiction of where the credit was generated. The Company currently has no taxable income in certain state jurisdictions and thus management has determined that it is more likely than not that the Company will not recognize the benefits of state research and development tax credits generated in those jurisdictions, and as a result, a valuation allowance of $49 and $27 has been established at December 31, 2018 and 2017, respectively. The remaining deferred tax assets will be fully utilized in the United States based on future income generated under the cost-plus arrangement in place.

Utilization of the state research and development tax credits may be subject to substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership changes that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain shareholders or public groups in the stock of a corporation by more than 50% over a three-year period.

(Amounts in thousands, except share and per share amounts)

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2018 and 2017 were due primarily to an increase in state research and development tax credits and were as follows:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Valuation allowance at beginning of year | $ (27) | $ (10) |
| Increases recorded to income tax provision | (22) | (17) |
| Valuation allowance at end of year | $ (49) | $ (27) |

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2018 or 2017. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision. As of December 31, 2018 and 2017, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts had been recognized in the Company's consolidated statements of operations and comprehensive loss.

The Company files income tax returns in the United States and certain state jurisdictions. Kiniksa US's federal and state income tax returns are subject to tax examinations for the tax years ended December 31, 2015 and subsequent years. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service and state tax authorities to the extent utilized in a future period. There are currently no income tax examinations pending.

## 11. Net Loss per Share

The rights, including the liquidation and dividend rights, of the holders of Class A, Class B, Class A1 and Class B1 common shares are identical, except with respect to voting, transferability and conversion (see Note 7). As the liquidation and dividend rights are identical, losses are allocated on a proportionate basis and the resulting net loss per share attributed to common shareholders will, therefore, be the same for both Class A and Class B common shares on an individual or combined basis.

Basic and diluted net loss per share attributable to common shareholders was calculated as follows:

| | Years Ended December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Numerator: | | |
| Net loss attributable to common shareholders | $ (103,227) | $ (64,873) |
| Denominator: | | |
| Weighted average common shares outstanding—basic and diluted | 29,547,427 | 1,809,751 |
| Net loss per share attributable to common shareholders— basic and diluted | $ (3.49) | $ (35.85) |

The Company's unvested restricted common shares have been excluded from the computation of basic net loss per share attributable to common shareholders.

The Company's potentially dilutive securities, which include options, unvested restricted shares and convertible preferred shares, have been excluded from the computation of diluted net loss per share attributable to common shareholders as the effect would be to reduce the net loss per share attributable to common shareholders. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common shareholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share

(Amounts in thousands, except share and per share amounts)

attributable to common shareholders for the periods indicated because including them would have had an anti-dilutive effect:

| | As of December 31, | |
|---|---|---|
| | 2018 | 2017 |
| Options to purchase common shares | 5,960,939 | 3,123,064 |
| Unvested restricted shares | 877,219 | 1,947,724 |
| Convertible preferred shares | — | 22,885,492 |
| | 6,838,158 | 27,956,280 |

## 12. Commitments and Contingencies

***Lease Agreements***

On July 24, 2015, Kiniksa US entered into an operating lease in Wellesley Hills, Massachusetts for office space that comprised the former headquarters for Kiniksa US. In March 2016, effective August 1, 2016, Kiniksa US entered into an expansion and extension on its lease, which expanded its leased space to a total of 10,800 square feet. On March 31, 2017, Kiniksa US renewed this lease and extended the lease term to August 2018. Monthly lease payments, inclusive of base rent and ancillary charges, were $27.

On March 13, 2018, Kiniksa US entered into an operating lease in Lexington, Massachusetts for office and laboratory space that comprises the new headquarters for Kiniksa US and on June 26, 2018, Kiniksa US entered into an amendment to the lease expanding the rentable space to a total of 27,244 square feet. On November 7, 2018, Kiniksa US entered into an amendment (the "Third Amendment") to the lease expanding the rentable space to a total of 55,924 square feet which will be occupied in phases through December 2019. The lease expires on July 31, 2021. Monthly lease payments include base rent, as well as, ancillary charges such as the share of operating expenses and real estate taxes. Base rent under the Third Amendment increased from $73 to $101 as of January 1, 2019 and will increase up to $138 by the earlier of occupation of the certain additional expansion space or to $138 in December 2019.

On December 21, 2018, Kiniksa US entered into an operating lease in San Diego, California for office space comprising a total of 4,400 square feet. The lease commenced on January 1, 2019 and expires on December 31, 2020. Monthly lease payments for base rent are $13. Additional fees for ancillary charges such as the share of operating expenses, parking and real estate taxes are not included in the base rent.

The following table summarizes the future minimum lease payments under non-cancelable operating lease commitments, for the Lexington and San Diego offices, as of December 31, 2018:

| Year Ending December 31, | |
|---|---|
| 2019 | $ 1,394 |
| 2020 | 1,821 |
| 2021 | 972 |
| 2022 and thereafter | — |
| | $ 4,187 |

The Company recognizes rent expense on a straight-line basis over the respective lease period and has recorded deferred rent for rent expense incurred but not yet paid. The Company recorded rent expense of $935 and $402 during the years ended December 31, 2018 and 2017, respectively.

***License Agreements***

The Company has entered into license agreements with various parties under which it is obligated to make contingent and non-contingent payments (see Note 9).

***Manufacturing Commitments***

During the year ended December 31, 2018, the Company entered into agreements with several contract manufacturing organizations to provide pre-clinical and clinical trial materials. As of December 31, 2018, the Company had non-cancelable purchase commitments under these agreements totaling $12,012, which are due during the year ending December 31, 2019.

***Indemnification Agreements***

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2018 or 2017.

***Legal Proceedings***

The Company is not a party to any litigation and does not have contingency reserves established for any litigation liabilities.

## 13. Benefit Plans

The Company has established a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code. This plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. The Company provides matching contributions of 100% of the first 3% of each participant's salary contributed, plus 50% for each of the next 2% contributed. Employees are immediately and fully vested in their own contributions and the Company's match. During the years ended December 31, 2018 and 2017, the Company contributed $315 and $264, respectively, to the plan.

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## MANAGEMENT TEAM

**Sanj K. Patel***
*Chief Executive Officer & Chairman of the Board*

**Stephen Mahoney***
*President & Chief Operating Officer*

**John F. Paolini, MD, PhD***
*Senior Vice President,*
*Chief Medical Officer*

**Chris Heberlig***
*Executive Vice President, Chief Financial Officer & Treasurer*

**Thomas Beetham***
*Executive Vice President,*
*Chief Legal Officer & Corporate Development, Secretary*

**Christine Maurer**
*Senior Vice President, Program Management*

**Eben Tessari**
*Senior Vice President, Chief Business Officer*

**Raj Kannan**
*Senior Vice President, Chief Commercial Officer*

**Carsten Boess**
*Executive Vice President, Corporate Affairs*

**Martina Struck, PhD**
*Vice President, Regulatory Affairs*

**Melissa Manno**
*Vice President, Human Resources*

** Executive officers as defined under Rule 3b-7 under the Securities Exchange Act of 1934, as amended.*

## BOARD OF DIRECTORS

### Chairman

**Sanj K. Patel**
*Chief Executive Officer*

### Lead Independent Director

**Felix J. Baker, PhD**
*Co-Managing Member, Baker Bros. Advisors LP*

### Directors

**Stephen R. Biggar, MD, PhD**
*Partner, Baker Bros. Advisors LP*

**Richard S. Levy**
*Biopharmaceutical Consultant*

**Thomas R. Malley**
*President, Mossrock Capital, LLC*

**Tracey L. McCain**
*Executive Vice President, Chief Legal and*
*Compliance Officer, Blueprint Medicine Corporation*

**Kimberly J. Popovits**
*Chairman of the Board, Chief Executive Officer & President,*
*Genomic Health, Inc.*

**Barry D. Quart, PharmD**
*President, Chief Executive Officer & Director,*
*Heron Therapeutics, Inc.*

## ADDRESS

Kiniksa Pharmaceuticals, Ltd.
Clarendon House
2 Church Street
Hamilton HM 11
Bermuda

## WEBSITE

Kiniksa.com

## LEGAL COUNSEL

Latham & Watkins LLP
Boston, Massachusetts

Conyers Dill & Pearman Limited
Hamilton, HM CX, Bermuda

## INDEPENDENT REGISTERED ACCOUNTING FIRM

PricewaterhouseCoopers LLP
Boston, Massachusetts

## TRANSFER AGENT AND REGISTRAR

American Stock Transfer & Trust Company, LLC
Brooklyn, New York

## ANNUAL MEETING

**Date**
Wednesday, May 29, 2019

**Time**
7:30 a.m., ADT

**Location**
Fairmont Southampton Hotel
101 South Shore Road
Southampton SN02
Bermuda

## STOCK INFORMATION

Nasdaq Global Select Market: KNSA

## INVESTOR RELATIONS

**Mark Ragosa**
*Vice President, Investor Relations*
ir@kiniksa.com

*This Annual Report contains forward-looking statements that involve risks, uncertainties and other important factors that could cause results to differ materially from those projected. In some cases, you can identify these statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "goal," "design," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or their negative or other similar expressions. These important factors include those discussed in our Annual Report on Form 10-K for the year ended December 31, 2018 (which forms a part of this Annual Report) under the caption "Risk Factors." Accordingly, you are cautioned not to place undue reliance on such statements. We undertake no obligation to update any forward-looking statements.*

*Unless otherwise expressly stated, we obtained the industry, business, market and other data contained in this Annual Report from reports, research surveys, clinical trials, studies and similar data prepared by market research firms and other third parties, from industry, medical and general publications, and from government data and similar sources.*

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