ANNUAL REPORT 2019

Every Second Counts!™

Dear Fellow Shareholders,

I hope this letter finds you well as we adjust our routines and navigate the impact of the coronavirus. At Kiniksa, the health and safety of our employees as well as the patients and people participating in and operating our clinical trials are of paramount importance. We entered 2020 with our clinical-stage assets having the potential to generate data-driven value; these milestones remain on track thanks to the utmost dedication of our team. We will continue to monitor the pandemic and plan to adapt as necessary.

Kiniksa is a clinical-stage biopharmaceutical company focused on developing immune-modulating medicines for the benefit of patients suffering from devastating diseases. Through highly-dedicated clinical and corporate execution, 2019 represented a significant step toward this vision.

Our clinical-stage assets are based on strong biologic rationale and/or validated mechanisms.

- Rilonacept is an inhibitor of interleukin-1 alpha (IL-1α) and interleukin-1 beta (IL-1β), cytokines that have been shown to play a role in inflammatory diseases.
- Mavrilimumab is a monoclonal antibody inhibitor of granulocyte macrophage colony stimulating factor (GM-CSF) receptor signaling. GM-CSF is a key growth factor and cytokine in autoinflammation and autoimmunity.
- Vixarelimab (KPL-716) is a monoclonal antibody inhibitor of signaling through oncostatin M receptor beta (OSMRβ), the shared receptor subunit for interleukin-31 (IL-31) and oncostatin M (OSM) signaling. IL-31 and OSM are key cytokines implicated in inflammation, pruritus, and fibrosis.
- KPL-404 is a monoclonal antibody inhibitor of the CD40-CD40 ligand (CD40L) interaction, a key T-cell co-stimulatory signal critical for B-cell maturation and immunoglobulin class switching.

Additionally, our clinical-stage assets target diseases with significant unmet medical need and offer the potential for differentiation.

- We are evaluating rilonacept for the potential treatment of recurrent pericarditis, a painful autoinflammatory cardiovascular disease, in a pivotal Phase 3 trial. There are no U.S. Food and Drug Administration (FDA)-approved therapies for recurrent pericarditis.
- We are evaluating mavrilimumab for the potential treatment of giant cell arteritis (GCA), a chronic inflammatory disease of medium-to-large arteries, in a Phase 2 trial. While there is one FDA-approved therapy for GCA, we believe mavrilimumab's mechanism of action acts upstream and that an unmet medical need remains. We are also exploring the potential for mavrilimumab to treat a host of vasculitides and oncologic indications.
- We are evaluating vixarelimab for the potential treatment of prurigo nodularis, a chronic inflammatory skin condition, in a Phase 2a trial. There are no FDA-approved therapies for prurigo nodularis. We are also investigating the potential for vixarelimab to treat diseases characterized by chronic pruritus in an exploratory Phase 2 trial.
- We are evaluating KPL-404 in a single-ascending-dose trial in healthy volunteers to measure safety data and pharmacokinetics as well as receptor occupancy and T-cell Dependent Antibody Response (TDAR). The CD40-CD40L pathway has been established in a broad range of autoimmune diseases, such as rheumatoid arthritis, Sjogren's syndrome, Graves' disease, systemic lupus erythematosus and solid organ transplant.

In 2019, each of our clinical-stage assets posted key accomplishments.

- Rilonacept: The FDA granted Breakthrough Therapy designation for rilonacept for the treatment of recurrent pericarditis. Additionally, we reported final data from an open-label Phase 2 trial of rilonacept in recurrent pericarditis. The data provided first evidence that rilonacept treatment in the study improved clinically meaningful outcomes associated with the unmet medical need in recurrent pericarditis. We also achieved target enrollment on time for our pivotal Phase 3 trial in recurrent pericarditis (RHAPSODY) and continued to prepare for commercialization by generating evidence on disease burden, building disease awareness with payers, physicians and advocacy groups and establishing core capabilities such as distribution and patient services.
- Mavrilimumab: We achieved target enrollment on time for our Phase 2 trial in GCA and initiated a clinical collaboration with Kite, a Gilead company, to evaluate the investigational combination of Yescarta® (axicabtagene ciloleucel) and mavrilimumab in relapsed or refractory large B-cell lymphoma.
- Vixarelimab: Interim repeated-single-dose Phase 1b data for vixarelimab in subjects with moderate-to-severe atopic dermatitis showed a rapid and sustained anti-pruritic response as measured by weekly average Worst-Itch Numeric Rating Scale (WI-NRS). These results supported further development of vixarelimab in a Phase 2a trial in prurigo nodularis and an exploratory Phase 2 trial in diseases characterized by chronic pruritus.
- KPL-404: The FDA accepted our investigational new drug application for the clinical study of KPL-404, and we initiated a Phase 1 trial.

In 2020, our clinical-stage assets are expected to generate clinical data, including pivotal Phase 3 data from rilonacept, Phase 2 data from mavrilimumab, Phase 2 data from vixarelimab, and Phase 1 data from KPL-404. We believe these data have the potential to help inform our portfolio strategy and capital allocation decisions.

As we continue to execute toward our goal of becoming a generational biopharmaceutical company, we will keep science and patients at the center of our decisions. Thank you for your ongoing support.

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, 2019

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	Trading Symbol(s)	Name of each exchange on which registered
Class A Common Shares	KNSA	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 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, 2019, was approximately $304.6 million.

As of February 29, 2020, there were 55,547,374 common shares outstanding in aggregate, comprised of:

20,388,482 Class A common shares, par value $0.000273235 per share

4,105,320 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 2020 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, 2019, 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, 2019

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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, market opportunity and competition, drug product supply, collaborators, license and other strategic arrangements, the expected timeline for achievement of our clinical milestones, the timing of, and potential results from, clinical and other trials, potential marketing authorization from the FDA or regulatory authorities in other jurisdictions, potential coverage and reimbursement for procedures using our product candidates, if approved, commercial strategy and pre-commercial activities, research and development costs, timing of regulatory filings and feedback, timing and likelihood of success, plans and objectives of management for future operations and funding requirements, 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 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;
- ownership concentration of our executive officers and certain members of senior management may prevent our shareholders from influencing significant corporate decisions;
- our ability to attract and retain skilled personnel;
- our ability to execute on our strategy; and
- our ability to identify, in-license, acquire, discover or develop additional product candidates.

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 Report 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.

TRADEMARKS

We own the rights to Kiniksa® and have certain rights to trademarks that we use in connection with the operation of our business, including ARCALYST®. Kiniksa® is a trademark of Kiniksa Pharmaceuticals, Ltd. ARCALYST® is a registered trademark of Regeneron Pharmaceuticals, Inc. Solely for convenience, trademarks, service marks and trade names referred to in this Annual Report, including Kiniksa and ARCALYST, may be listed without the ®, SM and ™ symbols. We will assert, to the fullest extent under applicable law, our rights to our intellectual property. Trademarks, service marks and trade names of third parties are the intellectual property of such parties.

PART I

ITEM 1. BUSINESS.

Overview

We are a biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients with significant unmet medical need. We have a pipeline of clinical-stage product candidates, rilonacept, mavrilimumab, KPL-716 and KPL-404, that are expected to generate data-readouts throughout 2020. These product candidates are based on strong biologic rationales or validated mechanisms of action, target underserved conditions and offer the potential for differentiation. Further, by modulating different parts of the innate and adaptive immune system, we believe they have the potential to address multiple devastating diseases.

Rilonacept is a protein cytokine trap for inhibiting interleukin-1 alpha, or IL-1α, and interleukin-1 beta, 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. Currently, there is no FDA-approved therapy for the treatment of recurrent pericarditis.

We are conducting a single, pivotal, global, Phase 3 clinical trial in recurrent pericarditis, named RHAPSODY. We expect to 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 received Breakthrough Therapy designation from the FDA for rilonacept for the treatment of recurrent pericarditis in 2019, and reported final data from our open-label Phase 2 proof-of-concept clinical trial of rilonacept in a range of recurrent pericarditis populations at the American Heart Association Scientific Sessions in November 2019. The data derived from the study provided early evidence that rilonacept treatment in the study improved clinically meaningful outcomes associated with the unmet medical need in recurrent pericarditis. Rilonacept was generally well-tolerated in the study, with adverse events consistent with the FDA approved rilonacept label for the treatment of CAPS, including Familial Cold Autoinflammatory Syndrome and Muckle-Wells Syndrome. The most common adverse events were mild, transient injection site reactions that did not cause discontinuation. There was one treatment-related serious adverse event which resulted in discontinuation: a skin abscess which responded to medical treatment. Infections are included in the rilonacept label for CAPS.

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-to-large arteries with an estimated U.S. prevalence of approximately 75,000 – 150,000 patients. We licensed mavrilimumab from MedImmune Limited, or MedImmune, in 2017. There is only one FDA-approved therapy for GCA, which is an adjunct to corticosteroid therapy, and we believe an unmet need persists. We are conducting a randomized, double-blind, placebo-controlled, global Phase 2 proof-of-concept clinical trial of mavrilimumab in GCA. We expect to report top-line data in the second half of 2020.

In December 2019, we entered into a clinical collaboration with Kite, a Gilead Company, or Kite, to initiate a Phase 2 clinical trial evaluating the combination of Yescarta (axicabtagene ciloleucel) and mavrilimumab in relapsed or refractory large B-Cell lymphoma. The objective of the Phase 2 trial is to determine the effect of mavrilimumab on the safety of Yescarta. Treatment related induction of GM-CSF has been identified through clinical, translational and preclinical studies as a potential key signal associated with side effects of chimeric antigen receptor T, or CAR T, cell therapy. Preclinical evidence suggest the potential for interruption of GM-CSF signaling to disrupt CAR T cell mediated inflammation without disrupting anti-tumor activity. Kite will be the sponsor of this study and responsible for its conduct.

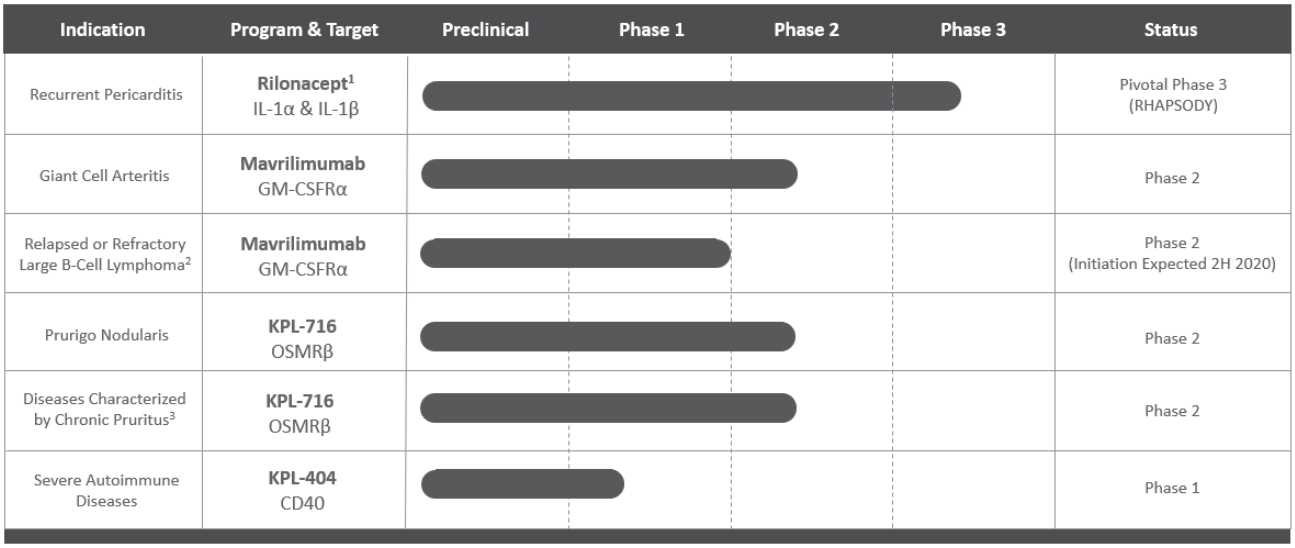
KPL-716 is a monoclonal antibody that simultaneously inhibits the signaling of the cytokines interleukin-31, or IL-31, and oncostatin M, or OSM, by targeting their common receptor subunit, oncostatin M receptor beta, or OSMRβ. We licensed KPL-716 from Biogen MA, Inc., or Biogen, in 2016. We believe KPL-716 is the only monoclonal antibody in development that simultaneously targets both pathways.

We are conducting a randomized, double-blind, placebo-controlled, Phase 2a clinical trial of KPL-716 in subjects with prurigo nodularis, a chronic inflammatory skin condition with an estimated U.S. prevalence of approximately 300,000 patients. Currently, there is no FDA-approved therapy for prurigo nodularis. We expect data from this trial by the end of April 2020. We are also conducting an exploratory Phase 2 clinical trial in diseases characterized by chronic pruritus. This randomized, double-blind, placebo-controlled trial is designed to identify chronic pruritic conditions where signaling through OSMRβ may be playing a role and to investigate the efficacy, safety and tolerability of KPL-716 in reducing the moderate-to-severe pruritus experienced by these subjects. We expect interim data from cohorts of this study in the first half of 2020.

In August 2019, we reported interim results from the repeated-single-dose Phase 1b clinical trial of KPL-716 in subjects with moderate-to-severe atopic dermatitis. Atopic dermatitis provided a proxy for evaluating target engagement in IL-31 driven pruritic diseases. Interim repeated-single-dose Phase 1b data through the 12-week treatment period showed a rapid and sustained anti-pruritic response as measured by Worst-Itch Numeric Rating Scale (WI-NRS). There were no serious adverse events. However, there were more atopic dermatitis flares in the KPL-716-treated population versus placebo (47.6% versus 4.5%); all subjects who experienced a flare were successfully managed with topical corticosteroids. KPL-716 was otherwise well-tolerated by all subjects.

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. In the first quarter of 2019, we acquired 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. In the second half of 2019, we initiated a single-ascending-dose Phase 1 clinical trial of KPL-404 in healthy volunteers. The first-in-human trial is designed to provide safety and pharmacokinetics data as well as receptor occupancy and T-cell dependent antibody response (TDAR). We expect top-line data in the second half of 2020.

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.

(2) Clinical collaboration with Kite.

(3) Chronic Idiopathic Pruritus, Chronic Idiopathic Urticaria, Plaque Psoriasis, Lichen Simplex Chronicus, Lichen Planus. IL-1α = interleukin-1 alpha ; IL-1β = interleukin 1 beta; GM-CSFRα = granulocyte macrophage colony stimulating factor receptor alpha; OSMRβ = oncostatin M receptor beta

We evaluate options across our portfolio in an effort to maximize value and improve capital allocation based on data, including potential additional indications for rilonacept, mavrilimumab and KPL-716, being opportunistic in our business development activities, considering appropriate opportunities to partner or out-license our programs, as well as conducting internal research to discover and develop molecules to expand our portfolio.

Our Team

We have assembled an experienced management team with a successful track record. Our management 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 18 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 pharmaceutical and biotechnology 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 goal is to build a fully-integrated, global biopharmaceutical company by discovering, acquiring, developing and commercializing life-changing therapies for debilitating diseases. We have a pipeline of immune-modulating, clinical-stage product candidates that are based on strong biologic rationale or validated mechanisms, target underserved conditions and offer the potential for differentiation.

Critical components of our business strategy include the following:

- **Advance Our Product Candidates Through the Development Process**. We are pursuing multiple product candidates in parallel and have advanced these programs with the goal of delivering differentiated therapies to patients. We believe that each of our product candidates have the potential to address significant unmet medical needs and intend to develop them efficiently in a data-driven manner.

- **Commercialize Our Product Candidates to Bring Therapies to Patients.** We intend to market and commercialize our product candidates, if approved, in the United States and potentially in select international markets by developing sales, marketing, medical affairs, access and reimbursement capabilities, as appropriate for the potential commercial opportunity. We believe this approach will enable us to effectively reach patients and prescribers that our product candidates target and leverage the commercial potential of our product candidates.

- **Explore Opportunities to Drive Value and Maximize the Potential of Our Existing Portfolio.** We believe that our product candidates have potential in multiple indications. Our assets are designed to modulate immunological signaling pathways that are implicated across a spectrum of diseases. We may also seek collaborations, licenses and other strategic relationships to assist in advancing and expanding our current programs, as appropriate to drive value.

- **Work to Identify, Discover, Acquire and Develop New Therapies.** We aim to leverage our internal discovery efforts and business development capabilities to complement our existing portfolio. 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. We intend to continue to be opportunistic in our business development activities.

Our Product Candidates

Rilonacept

Overview

Rilonacept was approved by the FDA for the treatment of 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 conducting a single, pivotal, global, Phase 3 clinical trial in recurrent pericarditis, named RHAPSODY. We expect to report top-line data from this trial in the second half of 2020.

There is currently no FDA-approved therapy for the treatment of recurrent pericarditis. There is currently one other FDA-approved agent that blocks both IL-1α and IL-1β signaling, anakinra (KINERET), produced by Sobi, Inc, or Sobi, is approved for other indications, and one that blocks only IL-1β, canakinumab (ILARIS), produced by Novartis Pharmaceuticals Corporation, or Novartis, is approved for other indications. We believe both therapies have limitations. Anakinra requires once-daily injections, and canakinumab blocks only IL-1β, making it less effective or potentially 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 diseases mediated by both IL-1α and IL-1β.

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 NLRB-3 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 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 patients in the United States per year. 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 all conventional treatments (approximately 3,000);
- patients who are refractory to nonsteroidal anti-inflammatory drugs, or NSAIDs / colchicine but where steroid usage is not appropriate (approximately 5,000);
- patients otherwise not well-managed and have multiple recurrences despite previously responding to NSAIDs, colchicine and/or steroids (approximately 5,000); and
- patients who are dependent on steroids (approximately 1,000).

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 post-cardiac injury, 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 conducting a pivotal, global Phase 3 clinical trial, named RHAPSODY. The trial is intended to evaluate the efficacy and safety of rilonacept treatment in subjects with recurrent pericarditis. RHAPSODY is a double-blind, placebo-controlled, RW trial, with an open-label extension period. The trial is designed to randomize up to 75 subjects into the RW period. Eligible subjects 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 NSAIDs or colchicine or oral corticosteroid treatment in any combination.

The clinical trial 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, or SC, followed by a weekly 160 mg SC dose 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 have reached our target enrollment and expect to report top-line data in the second half of 2020.

Phase 2 Study Results

In 2019, we completed a Phase 2 proof-of-concept clinical trial of rilonacept in recurrent pericarditis and reported final data at the American Heart Association Scientific Sessions in November 2019.The Phase 2 trial evaluated the treatment response to rilonacept in a range of recurrent pericarditis populations and was divided into five parts across two cohorts.

Actively symptomatic recurrent pericarditis patients:

- Part 1: Symptomatic patients with recurrent pericarditis and CRP > 1 mg/dL;
- Part 2: Symptomatic patients with recurrent pericarditis and CRP ≤ 1 mg/dL but pericardial inflammation confirmed by magnetic resonance imaging, or MRI; and
- Part 4: Symptomatic patients with post-pericardiotomy syndrome, or PPS and CRP > 1 mg/dL.

Corticosteroid-dependent recurrent pericarditis patients not actively experiencing a recurrence:

- Part 3: Asymptomatic patients with recurrent pericarditis who were dependent upon or unable to wean off corticosteroids; and
- Part 5: Asymptomatic patients with PPS who were dependent upon or unable to wean off corticosteroids.

In this study, all patients received a loading dose of rilonacept 320 mg subcutaneously, or SC, followed by 160 mg SC weekly maintenance on top of any combination of co-administered NSAIDs and/or colchicine and/or corticosteroids during a six-week base treatment period. There was an optional 18-week extension treatment period, during which physicians were given the option to wean patients off concomitant NSAIDs, colchicine, and/or corticosteroids. The assessed efficacy outcomes measures included an 11-point NRS, CRP, electrocardiogram, and echocardiogram to measure the size of the pericardial effusion. 25 unique patients enrolled in the six-week base treatment period across Parts 1 through 5 of the Phase 2 trial, and 23 patients continued into the optional 18-week extension treatment period and completed 24 weeks of treatment.

The Phase 2 data provided first evidence that rilonacept treatment in the study improved clinically meaningful outcomes associated with the unmet medical need in recurrent pericarditis.

Resolution of Pericarditis Episodes

We expect that symptomatic recurrent pericarditis patients with CRP > 1mg/dL (Parts 1 and 4; n = 13), who were failing standard of care management with NSAIDs, colchicine, and/or corticosteroids, will be the most relevant to the enrollment population for the RHAPSODY Phase 3 clinical trial. In the Phase 2 trial, a rapid reduction in both reported pain and inflammation after the first dose as well as a persistent and clinically meaningful response were observed throughout the trial for these patients.

- Mean patient-reported pericardial pain on an 11-point NRS decreased from 4.5 at baseline to 0.5 at 24 weeks;
- mean CRP decreased from 4.6 mg/dL at baseline to 0.2 mg/dL at 24 weeks; mean time to CRP normalization was nine days; and

- pericardial signs resolved or improved in all patients, including pericardial effusion (6/7 patients), PR depression (2/3 patients), widespread ST elevation (2/2 patients) and pericardial rub (2/2 patients).

In symptomatic recurrent pericarditis patients with CRP ≤ 1mg/dL (Part 2; n = 3), who were failing standard of care management with NSAIDs, colchicine, and/or corticosteroids, a reduction in both reported pain and inflammation after the first dose as well as a persistent and clinically meaningful response were observed throughout the trial.

- Mean patient-reported pericardial pain on an 11-point NRS decreased from 4.7 at baseline to 0.0 at 24 weeks; and

- mean CRP decreased from 0.46 mg/dL at baseline to 0.32 mg/dL at 24 weeks.

Tapering and Discontinuation of Corticosteroids without Pericarditis Recurrence

15 recurrent pericarditis patients on corticosteroids at baseline enrolled in the six-week base treatment period, and 13 continued into the optional 18-week extension treatment period and completed 24 weeks of treatment. During the optional 18-week extension treatment period, investigators were given the option to wean patients from concomitant medications while continuing weekly rilonacept treatment. All patients on corticosteroids stopped or tapered corticosteroids during this part of the study without experiencing a recurrent pericarditis episode.

- 11 recurrent pericarditis patients discontinued corticosteroids completely: four symptomatic patients across Parts 1 and 2 as well as seven corticosteroid-dependent patients across Parts 3 and 5.

- The corticosteroid dose was successfully tapered in the two remaining recurrent pericarditis patients by the end of the 24-week study period: one symptomatic patient in Part 2 and one corticosteroid-dependent patient in Part 3.

Reduction in Recurrences of Pericarditis Episodes while on Treatment

A comparison of the annualized incidence of pericarditis episodes during the trial while receiving rilonacept versus patients' own natural history in the period prior to the trial showed a decrease in annualized incidence of pericarditis episodes across all parts from 3.9 episodes/year prior to the study to <0.18 episodes/year.

Improved Quality of Life Scores

Rilonacept treatment also resulted in improvement of Patient Reported Outcomes Measurement Information System, or PROMIS, Global Health scores for Physical and Mental Global Health (U.S. mean score = 50.0; standard deviation = 10). A clinically meaningful difference is thought to be one standard deviation, or ten points.

- In symptomatic recurrent pericarditis patients (Parts 1, 2, and 4), the mean Physical and Mental Global Health baseline scores were 39.9 and 44.5, respectively, and improved to 51.3 and 50.5, respectively, at 24 weeks.

- In corticosteroid-dependent recurrent pericarditis patients (Parts 3 and 5), the mean Physical and Mental Global Health baseline scores were 43.3 and 46.5, respectively, and improved to 46.8 and 50.7, respectively, at 24 weeks.

Rilonacept was generally well-tolerated in the study, with adverse events consistent with the FDA-approved label for the treatment of CAPS, including Familial Cold Autoinflammatory Syndrome and Muckle-Wells Syndrome. The most common adverse events were mild, transient injection site reactions that did not cause discontinuation. There was one treatment-related serious adverse event which resulted in discontinuation: a subcutaneous abscess which responded to medical treatment. Infections are included in the rilonacept label for CAPS.

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 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 the immune system, 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 medium-to-large arteries 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, up to 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 exists.

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 that data provide a solid rationale for antagonizing this signaling with mavrilimumab.

Preclinical Rationale for GCA

At the 2019 American College of Rheumatology conference, each of Dr. Cornelia M. Weyand and Dr. Maria C. Cid presented preclinical data sponsored by Kiniksa, which we believe support the mechanistic rationale for targeting granulocyte macrophage colony stimulating factor receptor alpha, or GM-CSFRα, in patients with GCA.

Dr. Weyand made an oral presentation entitled GM-CSF is a Pro-Inflammatory Cytokine in Experimental Vasculitis of Medium and Large Arteries. Data from a validated *in vivo* model of vasculitis showed that, compared to treatment controls, mavrilimumab, reduced tissue inflammation in the arteries. In the model, normal human arteries were engrafted into immune-deficient mice. Following the engraftment, peripheral blood mononuclear cells from patients with GCA were transferred to the mice which then caused vasculitis to appear in the normal arteries within 7-10 days. After the inflammation had been established, animals were treated with mavrilimumab. Treatment with mavrilimumab resulted in statistically significant reductions in the number of CD3+ T-cells and in innate and adaptive immune responses in the inflamed arteries in addition to significant reductions in key cytokines known to play a role in GCA pathology including interferon-gamma, or IFN-γ. These data illustrate that blockade of GM-CSFRα signaling had a strong anti-inflammatory activity. We believe the reduced expression of IFN-γ in mice treated with mavrilimumab is of significance for treating GCA as it is the signature cytokine produced by the T helper type 1, or TH1, cell lineage which, along with GM-CSF, has been implicated in multinucleated giant cell formation. The TH1 signature is relatively unresponsive to glucocorticoid therapy and often persists in steroid-treated patients and is believed to mediate chronic and refractory disease.

Dr. Cid presented a poster entitled GM-CSF Pathway Signature Identified in Temporal Artery Biopsies of Patients with Giant Cell Arteritis. Data from this study examining human temporal artery biopsies from two independent sources showed the GM-CSF signaling pathway molecular signature was upregulated in GCA biopsies versus control at both the messenger ribonucleic acid (mRNA) and protein level. GM-CSF and TH1 pathway signatures (including IFN-γ) were demonstrated in GCA patient temporal arteries by independent analytical techniques. The data also demonstrated active GM-CSF signaling in diseased tissue is evidenced by increased expression of PU.1, a transcription factor downstream of GM-CSF signaling, in the vessel wall. Additionally, treatment of ex vivo cultures of GCA arteries with mavrilimumab suppressed expression of these gene products, suggesting the biological activity of mavrilimumab on genes relevant to GCA pathophysiology.

Phase 2 Clinical Trial for GCA

We are conducting a randomized, double-blind, placebo-controlled, global Phase 2 proof-of-concept trial in multiple countries. The Phase 2 clinical trial of mavrilimumab for the treatment of GCA was designed to enroll approximately 60-70 subjects with new-onset and refractory disease. Subjects are 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.

In the United States, the FDA initially placed our Investigational New Drug application, or 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 preclinical toxicology data nor the design of our trial. We provided the FDA with the requested information and the IND became active. We expect to report top-line data in the second half of 2020.

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.

Phase 2 Clinical Trial Evaluating Mavrilimumab with YESCARTA

In December of 2019, we entered into a clinical collaboration with Kite to initiate a Phase 2 clinical trial evaluating the combination of Yescarta and mavrilimumab in relapsed or refractory large B-Cell lymphoma. The objective of the trial is to determine the effect of mavrilimumab on the safety of Yescarta. Treatment related induction of GM-CSF has been identified through clinical, translational and preclinical studies as a potential key signal associated with side effects of CAR T cell therapy. Preclinical evidence suggests the potential for interruption of GM-CSF signaling to disrupt CAR T cell mediated inflammation without disrupting anti-tumor activity. Kite will be the sponsor or this study and responsible for its conduct.

Clinical History in Rheumatoid Arthritis

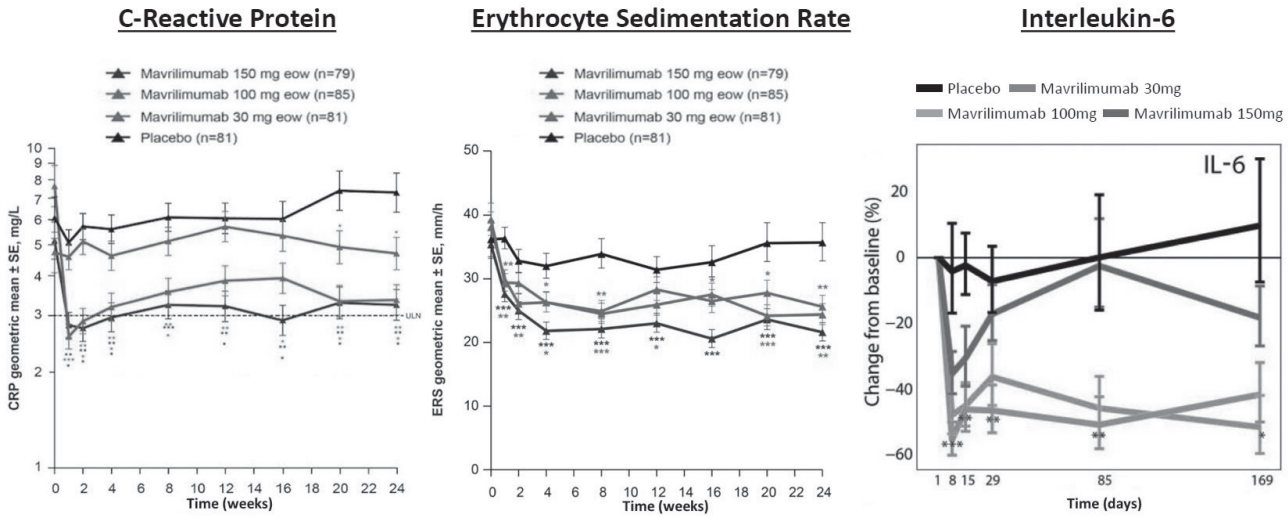
MedImmune had received authorization to conduct clinical trials for rheumatoid arthritis, or RA, in Europe and executed an extensive Phase 1 and Phase 2 clinical program where the company studied mavrilimumab in over 550 patients with RA through Phase 2b. All of MedImmune's European clinical trials achieved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



Source: Burmester GR, et al. Ann Rheum Dis 2017. *p<0.05, **p<0.01, ***p<0.001 mavrilimumab versus placebo

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 completed our Phase 1a/1b program in atopic dermatitis subjects in 2019. Both the single IV dose and repeated-single doses of KPL-716 resulted in a marked and rapid reduction in pruritus as measured by a numerical rating score. We believe these results support further development of KPL-716 as an anti-pruritic treatment for diseases with upregulated signaling through OSMRβ. We are initially evaluating KPL-716 for the treatment of a variety of pruritic diseases potentially driven by signaling through OSMRβ, including prurigo nodularis, a chronic inflammatory skin condition with an estimated U.S. prevalence of approximately 300,000 patients.

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 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 and Other Chronic Pruritic Diseases

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

Our exploratory, pilot Phase 2 clinical trial was designed 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.

Current Treatment Landscape for Prurigo Nodularis

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.

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.

Preclinical Development

In our preclinical 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 preclinical 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 ten subjects with moderate-to-severe atopic dermatitis receiving a single dose of KPL-716 7.5 mg/kg IV versus ten 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 $\geq$ 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 $\geq$ 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 Eczema Area and Severity Index, or 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.

In August of 2019 we reported interim results from the repeated-single-dose Phase 1b clinical trial. The Phase 1b trial used a weekly 360 mg SC dose in a randomized, double-blind, placebo-controlled design in order to evaluate safety and exploratory disease response markers. The 360 mg SC dose was intended to replicate and extend exposures from the prior single-ascending-dose Phase 1b clinical trial where an early signal of efficacy had been observed in reducing pruritus after a single 7.5 mg/kg intravenous dose. In the repeated-single-dose Phase 1b clinical trial, 43 subjects with moderate-to-severe atopic dermatitis were enrolled and randomized 1:1 to KPL-716 or placebo once weekly for 12 weeks. There was a seven-day wash out period of all other therapies before treatment, and topical

corticosteroids were not allowed throughout the 12-week treatment period. However, rescue medication was available for atopic dermatitis flares throughout the study. In an interim analysis of the data through the 12-week treatment period as of August 19, 2019, KPL-716 showed a rapid and sustained anti-pruritic response as measured by a reduction in Worst-Itch Numeric Rating Scale, or WI-NRS, in subjects with moderate-to-severe atopic dermatitis:

- Mean change from baseline in weekly-average WI-NRS at Week 1 was -28.1% in KPL-716 recipients compared to -6.8% in placebo recipients.

- Mean change from baseline in weekly-average WI-NRS at Week 12 was -55.0% in KPL-716 recipients compared to -30.9% in placebo recipients.

- 52.6% of KPL-716 recipients demonstrated a ≥ 4-point reduction in weekly-average WI-NRS at Week 12 compared to 26.3% of placebo recipients.

There was no meaningful benefit of repeated-single-doses of KPL-716 on other efficacy endpoints specific to atopic dermatitis, including EASI and Scoring Atopic Dermatitis, or SCORAD. There were no serious adverse events. However, there were more atopic dermatitis flares in the KPL-716-treated population versus placebo (47.6% versus 4.5%) through the 12-week treatment period; all subjects who experienced a flare were successfully managed with topical corticosteroids. KPL-716 was otherwise well-tolerated by all subjects. We believe these single-dose and repeated-single doses study 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.

OSMRβ Axis Identified in Prurigo Nodularis

In 2019 at the Annual Meeting of the Society for Investigative Dermatology, we presented preclinical data identifying the OSMRβ axis in prurigo nodularis subjects. Data from Kiniksa's longitudinal observational study in prurigo nodularis, or LOTUS-PN suggest that the OSMRβ axis (IL-31, OSM, IL-31 receptor alpha (IL-31Rα) and OSMRβ) may play a role in the pathogenesis of prurigo nodularis given its prevalent expression in lesional prurigo nodularis. IL-31 messenger ribonucleic acid, or mRNA, was expressed in approximately two-thirds of lesional biopsies from prurigo nodularis patients with WI-NRS ≥ 7 compared to one-tenth in healthy volunteers. Additionally, lesional biopsies from prurigo nodularis patients contained mononuclear cells expressing OSM, OSMRβ, IL-31 and IL-31Rα protein compared with non-lesional biopsies.

Phase 2a Clinical Trial in Prurigo Nodularis

Based on the results from the single-dose IV Phase 1b results in atopic dermatitis subjects, we initiated a Phase 2a randomized, double blind, placebo-controlled clinical trial to evaluate 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. The Phase 2a study includes two arms: one placebo arm and one active arm administered as 360mg weekly subcutaneous doses after a 720mg loading dose; designed to achieve maximum or near maximum steady-state exposures. The primary and key secondary endpoints, which focus on pruritus, are to be evaluated at 8 weeks. Other secondary endpoints 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 8). We expect to report data from this trial by the end of April 2020.

OSMRβ Axis Identified in Other Chronic Pruritic Diseases

At the Annual Meeting of the European Society of Dermatological Research in 2019, we presented preclinical data identifying the OSMRβ axis as relevant in chronic pruritic diseases. Increased mRNA transcript levels and protein levels of OSMRβ, in formalin-fixed paraffin-embedded biopsies from patients with chronic idiopathic urticaria, chronic idiopathic pruritus, lichen planus and lichen simplex chronicus, relative to healthy control skin samples, suggest the IL-31/OSM signaling axis is associated with these pruritic diseases.

Elevated levels of OSMRβ mRNA and protein observed in regions of inflammatory infiltrate of all chronic pruritic diseases tested, relative to healthy controls, suggest that the OSMRβ axis may be active in, and contributing to, these skin disorders. Particularly, OSM and IL-31 mRNA and protein were present in each disease evaluated for these cytokines.

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 initiated 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, with study populations in chronic idiopathic urticaria, chronic idiopathic pruritus, plaque psoriasis, lichen planus, and lichen simplex chronicus.

The trial is comprised of multiple cohorts, each for a different study population. Each cohort being 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 remain blinded throughout the study. Each cohort could enroll up to a maximum of 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 is 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. We expect to report interim data from cohorts of this trial in the first half of 2020

KPL-404

Overview

KPL-404 is a humanized monoclonal antibody that is designed to inhibit the CD40-CD40 ligand interaction (CD40L), 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 connection with our acquisition of Primatope, we acquired an exclusive world-wide license with Beth Israel Deaconess Medical Center for certain patent applications and patents related to KPL-404.

Mechanism of Action and External Clinical Proof of Concept

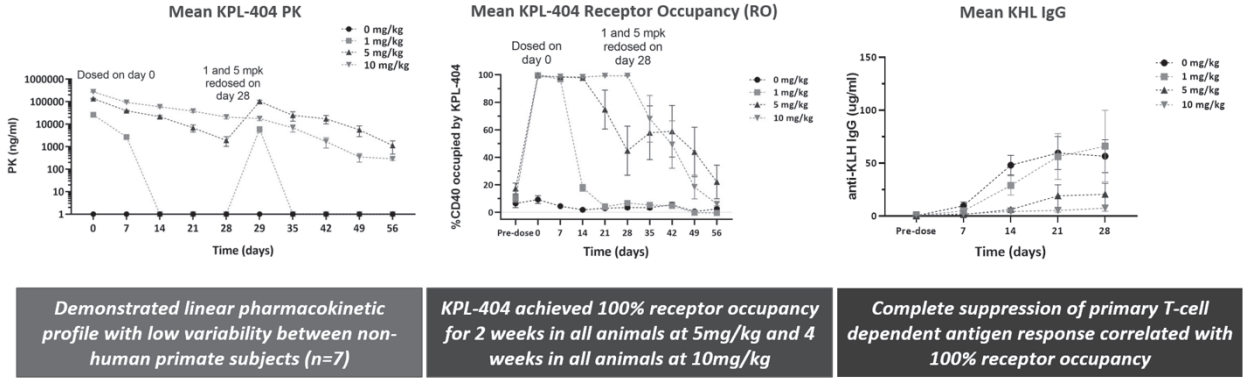
KPL-404 is designed to block the CD40/CD40L interaction by binding to and inhibiting signaling through CD40 receptor. CD40 is a member of the Tumor Necrosis Factor Receptor superfamily which is constitutively or inducibly expressed on the surface of a variety of immune and non-immune cell types including B cells, macrophages, dendritic cells, microglia, endothelial cells, epithelial cells, and keratinocytes and can also be upregulated on other cell types in the context of autoimmune disease. Interactions between B cell-expressed CD40 and its binding partner, CD40L, mainly expressed on activated CD4+ T cells, play a critical role in promoting germinal center formation and the production of class-switched antibodies. The role of CD40 in B cells has been extensively characterized and has been shown to be essential for productive primary and secondary humoral immune responses to T dependent antigens. External clinical data point to the broad potential power of the mechanism has been established in rheumatoid arthritis, systemic lupus erythematosus, primary Sjogren's syndrome, Graves' disease and prevention of kidney transplant rejection. Ongoing Phase 2 trials from competitors implicate additional indications for potential development, including type 1 diabetes, inflammatory bowel disease, prevention of liver transplant rejection, hidradenitis suppurativa, lupus nephritis and multiple sclerosis.

Our Solution

KPL-404 inhibits the signaling of CD40 and CD40L with low-single digit nanomolar affinity *in vitro*. The presentation of KPL-404 is as a high-concentration, liquid formulation potentially suitable for subcutaneous administration at doses of up to 5mg/kg, which we believe may, allow for a higher delivered dose in one SC injection than all other competitors who are mainly limited to high dose IV formulations or SC formulations. We believe high-dose IV and SC formulations do not fully antagonize signaling as evidenced by the generation of anti-drug antibodies ever at maximum delivered dose levels.

Preclinical Development

In preclinical development, KPL-404 has been observed to have a favorable pharmacokinetic and toxicology profile and has shown activity in multiple non-human primate models of organ transplant rejection, as well as in multiple TDAR models. The data in the graphs below show in a non-human primate TDAR model that KPL-404 had linear pharmacokinetics with low variability which translated into complete suppression of antibody responses to a novel antigen (keyhole limpet hemocyanin, or KLH) at drug levels achieving 100% receptor occupancy.



Phase 1 Program

In 2019, we began enrollment and dosing of subjects in a single-ascending-dose Phase 1 clinical trial of KPL-404 in healthy volunteers. The first-in-human trial is designed to provide safety and pharmacokinetics data as well as receptor occupancy and TDAR data from a KLH challenge for single rising intravenous and subcutaneous dose levels of KPL-404. We expect to report top-line data from this trial in the second half of 2020.

Discovery Activities

We conduct internal discovery activities directed toward wholly owned molecules for the treatment of debilitating disease targets where we believe there to be a strong mechanistic rationale and potential for 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 United 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 biologics license application, or 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 new patent applications related to mavrilimumab, the granting of such 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 made milestone payments of $10.3 million during the year ended December 31, 2019, primarily 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 $315.0 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 $165.0 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 new patent applications related to Antibody Products, the granting of such 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 late stage manufacturing facilities. Although we built early stage manufacturing facilities to produce drug substance to support certain of our preclinical and early clinical studies, we rely on third parties to manufacture all of our drug 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 earlier 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. 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 drug product currently in use in our on-going clinical trials. 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 clinical drug substance for certain preclinical studies, but we intend to produce our preclinical product candidates for Phase 1 and Phase 2 studies in our own early stage manufacturing facilities. We plan to continue to use CMOs to produce the corresponding drug product clinical material. 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 perform process development internally but are reliant on CMOs for late stage clinical manufacturing, process qualification and validation and commercial supply. 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, and any other product candidates that we may develop, 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, preclinical 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 our product candidates, including 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, 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, 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, Janssen, 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. is recruiting a Phase 3 clinical trial with their anti-IL-6 program and Novartis International AG, is recruiting a trial with their IL-17 antagonist secukinumab (Cosentyx).

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., Regeneron, and Galderma SA, or Galderma, have programs in various stages of clinical development for the treatment of prurigo nodularis.

KPL-404

Novartis AG, Abbvie and Boehringer Ingelheim International GmbH, Biogen Inc. / UCB, Viela Bio, Astellas, Sanofi S.A. /ImmuNext Inc. and Anelixis Therapeutics all have active development programs with either CD40 or CD40L antagonists.

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, methods-of-use and methods of manufacture, 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.

Rilonacept

We have a field-specific exclusive license under the Regeneron Agreement to granted patents and pending applications in the United States and numerous other jurisdictions relating to rilonacept. As of December 31, 2019, the patent rights in-licensed under the Regeneron Agreement relating to our program include one granted patent in the United States and 47 patents granted in other 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 counterparts outside of the United States 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.

Mavrilimumab

We have an exclusive license under the MedImmune Agreement to granted patents and pending patent applications in the United States and numerous other jurisdictions relating to mavrilimumab. These patents and patent applications cover mavrilimumab as a composition of matter and its use. As of December 31, 2019, 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 other 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."

KPL-716

We own, via our acquisition of certain assets from Biogen, granted patents and pending patent applications in the United States and numerous other 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, 2019, the patent rights acquired from Biogen include four patents granted in the United States and 32 patents granted in other 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 comparable regulatory authorities in other jurisdictions, provided the legal requirements are met, 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.

KPL-404

We own, via our acquisition of Primatope, granted patents and pending patent applications in the United States and numerous other jurisdictions relating to KPL-404. We also have an exclusive license with Beth Israel Deaconess Medical Center to granted patents and pending patent applications in the United States and numerous other jurisdictions relating to KPL-404. These patents and patent applications cover KPL-404 as a composition of matter and its use. As of December 31, 2019, the patent rights acquired from Primatope include two patents granted in the United States. In addition, the patent rights acquired from Primatope include patent applications pending in the United States, Europe, Canada, and selected countries in Asia. The issued composition of matter patents have statutory expiration dates in 2036. As of December 31, 2019, the patent rights licensed from Beth Israel Deaconess Medical Center include two patents granted in the United States and 30 patents granted in other jurisdictions, including Australia and selected countries in Europe and Asia. In addition, the patent rights licensed from Beth Israel Deaconess Medical Center include patent applications pending in the United States, Europe, Canada, and selected countries in Asia. The issued composition of matter patents licensed from Beth Israel Deaconess Medical Center have statutory expiration dates in 2032, not including any patent term extensions or adjustments. 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.

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 preclinical 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 preclinical 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 preclinical 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.

Preclinical Studies and CMC Evaluations

Before testing any biological product candidate, including our product candidates, in humans, the product candidate must undergo rigorous preclinical testing. The preclinical 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 preclinical tests must comply with federal regulations and requirements, including GLP regulations. The sponsor must submit the results of the preclinical studies, together with chemistry manufacturing and controls, or CMC, 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 preclinical 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 CMC issues, preclinical issues, or one or more issues in the 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 necessarily result in the FDA allowing clinical trials to commence. The FDA also may place the IND on partial clinical hold, and a proposed study may only be partially executable, including due to FDA restrictions.

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 inclusion/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 in 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 disclose the results of certain qualifying 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 product candidates.

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 optimal 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 labeling and 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 a clinical hold on 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 preclinical 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 preclinical 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 preclinical 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 60-day 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 challenges in interpretation of the safety or efficacy data 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 Risk Evaluation and Mitigation Strategy, or 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 iPSP, 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 iPSP 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 iPSP. A sponsor can submit amendments to an agreed upon initial iPSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical 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, preclinical 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 label. 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, or new safety findings after market introduction. 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. 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. 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. 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.

Any product submitted to the FDA for approval, including a product with a Fast Track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it is designed to treat a serious condition, and if approved, would provide a significant improvement in safety or effectiveness compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a biologic designated for priority review in an effort to facilitate the review. The FDA endeavors to review original BLAs with priority review designations within six months of the filing date as compared to ten months under its standard review goals.

In addition, a product may be eligible for accelerated approval. Drug and biologics intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination 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 irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials to verify the predicted clinical benefit. A product that receives accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails conduct the required post-marketing trials or if such trials fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

In addition, 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. The designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance. The Breakthrough Therapy designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met. If a product is designated as Breakthrough Therapy, the FDA will expedite the development and review of such drug. All requests for Breakthrough Therapy designation will be reviewed within 60 days of receipt, and the FDA will either grant or deny the request.

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 preclinical 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 continues 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 remain 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 preclinical 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 2020. 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 (mandatory for biologics) or national authorization procedures (if intended to submit only in a single country).

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 preclinical 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, Asia, or Japan, 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 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. 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. 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. 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 (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), and additional categories of health care in 2022, and teaching hospitals 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 we believe that we would not 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. Even when HIPAA does not apply, according to the Federal Trade Commission or the FTC, failing to take appropriate steps to keep consumers' personal information secure constitutes unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act, or the FTCA, 15 U.S.C § 45(a). The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Individually identifiable health information is considered sensitive data that merits stronger safeguards. The FTC's guidance for appropriately securing consumers' personal information is similar to what is required by the HIPAA security regulations.

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. For example, California enacted the California Consumer Privacy Act, or the CCPA, on June 28, 2018, which went into effect on January 1, 2020. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation.

Similar laws and regulations in jurisdictions outside of the United States, which may include, for instance, applicable post-marketing requirements, anti-fraud and abuse laws and implementation of corporate compliance programs, reporting of payments or other transfers of value to healthcare professionals or data privacy and security laws,

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 governments outside the United States 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 a substantial 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 substantial portion of the cost of our products. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. 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 and the related coverage criteria for a biological product typically is separate from, but related to, the process for setting the price of such product or for establishing the level of reimbursement 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, which results in higher cost-sharing financial obligation imposed on patients. A decision by a third-party payor not to cover our product candidates, or to impose coverage criteria the limiting situations in which our product candidates are covered, 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, coverage criteria, 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, clinical and health economic support for the use of their products to each payor separately, which 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. The

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 are 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-benefit of pharmaceutical products, in addition to questioning safety and efficacy. If third-party payors do not consider a product to offer a favorable cost-benefit 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 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 level 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. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the district court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. It is unclear how these decisions, subsequent appeals, if any, or other efforts to challenge, repeal or replace the Affordable Care Act will impact the law or 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 2029 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 governments and third party payors will pay for healthcare products and services.

Employees

As of December 31, 2019, we had 137 employees, 135 of which were full-time 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 audited 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.

Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. Typically, it takes many years to develop one new product from the time it is discovered to when it is available for treating patients, and development may cease for a number of reasons. We have incurred operating 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 a Phase 3 or other pivotal clinical trial, 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. 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, 2019 and 2018 were $161.9 million and $103.2 million, respectively. As of December 31, 2019, we had an accumulated deficit of $356.1 million. We expect to continue to incur losses for the foreseeable future, and we anticipate these losses will increase substantially as a result of many factors, including:

- our research and preclinical and clinical development of our product candidates, including our global, pivotal Phase 3 clinical trial for rilonacept for the treatment of recurrent pericarditis, named RHAPSODY, our global Phase 2 clinical trial with mavrilimumab for the treatment of GCA our Phase 2a clinical trial with KPL-716 in prurigo nodularis and our exploratory Phase 2 clinical trial for KPL-716 in diseases characterized by chronic pruritus, as well as our Phase 1 clinical trial in healthy volunteers for KPL-404;
- initiating potential additional preclinical studies and clinical trials for our product candidates;
- increasing our manufacturing capabilities or adding additional manufacturers or suppliers;
- seeking regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;

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

- seeking 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;

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

- entering into licensing, acquisition, collaboration or other strategic transaction agreements;

- making milestone or other payments under any current or future license, acquisition, collaboration or other strategic transaction agreements;

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

- seeking to attract and retain skilled personnel;

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

- experiencing delays or encountering issues with any of the above, including but not limited to failed trials, complex results, safety issues, 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. 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, will force us delay, limit, reduce or cease one or more of our product development plans, research and development programs for our product candidates, or other operations or commercialization efforts.

The development and commercialization of biopharmaceutical products is capital intensive. We are advancing our product candidates through research, preclinical and clinical development, including our global, pivotal Phase 3 clinical trial for rilonacept for the treatment of recurrent pericarditis, named RHAPSODY, our global Phase 2 clinical trial for mavrilimumab for the treatment of giant cell arteritis, or GCA, our Phase 2a clinical trial for KPL-716 for the treatment of prurigo nodularis, our exploratory Phase 2 clinical trial with KPL-716 in diseases characterized by chronic pruritus, and our Phase 1 clinical trial with KPL-404 in healthy volunteers. We expect our expenses to increase in connection with our ongoing activities as we continue the research and development of our product candidates, establish and expand our sales, marketing and distribution capabilities, infrastructure and organization, or enter into agreements with third parties to conduct one or more of these commercialization activities. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant additional 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 make royalty payments to the licensors and other third parties from whom we have acquired our product candidates. 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 acceptable terms, if at all, we will be forced to delay, limit, reduce or cease one or more of our product development plans, research and development programs for our product candidates, or commercialization efforts. We

also may not be able to expand our operations or otherwise capitalize on our business opportunities, or may be required to relinquish rights to our product candidates or products.

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 securities offerings, 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 from, and the time and cost necessary for, completing our global, pivotal Phase 3 clinical trial for rilonacept in recurrent pericarditis, RHAPSODY, our global Phase 2 clinical trial for mavrilimumab in GCA, our Phase 2a clinical trial for KPL-716 in prurigo nodularis, our exploratory Phase 2 clinical trial for KPL-716 in diseases characterized by chronic pruritus, and our Phase 1 clinical trial in healthy volunteers for KPL-404;

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

- the costs, timing and outcomes of seeking and potentially obtaining approvals from the U.S. Food and Drug Administration, or FDA, or comparable regulatory authorities outside of the United States, including the potential for the FDA or such comparable regulatory authorities to require that we conduct more studies than we currently plan 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 product candidates 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 Pharmaceuticals, Inc., or Regeneron, MedImmune, Limited, or MedImmune, Biogen MA Inc., or Biogen, 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 establishing and expanding our sales, marketing and distribution capabilities, infrastructure and organization or entering into agreements with third parties to conduct one or more of these activities for any of our product candidates, if approved or in anticipation of such approval;

- 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' and physicians' receptivity to our product candidates and the technology underlying them in light of competitive products and technologies;

- the cash requirements of any future in-license, acquisition, development or discovery of additional product candidates, including in connection with 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 related activities;
- the costs associated with being a public company;
- our need and ability to hire and retain skilled personnel; and
- the receptivity of the capital markets to financings by biopharmaceutical 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 securities offerings 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 when needed, we will be forced to curtail, delay, limit, reduce or cease one or more of our product development plans, research and development programs for our product candidates, or commercialization efforts 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 private or public securities offerings, debt financings, or other sources, including licensing, collaboration or other strategic transactions or arrangements with third parties. 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. Obtaining funds through licensing, collaboration or other strategic transactions or arrangements with third parties may require us 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

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 are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable regulatory authorities outside of the United States. We have four product candidates in various stages of clinical development. Our assumptions about why our product candidates are worthy of future development and potential approval in the indications for which we are studying them, or any other indications, are based on either indirect data primarily collected by other companies or our preclinical and clinical trials. 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 cryopyrin-associated periodic syndrome, or 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 a global, pivotal Phase 3 clinical trial, RHAPSODY. Mavrilimumab has been through Phase 2 clinical trials conducted by MedImmune for the treatment of rheumatoid arthritis, or RA, but our global Phase 2 clinical trial with mavrilimumab is for the treatment of GCA. KPL-716, is being studied in a Phase 2a clinical trial in prurigo nodularis and an exploratory Phase 2 clinical trial in diseases characterized by chronic pruritus. In addition, KPL-404 has progressed into a Phase 1 clinical trial in healthy volunteers. Our future preclinical product candidates would need to progress through toxicology studies and other requirements to enable an Investigational New Drug application, or IND, prior to clinical development.

We have not submitted, and we may never submit marketing applications to the FDA or comparable regulatory authorities outside of the United States 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 we complete a successful clinical trial. We may determine that the potential product and commercial profile of any of our product candidates would not ultimately be commercially successful and could therefore elect not to continue its development. 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 preclinical or clinical development, regulatory approval in one or more jurisdictions, manufacturing capacity and expertise, successful manufacture of clinical supply, building an organization to support commercialization, substantial investment and significant marketing efforts before we will be able to generate any revenue from product sales. The success of our product candidates or potential future product candidates depends upon several factors, including the following:

- successful completion of preclinical 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 and acceptance by the FDA of INDs and of clinical trial applications to governmental authorities outside of the United States for our product candidates to commence planned clinical trials or future clinical trials;

- successful site activation for, 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 and maintenance of any such approvals;

- pediatric study plans acceptable to the FDA and comparable regulatory authorities outside of the United States, and follow through of any pediatric study commitments, including development of pediatric formulations where indicated;

- establishment and maintenance 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;

- successful manufacture of sufficient supplies of our product candidates within approved specifications for purity and efficacy from our facility and from our CMOs in order to meet clinical or commercial demand, as applicable;

- 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, patient-advocates, 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 trial commitments 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 in, 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 smaller than we estimate, we may not generate projected revenue levels from sales of such products, if approved.

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 our product candidates in humans. Clinical testing is expensive, time consuming and uncertain as to outcome. We are conducting a global, pivotal Phase 3 clinical

trial with rilonacept for the treatment of recurrent pericarditis, RHAPSODY, a global Phase 2 clinical trial with mavrilimumab for the treatment of GCA, a Phase 2a clinical trial with KPL-716 in prurigo nodularis, an exploratory Phase 2 clinical trial with KPL-716 in diseases characterized by chronic pruritus, and a Phase 1 clinical trial with KPL-404 in healthy volunteers. We cannot guarantee that any of our current or potential future clinical trials will be conducted as planned or completed on schedule, if at all.

Commencing a clinical trial is subject to acceptance by the FDA of an IND or IND amendments, acceptance by European regulatory authorities of a Clinical Trial Application, or CTA, 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. We may receive feedback or guidance from regulatory authorities on our clinical trial design and protocols and even after we incorporate such feedback or guidance from these regulatory authorities, such regulatory authorities may impose other requirements for our clinical trials, could disagree that we have satisfied their requirements to commence our clinical trials, disagree with our interpretation of data from the relevant preclinical 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 level or schedule, treatment duration, our definitions of the patient populations or the clinical endpoints selected, which may require us to complete additional preclinical studies, clinical trials, CMC development, other studies or impose stricter approval conditions than we currently expect. For example, the FDA has provided feedback 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. In addition, we anticipate that other potential indications for mavrilimumab would need to be in serious or life-threatening diseases where the burden of the disease is sufficient to justify the risk-benefit of mavrilimumab to pursue clinical development in such indications. Further, based on FDA feedback we received in connection with its review and approval of an IND for our global Phase 2 clinical trial of mavrilimumab in GCA, we anticipate that to help inform the risk-benefit profile for the use of mavrilimumab in GCA, we will need to demonstrate the safety and effectiveness of mavrilimumab at the 26 weeks of dosing stipulated in our Phase 2 clinical trial, and eventually demonstrate safety and effectiveness of mavrilimumab beyond 26 weeks as well as evaluate its pharmacokinetic profile and the effectiveness of mavrilimumab at different doses in GCA.

Commencing our planned clinical trials is also subject to approval by an IRB at each clinical trial site before a trial may be initiated, which approval could be delayed, rejected or suspended. Further 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 may impose a suspension or termination of our clinical trials even after approval and initiation of trial sites 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, unforeseen safety issues or adverse side effects that arise in the trial, failure to demonstrate a benefit from using a drug, any of which resulting in the imposition of a clinical hold as well as changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Successful completion of our clinical trials is a prerequisite to submitting a biologics license application, or BLA, or New Drug Applications to the FDA and a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, or other applicable regulatory authorities in other countries 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 future clinical trials will begin as planned or any of our current or future clinical trials will be completed on schedule, if at all.

A failure of one or more of our current or future clinical trials can occur at any stage of testing, and our 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, 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

commencement or successful completion of clinical development of our product candidates as planned, if at all, include but are not limited to:

- inability to generate sufficient preclinical, toxicology or other *in vivo* or *in vitro* data to support the initiation of human clinical trials;
- delays or failure in reaching a consensus with regulatory agencies on trial design or implementation;
- delays or failure in establishing the appropriate dosage levels or frequency of dosing or treatment period in clinical trials;
- delays or failure in reaching 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 or failure in obtaining regulatory approval to commence a trial, or imposition of a clinical hold by regulatory authorities, 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 or a sufficient number thereof to participate in our clinical trials;
- amendments to clinical trial protocols impacting study criteria, endpoints or design, including amendments that either we initiate or are requested by regulatory authorities;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, medical institutions, other third parties we contract with in connection with our clinical trials, 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 comparable regulatory guidelines in other countries;
- patients not completing participation in a clinical trial or returning for post-treatment follow-up, in either case including as a result of trial demands on participants among other things;
- clinical trial sites or patients withdrawing from a clinical trial;
- participating patients experiencing serious adverse events or undesirable side effects or being exposed to unacceptable health risks;
- participating patients failing to experience confirmed pre-specified events during the clinical trial within an expected time-frame, if at all;
- safety issues, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- difficulty in identifying the patient 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, inconclusive or uncompetitive results, which may result in us deciding, or regulators requiring us, to conduct additional clinical trials or modify or cease development programs for our product candidates;

- suspensions or terminations of our clinical trials by us or the IRBs of the institutions in which our clinical trials are being conducted, the Data Safety Monitoring Board for such trials or the FDA or comparable regulatory authorities;

- failure of manufacturers, or us, to produce supplies of our product candidates for use in our clinical trials in accordance with current good manufacturing practices, or cGMP, requirements and regulations or applicable comparable regulatory guidelines in other countries; 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.

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 delay or denial of regulatory approval of our product candidates.

If we experience delays in the commencement or 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. 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 or us deciding to modify or cease development of our product candidates.

Furthermore, clinical trials must be conducted in accordance with the laws, rules and regulations, guidelines and other requirements of the FDA, European Union, or EU, and other applicable regulatory authorities outside of those jurisdictions and are subject to oversight by these regulatory authorities and IRBs at the medical institutions where the clinical trials are conducted. Further, conducting global clinical trials, as we do for certain of our product candidates, may require that we coordinate among the legal requirements and guidelines of regulatory authorities across a number of jurisdictions, including the United States, EU and countries outside of those jurisdictions, which could require that we amend clinical trial protocols or determine not to conduct a trial in one or more jurisdiction 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 that are conducted in countries outside the United States and the EU may subject us to risks associated with the engagement of non-United States and non-EU CROs, clinical investigators who are unknown to the FDA or the EMA, and may have different standards of diagnosis, screening and medical care, as well as risks associated with further delays and expenses as a result of increased shipment costs (including as a result of local quality release or in-country testing of a product candidate supply produced in a different jurisdiction for our clinical trials) and political and economic risks relevant to such countries outside the United States and the EU.

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 preclinical 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 or competing clinical studies in the same patient population. Difficulty in enrolling patients could delay or prevent completion of our 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 a sufficient number of patients to participate in testing our product candidates, particularly given that many of the conditions for which we are evaluating our current product candidates or may plan to evaluate them in the future are in small disease populations. In addition, 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 evaluate based on the primary and secondary endpoints of the study.

Further, 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 would reduce the number of patients who are available for our clinical trials at such clinical trial site.

Accordingly if we encounter these or other difficulties in enrollment we may experience delays or be prevented from completing our clinical trials. Patient enrollment depends on many factors, including:

- the size and nature of the patient population;
- the severity of the disease being studied;
- patient referral practices of physicians;
- patient eligibility criteria for the clinical trial;
- the proximity of patients to clinical sites;
- the complexity of the design and nature of the clinical protocol and trial;
- the availability and nature of competing clinical trials;
- the availability of standard of care or new drugs approved for the indication the clinical trial is investigating;
- failure to obtain and maintain patient consents;
- our ability to recruit clinical trial investigators with applicable competencies and experience;
- the risk that patients enrolled in clinical trials will withdraw from the trials before completion of their treatment or follow-up period (in either case including as a result of trial demands on participants among other things);

- clinicians' and patients' perceptions as to the safety and potential advantages of the product candidate being studied in relation to other available therapies; and

- the occurrence of adverse events, or AEs, or undesirable side effects attributable to our product candidates.

The process of finding and enrolling patients may prove costly, especially since we are looking to identify a subset of the patients eligible for our studies from a relatively small patient population for many of the diseases we are studying. If patients are unable or unwilling to participate in our clinical trials for any reason, or we experience difficulties in patient enrollment for any other reasons, our costs and the timeline for recruiting patients, conducting trials and obtaining regulatory approval of our product candidates may be delayed or prevented, the commercial prospects of our product candidates may be harmed, and our ability to commence product sales and generate product revenue from any of these product candidates could be delayed or prevented. Any of these occurrences may harm our business, financial condition and prospects significantly.

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 more restrictive labels or the delay or denial of regulatory approvals by the FDA or other comparable regulatory authorities outside of the United States.

Our product candidates modulate the immune system and carry risks associated with immunosuppression, including the risk of serious infections and other potential serious health risks. For example, 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 or to delay symptomatology and diagnosis of infections. Serious, life-threatening infections have been reported in patients taking rilonacept. In our open-label 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 pulmonary alveolar proteinosis, or PAP. PAP is a rare lung disorder in which surfactant-derived lipoproteins accumulate excessively within pulmonary alveoli due to loss of granulocyte macrophage colony stimulating factor, or GM-CSF, function. The disease can range in severity from a sub-clinical reduction in diffusion capacity to significant dyspnea during mild exertion. In preclinical 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. Preclinical 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 an unacceptable severity and prevalence of these or other side effects, the FDA or applicable regulatory authority outside of the United States may suspend or terminate our clinical trials, or not authorize us to initiate further trials. In addition, if other anti-GM-CSF molecules in development by third parties show these or similar side effects, it could have an impact on the entire class of anti-GM-CSF molecules in development and the applicable regulatory agency may suspend or terminate our clinical trials, or not authorize us to initiate further trials. The FDA or comparable regulatory authorities outside of the United States could order us to cease further development of, or deny or withdraw any approval of, any of our product candidates for any or all targeted indications.

In addition, subsequent to MedImmune's original IND submission for RA and the availability of additional clinical safety data that MedImmune generated in human clinical trials conducted outside of the United States for RA, the FDA provided feedback 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. Further, we anticipate that other potential indications for mavrilimumab would need to be in serious or life-threatening diseases where the burden of the disease is sufficient to justify the risk-benefit of mavrilimumab to be studied in such indications. In addition, based on FDA feedback we received in connection with its review and authorization of an IND for our global Phase 2 clinical trial of mavrilimumab in GCA, we anticipate that to help inform the risk-benefit profile for the use of mavrilimumab in GCA, we will need to demonstrate the safety and effectiveness of mavrilimumab at the 26 weeks of dosing stipulated in our Phase 2 clinical trial, and eventually demonstrate safety and effectiveness of mavrilimumab beyond 26 weeks as well as evaluate its pharmacokinetic profile and the effectiveness of mavrilimumab at different doses in GCA.

In our repeated-single-dose Phase 1b clinical trial of KPL-716, there were no serious AEs. However, there were more atopic dermatitis flares in the KPL-716-treated population versus placebo (47.6% versus 4.5%) through the 12-week treatment period; all subjects who experienced a flare were successfully managed with topical corticosteroids. KPL-716 was otherwise well-tolerated by all subjects.

Additionally, clinical trials by their nature utilize a sample of the potential patient population. With a limited number of patients, certain rare and severe side effects associated with our product candidates may only be uncovered with a significantly larger number of patients exposed to the product candidates. If one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by the 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, and KPL-716, we were not involved in the development of these product candidates and, as a result, we are dependent on Regeneron, MedImmune, and Biogen having accurately reported the results and correctly collected and interpreted the data from all preclinical and clinical trials conducted prior to our in-license or acquisition.

We had no involvement with or control over the preclinical and clinical development of any of our product candidates prior to our in-license or acquisition of them. We are dependent on Regeneron, MedImmune, and Biogen 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 preclinical 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 preclinical 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 preclinical 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 be predictive of the results from any required later preclinical studies and clinical trials. Similarly, the positive results from the preclinical studies and clinical trials of our product candidates may not be replicated in our subsequent preclinical studies or clinical trial results. The mechanisms of action of our product candidates may not prove to be safe or effective to treat the diseases that we are studying. Further, 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 later-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, preclinical findings made while clinical trials were underway or safety or efficacy observations made in preclinical studies and clinical trials, including AEs previously unreported in earlier studies and trials of our product candidates and favorable safety and efficacy observed in earlier studies and trials not replicated in later studies or trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain FDA or EMA approval. Furthermore, the approval policies or regulations of the FDA or the applicable regulatory authorities outside of the United States may significantly change in a manner rendering our clinical data insufficient for approval, which may lead to the FDA or such other regulatory authorities delaying, limiting or denying approval of our product candidates.

Preliminary, interim and "top-line" 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 publicly disclose interim, top-line or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the top-line or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Top-line or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the top-line or preliminary data we previously published. As a result, top-line and preliminary data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our preclinical studies and clinical trials. For example, we expect to release interim data from cohorts of the Phase 2 clinical trial of KPL-716 in diseases characterized by chronic pruritus in the first half of 2020. 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. Adverse differences between interim data and final data could significantly harm

our business prospects. Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our Class A common shares.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure.

If the interim, top-line or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Risks Related to Marketing Approval and Regulatory Matters

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 or we fail or otherwise cease to advance their development, we will be delayed in commercializing or will not be able to commercialize, 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 trial design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, pricing, 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 preclinical 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. In addition to the United States, we may seek regulatory approval to commercialize our product candidates in other jurisdictions. 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.

The process of obtaining regulatory approvals, both in the United States and in other countries, is time consuming, expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. 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 preclinical, clinical or other trials.

In addition, we plan to have Regeneron transfer its BLA for rilonacept in the United States for the treatment of CAPS following positive clinical data readout, if any, from our global, pivotal Phase 3 clinical trial, RHAPSODY to enable us to file an sBLA with the FDA to seek approval for the commercial marketing of rilonacept in the United States for the treatment of recurrent pericarditis. The rights with respect to CAPS would remain with Regeneron until the date that we receive such approval from the FDA, if at all. In order for the BLA to be transferred to us, we must coordinate numerous activities with Regeneron in advance and take over certain responsibilities and obligations with respect to owning the BLA. The process to transfer a BLA is complex and resource intensive, and may be more so than we anticipate. Further, if Regeneron or we do not perform in accordance with our expectations, or we are not ready to assume the responsibilities on a timely basis, or the timing of the BLA transfer is otherwise delayed, our ability to file an sBLA with the FDA may be delayed, which could delay FDA approval or our commercialization of rilonacept, and our ability to generate revenue from rilonacept could be materially impaired.

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 or we may fail or cease to advance their development for many reasons, including the following:

- the FDA or comparable regulatory authorities in other jurisdictions 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 regulatory authorities in other jurisdictions that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may produce negative, inconclusive or uncompetitive results, which may result in us deciding, or regulators requiring us, to conduct additional clinical trials or to modify or cease development programs for our product candidates;
- the results of clinical trials may not meet the primary or secondary endpoints of the applicable study or the level of statistical significance required by the FDA or comparable regulatory authorities in other jurisdictions;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable regulatory authorities in other jurisdictions may disagree with our interpretation of data from preclinical studies or clinical trials;
- the FDA or comparable regulatory authorities in other jurisdictions 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 regulatory authorities in other jurisdictions 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 risk-benefit profile for the use of mavrilimumab in GCA, we will need to demonstrate the effectiveness and safety of mavrilimumab at the 26 weeks of dosing stipulated in our global Phase 2 clinical trial, and eventually demonstrate effectiveness and safety of mavrilimumab beyond 26 weeks as well as evaluate its pharmacokinetic profile and the effectiveness of mavrilimumab at different doses;

- the FDA or comparable regulatory authorities in other jurisdictions could require us to conduct additional clinical studies to compare our product candidates to other therapies for the treatment of the same indication;

- 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 regulatory authorities in other jurisdictions 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, toxicities 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 regulatory authorities in other jurisdictions may significantly change in a manner rendering our clinical data, biologic manufacturing process, and other supporting information 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 or to advance our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenue will be materially impaired.

Our current product candidates and any of our future product candidates regulated as biologics in the United States may face biosimilar 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 approved under a BLA 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 for the same therapeutic indication if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and

implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when 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.

For example, 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, if our clinical trials are successful, 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, potentially creating the opportunity for biosimilar competition.

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 EU, we may not seek or 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. Regulatory requirements can vary widely from country to country, and 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 and may require additional preclinical 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 may seek orphan drug designation for certain of our product candidates, and ultimately 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 EU, 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 EU, 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 EU 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 EU would be sufficient to justify the necessary investment in developing the drug. In the EU, 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 EU. The EU 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 may pursue orphan drug designation for certain of our product candidates, 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. Such designation may not lead to a faster development or regulatory review or approval process and does not increase the likelihood that our product candidates will receive marketing approval.

In the third quarter of 2019, we received Breakthrough Therapy designation for rilonacept for the treatment of for recurrent pericarditis and we may seek Breakthrough Therapy or Fast Track designation for some of our other 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. In addition, if a product candidate is intended for the treatment of a serious or life-threatening condition and clinical or preclinical 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 Fast Track and Breakthrough Therapy designations, and even if we believe a particular product candidate is eligible for this designation, we cannot be certain that the FDA would decide to grant it. Even if we obtain such designations 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 or Breakthrough Therapy designations if it believes that such designations are no longer supported. Although products receiving Fast Track and Breakthrough Therapy designation are generally eligible for the FDA's priority review procedures, receiving such designations does not guarantee that the BLA for such products will receive priority review.

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 meets 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 pivotal 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, our global, pivotal Phase 3 clinical trial in rilonacept or any of our eventual other 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 or other government agencies may refuse to accept for substantive review any regulatory 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 or other government agencies do not accept our applications or issue marketing authorizations for our product candidates, they may require that we conduct additional clinical, preclinical or manufacturing validation trials and submit that data before they will reconsider our applications. Depending on the extent of these or any other required trials, approval or receipt of any marketing authorization 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 or other government agencies to approve or grant marketing authorizations. Any delay in obtaining, or an inability to obtain, marketing approvals would delay or 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 modify or cease 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 or other government agencies 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 government 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 Reliance on Third Parties

We contract with third parties for manufacturing our product candidates and for certain research and other preclinical 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 late-stage manufacturing facilities. Although we have built a development and manufacturing facility to produce drug substance to support certain research and other preclinical and early clinical development for our product candidates, we rely, and expect to continue to rely, on third parties for the manufacture of our late-stage product candidates and certain of our early-stage product candidates for the majority of our clinical development efforts, as well as for the potential commercial manufacture of our product candidates, if approved, as well as label and packaging activities. 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 have a contract with Regeneron to produce rilonacept on an exclusive basis for a period of time. Although Regeneron has produced rilonacept for commercial use for over ten years, the FDA or other applicable regulatory authorities in other jurisdictions might reevaluate rilonacept's current manufacturing process or route of administration in connection with evaluating whether to approve rilonacept for a new indication, such as recurrent pericarditis. We also have CMOs manufacture KPL-716 drug substance and drug product and entered into an agreement with a CMO to produce mavrilimumab beyond our current inventory. While we have built a manufacturing facility to support early development for our product candidates, we and our CMOs may not be able to produce sufficient quantities of our product candidates or produce them at an acceptable quality, which could delay, prevent or impair our development or commercialization efforts and increase costs.

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, 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, which would delay, prevent or impair the development of mavrilimumab.

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 or based on their work for other clinical trial sponsors. 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 other applicable regulatory authorities in other jurisdictions, they will not be able to secure or maintain regulatory approval for their manufacturing facilities. While we may review the compliance history and performance of our CMOs, we have no control over the ability of our CMOs to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or comparable regulatory authorities in other jurisdictions 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.

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. Further, Regeneron has an exclusive right to produce rilonacept for a period of time, which could impact our ability to find a replacement manufacturer for rilonacept in a short-period of time if needed.

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 a manufacturing facility 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 preclinical and early clinical studies for these product candidates.

We built a manufacturing facility to support early development of our product candidates. We may not be able to continually 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 facility 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 processes of mavrilimumab, KPL-716, KPL-404 and our preclinical product candidate and no experience overseeing the manufacturing process of rilonacept. 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, outbreak of disease, accidents, boycotts, labor disputes, political and economic instability, including 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 preclinical 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. If we or any of our third-party providers are not able to establish and maintain procedures and processes sufficient to satisfy cGMP standards, we could experience a delay, interruption or other issues in our manufacture, fill-finish, packaging, storage or delivery of our product candidates, and any related 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.

In addition, we plan to have Regeneron transfer its BLA for rilonacept in the United States for the treatment of CAPS following positive clinical data readout, if any, from our global, pivotal Phase 3 clinical trial, RHAPSODY to enable us to file an sBLA with the FDA to seek approval for the commercial marketing of rilonacept in the United States for the treatment of recurrent pericarditis. The rights with respect to CAPS would remain with Regeneron until the date that we receive such approval from the FDA, if at all. In order for the BLA to be transferred to us, we must coordinate numerous activities with Regeneron in advance and take over certain responsibilities and obligations with respect to owning the BLA. The process to transfer a BLA is complex and resource intensive, and may be more so than we anticipate. Further, if Regeneron or we do not perform in accordance with our expectations, or we are not ready to assume the responsibilities on a timely basis, or the timing of the BLA transfer is otherwise delayed, our ability to file an sBLA with the FDA may be delayed, which could delay FDA approval or our commercialization of rilonacept, and our ability to generate revenue from rilonacept could be materially impaired.

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 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, Regeneron has 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 manufacturing 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, alternative sources of commercial supply may need to be secured, 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 the related product candidate 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 preclinical and clinical programs could be materially and adversely impacted if any of the third-party suppliers upon

which we rely for preclinical and clinical stage product candidate supply 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, is unlikely to 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 of comparable quality 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.

We rely, and expect to continue to rely, on third parties, including independent investigators and CROs, to conduct our research, preclinical 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 rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct or otherwise support our GLP-compliant preclinical 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. While we have agreements governing their activities, we control only certain aspects of these parties' activities and have limited influence over their actual performance. The third parties with whom we contract for execution of our GLP-compliant preclinical 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 preclinical studies and GCP-compliant clinical trials, we 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 are 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, or EEA, and comparable regulatory authorities in other jurisdictions 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 regulatory authorities in other jurisdictions may require us to perform additional clinical trials before approving our marketing applications. We cannot ensure that, upon inspection, the FDA or comparable regulatory authorities in other jurisdictions 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 and oversee all of the clinical trials together with the various clinical trial sites that we engage to conduct the studies. As a result, many important aspects of our development programs for our product candidates, 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;
- have difficulty with or controlling the performance of their subcontractors;
- 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, their subcontractors or the clinical trial sites 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, their subcontractors or the clinical trial sites, 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 CROs, their subcontractors or the clinical trial sites 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 preclinical 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 preclinical 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, their subcontractors or the clinical trial sites 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, subcontractors or clinical trial sites 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, 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.

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, Executing our Strategy, 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 product that modulates the signaling of IL-1α and IL-1β, anakinra (KINERET), marketed by Swedish Orphan Biovitrum AB, and one product that modulates the signaling of IL-1β, canakinumab (ILARIS), marketed by Novartis Pharmaceuticals Corporation. There is also another therapy which modulates IL-1α in clinical

development for diseases other than recurrent pericarditis from Johnson & Johnson. We expect mavrilimumab, if approved, to experience competitive pressure from tocilizumab (ACTEMRA), marketed by Genentech USA, Inc., which was approved in 2017 for use in GCA as an adjunct to steroid taper. Additional competition may be experienced from Eli Lilly and Company and AbbVie, Inc., 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 Biotech, Inc., which is testing ustekinumab (STELARA) in two small studies for GCA. There are multiple other programs targeting GM-CSF antagonism not currently pursuing GCA in clinical trials that could decide in the future to engage in development of therapies for GCA, including GlaxoSmithKline plc, Izana Bioscience and Humanigen, Inc. 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. There are multiple agents targeting antagonism of the CD40/CD40L interaction across a variety of clinical uses including, Novartis International AG, Biogen Inc. and UCB, Inc., C.H. Boehringer Sohn AG & Ko. KG and Abbvie Inc., Annelixis Therapeutics LLC, ImmuNext Inc. and Sanofi S.A., Viela Bio and Astellas Pharma Inc. Further, the results of clinical trials for our product candidates may produce negative, inconclusive or uncompetitive results compared to those produced by any of these or other companies in the indications we are studying, which may result in us deciding, or regulators requiring us, to conduct additional clinical trials or modify or cease development programs for our product candidates.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved 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 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 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.

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 or we may refine or otherwise alter our growth strategy. We may seek to acquire businesses or undertake business combinations, collaborations, or other strategic transactions which may not be successful or on favorable terms, if at all, and we may not realize the intended benefits of such transactions.

We have acquired or in-licensed our existing product candidates, and as part of our strategy 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 transaction 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 and we may need to refine or otherwise alter this strategy. We cannot be certain that we will be successful in such efforts, and even if we are successful in such efforts, we cannot be certain that such discovery or transaction will be on favorable terms, 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 product candidates, technology or businesses often require significant payments and expenses and consume additional resources. We will need to continue to devote a substantial amount of time and personnel to research, develop and commercialize any such in-licensed or acquired product candidate or technology, or integrate any new business, and we may decide to reprioritize our efforts even after having expended resources on a particular prospect. 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;

- we may not succeed in formulation or process development;

- any product candidates to which we acquire the rights or that we discover may not succeed in preclinical studies or clinical trials or 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-party 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;

- any product candidates or technologies to which we acquire the rights or that we discover will take substantial additional financial resources to develop and commercialize and we may not have sufficient funds to do so;

- 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 to identify, discover, develop, in-license or acquire additional product candidates or technologies or to acquire businesses or undertake business combinations, collaborations, or other strategic transactions, or our growth strategy or strategic transactions may not deliver the anticipated results or we may refine or otherwise alter this strategy.

The consummation or performance of any acquisition, business combination, collaboration or other strategic transaction we may undertake in furtherance of our growth strategy or any refined or otherwise altered 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. 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 from the transaction and our business may be materially harmed.

We may seek to enter into collaboration, licensing or other strategic transactions or arrangements to further develop, commercialize or otherwise attempt to realize value from one or more of our product candidates and any such transactions or arrangements that we may enter into may not be successful or be on favorable terms, which could adversely affect our ability to develop, commercialize or attempt to realize value from our product candidates.

We may seek collaboration, licensing or other strategic transactions or arrangements to further develop, commercialize or otherwise attempt to realize value from one or more of our product candidates depending on the merits of retaining rights to develop or commercialize the product candidates ourselves as compared to entering into such transactions or arrangements. In addition, we may seek to jointly develop, commercialize or otherwise exploit one or more of our product candidates with a third party. To the extent that we decide to enter into such transactions or arrangements, we will face significant competition in seeking appropriate collaborators, licensees or other strategic parties. Moreover, these transactions and arrangements are complex and time consuming to negotiate, document, implement and to close or maintain. We may not be successful in our efforts to establish collaborations, licenses or other strategic transactions or arrangements should we so chose to do so. The terms of any such transactions or 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, licenses or other strategic transactions or arrangements that we enter into may not be successful. The success of these potential collaboration, license arrangements and other strategic transactions or arrangements may depend heavily on the efforts and activities of our collaborators, sublicensees or other strategic parties. Collaborations, licenses or other strategic transactions or arrangements are subject to numerous risks, which may include risks that the collaborator, licensee or other strategic party, as applicable:

- may have significant discretion in determining the efforts and resources that they will apply;
- may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out its activities;
- 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;
- may own or co-own intellectual property covering products that results from our arrangement 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; and
- may conduct sales and marketing activities or other operations that may not be in compliance with applicable laws, resulting in civil or criminal proceedings.

In addition, disputes may arise with respect to the ownership of any intellectual property developed pursuant to these arrangements. These arrangements may also be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

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 members of our executive and senior management teams, 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 and certain members of senior management, each of them or we 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. In November 2019, Stephen Mahoney, our Chief Operating Officer left our company to pursue other opportunities and we expect Chris Heberlig, our Chief Financial Officer, to transition to a consulting role in March 2020. Our EVP, Corporate Development and Operations and Chief Legal Officer, Thomas Beetham, and our SVP, Operations and Chief Commercial Officer, Qasim Rizvi, have taken on additional responsibilities with Mr. Mahoney's departure. We have also commenced a search for a new Chief Financial Officer, but we may experience difficulties or delays in identifying a qualified replacement. These or other changes in our senior management may be disruptive to our business, and, if we are unable to manage an orderly transition, our business may be adversely affected. 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 our company and expand our scope of operations, and we may encounter difficulties in managing this development and expansion, which could disrupt our operations.

We expect to continue to develop our company and expand 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 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 and expansion 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 development of our company, expansion of our operations or recruit and train qualified personnel. This may result in weaknesses of our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of one or more 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 of our company and expansion of our operations.

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 Beth Israel Deaconess Medical Center to patent applications and patents related to KPL-404.

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 other 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 EU 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 (an adjustment to the term of the U.S. patent to compensate the patentee for delays caused by the USPTO during the examination process), and in 2023 in Europe, not including any patent term extensions, and the patents covering mavrilimumab as a composition of matter have a term that expires in 2027 in the United States, not including any patent term adjustments or extensions, and in 2027 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 may pursue orphan drug designation for our product candidates in the United States, 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 "Risk Factors— Risks related to marketing approval and regulatory matters."

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. Further, it is 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 other 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 related to 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, 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 the MedImmune Agreement 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. In connection with our acquisition of Primatope Therapeutics, Inc., or Primatope, in March 2019, we acquired an exclusive world-wide license with Beth Israel Deaconess Medical Center for certain patent applications and patents related to KPL-404. 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. These agreements and any future such agreements that we enter into impose a variety of obligations and related consequences.

We are a party to 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 elected to terminate the relevant agreement, which we have the right to do under each of these agreements. 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. Termination of one of these agreements for any reason, and the related discontinuation of the development or commercialization of a product candidate could impair our ability to raise additional capital, generate revenue and may significantly harm our business, financial condition and prospects.

Regeneron has rights to develop rilonacept in its retained fields of local administration to the eye and ear, oncology, deficiency of the IL-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 oncostatin M 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 cease 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 patent lawsuit outside of the United States, alleging our infringement of a competitor's patents, we could be prevented from marketing our products in one or more such 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 governmental patent agencies outside of the United States 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 patent agencies outside of the United States 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 intellectual property laws outside of the United States. In addition, the patent laws of some such 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 jurisdictions outside of the United States. 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 countries outside of the United States 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 jurisdictions outside of the United States, 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 other 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 our patents that may be asserted against us by our competitors or other third parties. Any of these outcomes could have a material adverse effect on our business.

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 product candidates in the United States or jurisdictions outside of the United States 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 product candidates in the United States or any jurisdiction outside of the United States. 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 jurisdictions outside of the United States, 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 not know with specificity. 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 our extrapolation from available population data and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations, pharmacy claims analyses, or market research, and may prove to be incorrect. Further, new trials and therapeutic options may lead to changes in the estimated incidence or prevalence of these diseases, or relevant subpopulations thereof. As a result, the number of patients who may benefit from our product candidates may turn out to be lower than expected.

The total addressable market for any of our product candidates will ultimately depend upon, among other things, the diagnostic criteria and applicable patient population included in the final label for the product candidate approved for sale for its indication, the efficacy, safety, and tolerability demonstrated by the product candidate in our clinical trials, acceptance by the medical community and patients, pricing, access and reimbursement. The number of addressable patients in the United States and other major markets outside of the United States 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 significant market share.

If we are unable to establish and expand our sales, marketing and distribution capabilities, either directly or through agreements with third parties, we may not be successful in commercializing our product candidates, if approved, thus potentially impairing commercial potential for our product candidates to generate any revenue.

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, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. For example, we are currently undertaking plans to establish and develop our sales, marketing, and distribution capabilities and infrastructure to directly commercialize rilonacept, in anticipation of approval.

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 product 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 are unable to retain or reposition our sales and marketing personnel.

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

- our inability to recruit and retain adequate numbers of effective sales, marketing and access personnel;
- the inability of sales personnel to obtain access to physicians and for an adequate number of physicians to prescribe any of our future products;
- the lack of complementary products to be supported 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 relevant to our future products;
- our inability to effectively distribute products in a timely manner;
- our inability to develop or obtain sufficient operational functions and infrastructure to support our commercial activities; and
- unforeseen costs and expenses associated with creating a sales, marketing, and access 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 may have little contractual 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. Furthermore, developing a sales, marketing and access 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, marketing and access organization in the United States, the EU or other key markets. If we do not establish sales, marketing and access capabilities successfully, either on our own or through arrangements 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, patients, or third-party payors (e.g., governments and private health insurers) in which case our ability to generate product revenues will be impaired.

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, the medical community or third-party-payors may not accept or use our product candidates, or effectively block or limit their use in the case of third-party payors. If our product candidates do not achieve an adequate level of acceptance, we may not generate projected level of product revenue or sufficient profits from operations, if at all. 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 or disadvantages of our product candidates relative to 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;
- convenience and ease of administration, including relative to alternative therapies;
- pricing (including patient out-of-pocket costs), budget impact, affordability and cost effectiveness, particularly in relation to alternative treatments;
- the effectiveness of our sales, marketing and distribution activities;
- 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 relevant 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 third-party payors, including governmental authorities and private health insurers, provide funding, establish favorable coverage and pricing policies, and set adequate reimbursement levels. 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 of our product candidates successfully, particularly in orphan or rare disease indications, will depend in part on the availability of favorable coverage and the adequacy of reimbursement for the product candidate and alternative treatments from third-party payors (e.g., governmental authorities, private health insurers and other organizations). Obtaining coverage and adequate reimbursement is contingent on our ability to:

- obtain clinical data that supports payor value/benefit assessments;
- execute formal payor value/benefit assessment processes;
- obtain coverage that enables use in populations reflected in any product candidate's approved product label; and
- effectively negotiate favorable pricing and reimbursement terms.

While in some markets, there is a single payor, in other markets there are multiple payors that can have different ways of assessing prescription drugs. To commercialize our product candidates successfully, we will be required to have sufficient expertise and resources to execute on the respective product candidate's coverage and reimbursement strategy, which we cannot be certain we will be able to do.

Governmental authorities, private health insurers and other third-party payors have attempted to control costs by delaying the time to reimbursement, and by restricting the breadth of patient-coverage and limiting the amount of reimbursement for particular products in terms of lower pricing and increasing the proportion of the cost for which the patient is responsible. There may be significant delays in obtaining reimbursement for newly approved products or product indications, 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, and reimbursement rates may vary according to the use of the product and the clinical setting in which it is used. Coverage and reimbursement barriers by payors may materially impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available, or available only at limited levels, or if such coverage will require patient out-of-pocket costs that are unacceptably high, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval. Moreover, any coverage or reimbursement that may be obtained may be decreased or eliminated in the future.

Third-party payors continue to introduce new tactics to contain costs, including more rigorous value/benefit assessment processes and criteria. It is possible that third-party payors will change the clinical comparators that serve as benchmarks for determining relative value. The result of such a change would be a more challenging value/benefit assessment caused by a more challenging basis for comparison and the potential for a worse relative outcome. Third-party payors may determine that we have failed to generate sufficient evidence to support a value/benefit assessment and refuse to provide coverage and reimbursement, thereby impacting or preventing the progression to a price negotiation. The potential of third-party payors to introduce more rigorous value/benefit assessment processes and criteria could have a negative impact on our ability to commercialize our product candidates successfully.

Third-party payors are also introducing more challenging price negotiation tactics, including in re-visiting established coverage and reimbursement in cases when new competitors, including brands, generics and biosimilars enter the market. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to cover the cost of the alternative product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of competitive products may limit the amount we will be able to charge for our product candidates. Third-party 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. In some cases, when new competitor generic and biosimilar products enter the market, there are mandatory price reductions for the innovator compound, in other cases, payors employ "therapeutic category" price referencing and seek to lower the reimbursement levels for all treatment in the respective therapeutic category. In other cases, new competitor brand drugs can trigger therapeutic category reviews in the interest of modifying coverage and or reimbursement levels. The potential of third-party payors to introduce more challenging price negotiation tactics could have a negative impact on our ability to commercialize our product candidates successfully.

Evolving health policy and associated legislative changes related to coverage and reimbursement aimed at lowering healthcare expenditure could impact the commercialization of our product candidates. Pharmaceutical pricing has been, and likely will continue to be, a central component of these efforts.

The regulations that govern regulatory approvals, pricing and reimbursement for new pharmaceutical products vary widely from country to country. In markets of some countries we may pursue outside of the United States for any of our product candidates, the products may be subject to extensive governmental price control or other price regulations. 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 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 negotiations that delay our commercial launch of the product candidate, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product candidate in that country. Adverse pricing and reimbursement limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Net prices for products may be reduced by mandatory discounts or legislated rebates that must be paid in order to participate in government healthcare programs or paid to other third-party payors. Mandatory discounts can be legislated at any time in any market. Similarly, some markets currently have pricing legislation that sets the price of a pharmaceutical product in their market by referencing the price of that product in other markets, known as international reference pricing. International reference pricing has the potential to impact price cuts in individual countries and the countries that reference the pricing of certain other individual countries. Expansion of mandatory discounts and international reference pricing, including into the United States, presents a material risk to our ability to achieve favorable pricing and adequate reimbursement.

Drug importation and cross-border trade, both sanctioned and unsanctioned, occurs when a pharmaceutical product from a market where the official price is set lower is shipped and made commercially available in a market where the official price is set higher. Any future relaxation of laws that presently restrict or limit drug importation or cross-border trade, including in the United States, could have a material negative impact on our ability to commercialize our product candidates, if approved.

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, we may not be able to achieve or sustain favorable pricing for our product candidates and adequate reimbursement.

Our future growth may depend, in part, on our ability to penetrate markets outside of the United States, 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 markets outside of the United States for which we may rely on collaborations with third parties. For example, we are conducting a global, pivotal 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 markets outside of the United States. 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 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 markets outside of the United States, we would be subject to additional risks and uncertainties, including:

- our ability to obtain reimbursement for our product candidates in such markets;
- our inability to directly control commercial activities because we are relying on third parties;
- the burden of complying with complex and changing regulatory, tax, accounting and legal requirements of such countries;
- different medical practices and customs in such 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 such 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 laws of such country in the event of a contract dispute.

Sales of our product candidates outside of the United States 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, price negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. To obtain adequate reimbursement or favorable pricing approval in some countries, we may be required to conduct a clinical trial that compares 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.

The United Kingdom's withdrawal from the European Union may have a negative effect on global economic conditions, financial markets and our business.

Following a national referendum and enactment of legislation by the government of the United Kingdom, the United Kingdom formally withdrew from the European Union on January 31, 2020 and entered into a transition period during which it will continue its ongoing and complex negotiations with the European Union relating to the future trading relationship between the parties. Significant political and economic uncertainty remains about whether the terms of the relationship will differ materially from the terms before withdrawal, as well as about the possibility that a so-called "no deal" separation will occur if negotiations are not completed by the end of the transition period.

These developments, or the perception that any of them could occur, have had and may continue to have a material adverse effect on global economic conditions and the stability of global financial markets, and may significantly reduce global market liquidity, restrict the ability of key market participants to operate in certain financial markets or restrict our access to capital. For example, there may be uncertainty regarding how clinical trials may be regulated. This could have a material adverse effect on our business, financial condition and results of operations and reduce the price of our shares.

We are currently subject to ongoing obligations, regulatory requirements and review, and if we receive regulatory approval for any of our product candidates, will be subject to additional 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 regulatory authorities outside of the United States approves any of our product candidates, we will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, distribution, storage, advertising, promotion, sampling, record-keeping, AE reporting, conduct of post-marketing trials and submission of safety, efficacy and other post-market information for our approved product candidates, including both federal and state requirements in the United States and requirements of comparable regulatory authorities outside of the United States.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, and comparable regulatory authorities outside of the United States, 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 our CMOs 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 regulatory authorities outside of the United States. Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance.

While our current clinical and medical affairs activities are subject to certain ongoing regulatory requirements concerning appropriate exchange of medical and scientific information, if the FDA or a comparable regulatory authority outside of the United States approves any of our product candidates we will have to comply with additional 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.

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 our product, such as AEs 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 AEs 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 United States federal and state governments and the governments of other countries or jurisdictions 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 (defined to include doctors, dentists,

optometrists, podiatrists and chiropractors), additional categories of healthcare practitioners beginning in 2022 and teaching hospitals, as well as 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 or the appearance of a conflict of interest. For example, 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. The FDA or other regulatory authorities may conclude that a financial relationship between us and a principal investigator or a clinical trial site 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, which could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authorities and may ultimately lead to the denial of marketing approval of our product candidates. Furthermore, investigators for our clinical trials may become debarred by FDA or other regulatory authorities, which may impact the integrity of our studies and the utility of the clinical trial itself may be jeopardized. 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.

Other Risks Related to Our Business

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 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.

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

In the United States, EU 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, 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. For example, 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. On December 14, 2018, a U.S. District Court Judge in Texas ruled that the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the district court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. It is unclear how these decisions, subsequent appeals, if any, or other efforts to challenge, repeal or replace the Affordable Care Act will impact the law or 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 2029 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 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. 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, if approved, or put pressure on our product pricing.

In the EU, 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 EU, 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 the EU, 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 EU 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 or operational 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. Doing business internationally involves a number of other 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 operations outside of the United States;
- 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 technology systems, or those of our third-party CMOs, CROs or other contractors, consultants and service providers, may fail or suffer cyber-attacks or security breaches, which could result in a material disruption of our or such third-party's business or operations and our development programs for our product candidates' or loss of other assets, including funds.

Despite the implementation of security measures, our internal technology systems and those of our third-party CMOs, CROs and other contractors, consultants and service providers are vulnerable to damage from viruses, unauthorized access and attacks, theft, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our business and operations or those of our third-party CMOs, CROs and other contractors, consultants and service providers, it could result in a material disruption of our or such third-party's business or operations and our development programs of our product candidates' or loss of other assets, including funds. 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 acting 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.

In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation. For example, California enacted the California Consumer Privacy Act, or the CCPA, on June 28, 2018, which went into effect on January 1, 2020. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability.

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 EU 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 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.

Securities class action and derivative lawsuits and other legal proceedings are often brought against companies, which could result in substantial costs and divert management's attention.

Securities class action and derivative lawsuits and other legal proceedings are often brought against companies following a decline in the market price of their securities. There can be significant fluctuations in market price for the securities of early-stage biotechnology companies, such as us. As a result, we may be more susceptible to these types of lawsuits and legal proceedings than other companies with more stable security prices. In connection with any litigation or other legal proceedings, we could incur substantial costs, and such costs and any related settlements or judgments may not be covered by insurance. We could also suffer an adverse impact on our reputation and a diversion of management's attention and resources, which could have a material adverse effect on our business.

The EU's 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 EU and has become binding across all EEA member states. 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 business 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.

Additionally, following the United Kingdom's withdrawal from the European Union, we will have to comply with the GDPR and the United Kingdom GDPR, each regime having the ability to fine up to the greater of €20 million/ £17.5 million or 4% of global revenue. The relationship between the United Kingdom and the European Union in relation to certain aspects of data protection law remains unclear, for example around how data can lawfully be transferred between each jurisdiction, which exposes us to further compliance risk.

We and our employees and third parties with whom we contract 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 or third parties with whom we contract, such as our CROs or CMOs, 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 or information regarding our product candidates or clinical trials. Clinical trial patients may also knowingly or inadvertently make use of social media in ways that may not comply with legal or contractual requirements for participation in a clinical trial, including with respect to any AEs they may experience, which may give rise to liability and regulatory risk. 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 preclinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation.

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.

An epidemic or pandemic disease outbreak, including the recent coronavirus, could disrupt our business operations as well as the business or operations of our manufacturers, CROs or other third parties with whom we conduct business, including as a result of significant disruption in travel into and within the countries in which we conduct our clinical trials or our manufacturers produce our product candidates now or in the future, which may have a material adverse effect on our business.

An epidemic or pandemic disease outbreak, including the recent 2019 novel coronavirus, could cause significant disruption in our operations and third party manufacturers and CROs upon whom we rely as well as in the conduct of our clinical trials, including as a result of significant restrictions or bans in travel into and within the countries in which our manufacturers produce our product candidates or where we conduct our clinical trials, which could impede, delay, limit or prevent the production or delivery or release of our product candidates to our clinical trial sites, as well as clinical trial investigators, patients or other critical staff from traveling to our clinical trial sites, all of which could impede, delay, limit or prevent completion of our ongoing clinical trials, and ultimately lead to the delay or denial of regulatory approval of our product candidates, which would materially adversely affect our business and operations.

While there is significant uncertainty relating to the potential effect of the coronavirus on our business and operations, infections may become more widespread and travel restrictions may worsen, including in the United States and other countries where we have or are planning to develop operations, all of which could have a material adverse effect on our business and operations. There could be potential effect of the coronavirus to the business at FDA or other health Authorities, which could result in delays of reviews and approvals of our products. Furthermore, another epidemic or pandemic disease outbreak or the occurrence of a natural disaster, political unrest, war or other events that could disrupt the business or operations of our manufacturers, CROs or other third parties with whom we conduct business now or in the future could materially adversely affect our business and operations.

Risks Related to Our Common Shares

The concentration of ownership of our Class B common shares, which are held primarily by our executive officers and certain other members of our senior management, and the conversion rights of the holders of our Class A1 common shares, which shares are held primarily by entities affiliated with Baker Bros. Advisors LP, or Baker Bros. Advisors, and Class B1 common shares, all of which shares are held by entities affiliated with Baker Bros. Advisors means that such persons are, and such entities may in the future be, able to influence or control certain matters submitted to our shareholders for approval; and such concentration and conversion rights and resulting concentration of control 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.

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. Our Class A1 common shares and Class B1 common shares have no voting rights. As a result, all matters submitted to our shareholders are decided by the vote of holders of our Class A common shares and Class B common shares. 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 the outcome of certain matters submitted to our shareholders for approval. As of February 29, 2020, the holders of Class A common shares accounted for approximately 33% of our aggregate voting power and the holders of Class B common shares accounted for approximately 67% of our aggregate voting power. Our executive officers and certain other members of our senior management hold Class A common shares and Class B common shares representing approximately 51% of our aggregate voting power as of February 29, 2020 and have the ability to control the outcome of certain matters submitted to our shareholders for approval.

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 B common shares has the ability to convert any portion of its Class B common shares into Class B1 common shares or Class A common shares at any time with advance notice to us. 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 with advance notice to us, 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 with advance notice to us. 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 holder provides us with 61-days' prior notice that it intends to increase, decrease or waive such threshold upon conversion. As of February 29, 2020, entities affiliated with Baker Bros. Advisors could convert their Class A1 common shares and Class B1 common shares upon 61-days' prior written notice into Class A common shares and Class B common shares, respectively, which in the aggregate would result in such entities holding approximately 75% of our aggregate voting power.

Due to these conversion rights, holders of our Class A1 common shares and our Class B1 common shares could, at any time with appropriate advance notice to us, 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 and would decrease the ability of the current holders of our Class A common shares and Class B common shares to 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 the holders of Class B common shares who retain their shares in the long term.

This concentrated control limits certain 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 are able to influence or control the outcome of certain matters submitted to our shareholders for approval, including the election of directors. Due to the conversion rights of the holders of our Class A1 and B1 common shares, entities affiliated with the Baker Bros. Advisors could 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. These holders may have interests, with respect to their investment, that are different from our other shareholders. In addition, this concentration of control 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.

The price of our Class A common shares is likely to continue 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 continue 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, our shareholders may not be able to sell their Class A common shares at or above the price they 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 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, capitalization, 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 our inability to obtain additional funding;
- failure to meet or exceed the 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.

In addition, given the limited trading history of our Class A common shares, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common shares and thereby affect the ability of our shareholders to sell their shares. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

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 number of our Class A common shares in the public market, including Class A common shares issuable upon conversion of our Class B, Class A1 and Class B1 common shares, could cause the share price of our Class A common shares to fall.

A significant number of our Class A common shares are issuable upon conversion of our Class B, Class A1, and Class B1 common shares. Our Class B and Class B1 common shares automatically convert into Class A common

shares upon transfer by a holder of such shares to persons or entities not affiliated with such holder. In addition, each holder of our Class B common shares has the ability to convert any portion of its Class B common shares into Class B1 common shares or Class A common shares at any time with advance notice to us, 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 with advance notice to us 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 with advance notice to us. However, 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 holder provides us with 61-days' prior notice that it intends to increase, decrease or waive such threshold upon conversion.

As of February 29, 2020, upon such transfers or conversions up to approximately 35.2 million of additional Class A common shares would be issuable and eligible for resale in the public market to the extent permitted by the provisions of Rule 144 promulgated under the Securities Act of 1933, as amended, or the Securities Act and such rule, Rule 144. In addition, as of February 29, 2020, there were approximately 8.8 million Class A common shares subject to outstanding options and restricted share units under our equity incentive plans that may become eligible for sale in the public market to the extent permitted by the provisions of applicable vesting schedules and Rule 144 and Rule 701 under the Securities Act.

Over a majority of our common shares are held by our executive officers and other members of our senior management together with funds affiliated with certain of our directors. As of February 29, 2020, on an as-converted basis, these shareholders collectively held approximately 32.4 million of our Class A common shares. If any of these shareholders sell, convert or transfer, or indicate an intention to sell, convert or transfer, a substantial amount of their common shares (after certain restrictions on conversion or resale lapse), the market price of our Class A common shares could decline.

Further, as of (i) February 29, 2020, holders of approximately 35.4 million Class A common shares, including Class A common shares issuable upon the conversion of our Class B, Class A1 and Class B1 common shares and upon the exercise of certain rights to acquire Class A common shares, or collectively, registerable securities, are entitled to certain rights with respect to the registration of these shares under the Securities Act pursuant to our amended and restated investor rights agreement, or the investors rights agreement and (ii) June 10, 2019, we registered 28,882,977 Class A common shares (inclusive of 3,000,000 Class A common shares acquired by certain of these holders in our initial public offering of our Class A common shares, or IPO) on an as converted basis from the registrable securities held by certain of these holders pursuant to the investors rights agreement, which are freely tradable without restriction under the Securities Act, to the extent permitted by Rule 144 under the Securities Act.

If any of these 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.

Future sales or issuances of our common shares or rights to purchase common shares, including under our shelf registration statement or 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 under our shelf registration statement or otherwise. 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.

In addition, the consummation or performance of any acquisition, business combination, collaboration or other strategic transaction we may undertake in furtherance of our growth strategy may cause dilution to our existing

shareholders if we issue equity securities for all or a portion of the consideration. For example, we acquired the issued and outstanding equity securities of Primatope in exchange for upfront consideration of $10.0 million paid at closing in March 2019 as well as milestone payments of $5.0 million, which had been achieved and paid as of the closing date, and $3.0 million which was paid in June 2019, all of which paid 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, or the "Primatope Agreement".

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 Jumpstart Our Business Startups, or 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:

- the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more;
- the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO;
- the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or
- the date on which we are deemed to be a large accelerated filer under the rules of the SEC, 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 last business day of our most recently completed second fiscal quarter.

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 we no longer meet either of the criteria for being a smaller reporting company as follows:

- our voting and non-voting common shares held by non-affiliates is less than $250 million measured on the last business day of our second fiscal quarter; or

- our annual revenues are less than $100 million during the most recently completed fiscal year and our voting and non-voting common shares held by non-affiliates is less 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 the share price of our Class A common shares 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. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the SEC 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 increase our legal and financial compliance costs and make some activities more time-consuming and costly, and will increase after we are no longer an emerging growth company and a smaller reporting company. For example, 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 are 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 are not required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To maintain compliance with Section 404 within the prescribed period, we engage 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, engage outside consultants and refine and revise 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 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. 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;

- 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 the Companies Act, 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 Companies Act, or out of or in connection with our amended and

restated 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 amended and restated 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 circumstances 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 other 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 subsidiaries in the United States, the United Kingdom, Germany and France. If we succeed in growing our business, we expect to conduct increased operations through our subsidiaries in various tax jurisdictions subject to transfer pricing arrangements between us and such 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 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;
- changes to and 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 EU Economic and Financial Affairs Council, or 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, or 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. Based on the results of our operations and guidance notes published by the Bermuda Minister of Finance on December 24, 2019, we believe that we are not currently in scope of the Substance Act as we do not believe we currently earn gross income from a "relevant activity," which is a condition to the application of the Substance Act. We will continue to monitor our status with respect to the Substance Act based on our results of operations, and may become subject to the Substance Act in future.

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, 2019, 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 for the year ended December 31, 2019, and because our sole source of income has been and currently is interest on bank accounts held by us, we believe we will be classified as a "passive foreign investment company," or PFIC, for the taxable year ended December 31, 2019. 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 Class A 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 Class A common shares, regardless of whether we continue to meet the PFIC test described above, unless the U.S. Holder makes or has made a "qualified electing fund" election or a "mark-to-market" 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 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 (i) is subject to the supervision of a U.S. court and all substantial decisions of which are subject to 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 (ii) 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 Class A common shares, certain adverse U.S. federal income tax consequences could apply to such U.S. Holder, including (i) the treatment as ordinary income of all or a portion of any gain realized on a disposition of our shares and on the receipt of distributions on our shares to the extent such gain or distribution is treated as an "excess distribution", (ii) the application of a deferred interest charge on such gain and distributions and (iii) the obligation to comply with certain reporting requirements.

If a U.S. Holder is treated as owning at least 10% of our shares, by vote or by value, such holder may be subject to adverse U.S. federal income tax consequences.

We believe we will likely be classified as a controlled foreign corporation for the taxable year ended December 31, 2019. Even if we were not classified as a controlled foreign corporation, because 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 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 such controlled foreign corporation, regardless of whether such corporation makes 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 to 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 Class A common shares.

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

In 2017, the U.S. government enacted comprehensive tax legislation, known as the Tax Cuts and Jobs Act, or the TCJA, that included significant changes to the taxation of business entities. The TCJA remains unclear in many respects and has been, and may continue to be, the subject of amendments and technical corrections, as well as interpretations and implementing regulations by the Treasury and Internal Revenue Service, which have lessened or increased certain adverse impacts of the TCJA and may do so in the future. We continue to examine the impact this the TCJA may have on our business. The effect of the TCJA on our business, whether adverse or favorable, is uncertain, and may not become evident for some period of time. Holders of our shares should consult with their legal and tax advisors regarding the TCJA, and any other such legislation, and the potential tax consequences of investing in our 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 served as President and Chief Executive Officer and was a member of its board of directors until Synageva's 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 previously served as a member of the boards of directors of Syros Pharmaceuticals, Inc., BioCryst Pharmaceuticals, Inc., and Intercept Pharmaceuticals, Inc. 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 Independent Director and on our Board of Directors since October 2015. Dr. Baker is Co-Managing Member of Baker Bros. Advisors LP, a registered investment adviser focused on long-term investments in life-sciences companies, or Baker Bros. Advisors. Dr. Baker and his brother, Julian Baker, started their fund management careers in 1994 when they co-founded a biotechnology investing partnership with the Tisch Family. In 2000, they founded Baker Bros. Advisors. Dr. Baker currently serves on the boards of directors of Alexion, Seattle Genetics, Inc., and Kodiak Sciences Inc., or Kodiak Sciences, and previously served on the board of directors of Genomic Health, Inc., or Genomic Health, and 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 Bros. Advisors. Dr. Biggar joined Baker Bros. Advisors 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 served as a Senior Advisor at Baker Bros. Advisors from December 2016 to May 2019. Prior to that, Dr. Levy served as Executive Vice President and Chief Drug Development Officer at lncyte Corporation, a biopharmaceutical company, from January 2009 until June 2016, and as Senior Vice President of Drug Development from August 2003 to January 2009. Dr. Levy currently serves on the boards of directors of Madrigal Pharmaceuticals, Inc., ArTara Therapeutics, Inc. and Kodiak Sciences, and previously served on the board of directors of Aquinox Pharmaceuticals, 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, Inc., Puma Biotechnology, Inc. and Synageva. Mr. Malley holds a B.S. degree in Biology from Stanford University. Mr. Malley is also a Chartered Financial Analyst.

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. Ms. Popovits served as the Chief Executive Officer of Genomic Health from 2009-2020, and served as the chairman of the board of directors of Genomic Health from 2012-2020. 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 a member of 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 Executive Vice President, Corporate Development and Operations since November 2019 and serves as our Chief Legal Officer, a role he has held since our formation in July 2015, and also serves as our Secretary. Prior to that, Mr. Beetham held various roles at Synageva from October 2013 to June 2015, most recently serving as the Chief Legal Officer and Senior Vice President of Corporate Development, where he led the legal department and was responsible for business development activities. Prior to joining Synageva, from 2011 to 2013, Mr. Beetham was the General Legal Counsel for New England Biolabs, Inc., or Biolabs, where he was responsible for all legal matters and was a member of Biolabs' global business development team. Before Biolabs, Mr. Beetham held various roles at Genzyme, most recently as the lead corporate attorney responsible for Genzyme's hematology/oncology and multiple sclerosis products, and before that was a business and transactional attorney with the law firm of Palmer & Dodge, LLP. Mr. Beetham holds a J.D. from Boston College Law School, an M.B.A. from Boston College's Carroll School of Management, and a B.A. from the University of Rochester.

Chris Heberlig has served as our Chief Financial Officer since our formation in July 2015 and also serves as our Treasurer. 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. Earlier in his career, Mr. Heberlig held senior financial management positions at Panacos Pharmaceuticals, Inc., or Panacos, and EPIX Pharmaceuticals, Inc., or EPIX, both biotechnology companies. Prior to Panacos and EPIX, he worked at PricewaterhouseCoopers LLP, a national audit, tax, and advisory service firm. Mr. Heberlig holds an M.B.A. from Boston University and a B.A. from St. Lawrence University. Mr. Heberlig is also a Certified Public Accountant.

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, or Pfizer, 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. and a 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.

Qasim Rizvi MBChB., MBA., has served as our Senior Vice President of Operations & Chief Commercial Officer since November 2019, prior to that Dr. Rizvi served as our Chief Commercial Officer since July 2019 and was our Senior Vice President, Operations since August 2018. Prior to that, Dr. Rizvi served in roles of increasing responsibility at Genentech, Inc., or Genentech, a member of the Roche Group, or Roche, having joined the company in June 2006. From October 2017 to August 2018, Dr. Rizvi served as Genentech's Vice President and U.S. Franchise Head of Tecentriq, with commercial responsibilities for Genentech's Cancer Immunotherapy portfolio. From September 2016 to October 2017, Dr. Rizvi served as Vice President – Franchise Head Hematology; Global Product Strategy at

Roche, where he led the global hematology franchise. From October 2014 to September 2016, Dr. Rizvi served as Life Cycle Leader at Genentech, where he was responsible for bringing Roche's Hemophilia product from Phase 1b to the global commercialization. Prior to joining Genentech/Roche, Dr. Rizvi was at Novo Nordisk and Eli Lilly, where he served in numerous roles in sales, marketing, business development and new product planning. Dr. Rizvi received his medical degree from the University of Dundee School of Medicine. He completed his residency in general surgery and holds an M.B.A in Marketing and Corporate Strategy from the University of Michigan's Stephen M. Ross School of Business.

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 February 29, 2020, there were 25 holders of record of our Class A common shares, 11 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 shareholders 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

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) and Rule 506 of the Securities Act in a transaction not involving a public offering.

On June 6, 2019, we issued an aggregate of 94,284 Class A common shares to the former shareholders of Primatope, having an aggregate value of approximately $1.5 million, as payment, in part, for the achievement of the final milestone after the closing of the acquisition. These securities were issued under Section 4(a)(2) and Rule 506 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 initial public offering, or 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, or SEC, 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 SEC 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 SEC 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 included elsewhere in this Annual Report and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Annual Report. We have derived the consolidated statement of operations data for the years ended December 31, 2019 and 2018 and the consolidated balance sheet data as of December 31, 2019 and 2018 from our audited consolidated financial statements included elsewhere of this Annual Report. Our historical results are not necessarily indicative of results that may be expected in any future period.

	Years Ended December 31,	
	2019	2018
	(in thousands, except share and per share data)	
Consolidated Statement of Operations Data:		
Operating expenses:		
Research and development	$ 135,001	$ 86,597
General and administrative	34,962	21,563
Total operating expenses	169,963	108,160
Loss from operations	(169,963)	(108,160)
Interest income	6,049	4,719
Loss before benefit for income taxes	(163,914)	(103,441)
Benefit for income taxes	2,047	214
Net loss	$ (161,867)	$ (103,227)
Net loss per share attributable to common shareholders—basic and diluted[(1)]	$ (2.99)	$ (3.49)
Weighted average common shares outstanding—basic and diluted[(1)]	54,049,477	29,547,427

(1) See Note 12 to our consolidated financial statements included elsewhere in this Annual Report for further details on the calculation of basic and diluted net loss per share attributable to common shareholders.

	As of December 31,	
	2019	2018
	(in thousands)	
Consolidated Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$ 233,380	$ 307,304
Working capital[(1)]	213,797	271,196
Total assets	254,534	321,965
Accumulated deficit	(356,092)	(194,225)
Total shareholders' equity	225,423	279,267

(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 included elsewhere in this Annual Report on Form 10-K, or Annual Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report, including information with respect to our plans and strategy for our business, includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, 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 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 with significant unmet medical need. We have a pipeline of clinical-stage product candidates that are based on strong biologic rationale or validated mechanisms, target underserved conditions and offer the potential for differentiation. These assets are designed to modulate immunological signaling pathways that are implicated across a spectrum of diseases. Our product candidates include rilonacept, mavrilimumab, KPL-716 and KPL-404.

Our lead candidate is rilonacept, an interleukin-1α, and interleukin-1β, cytokine trap. We are developing rilonacept for the potential treatment of recurrent pericarditis, a painful inflammatory cardiovascular disease with an estimated U.S. prevalent population of approximately 40,000 patients seeking and receiving medical treatment. We are conducting a global, double-blind, placebo-controlled, randomized-withdrawal design, pivotal Phase 3 clinical trial of rilonacept in subjects with recurrent pericarditis, named RHAPSODY. We expect top-line data from this trial in the second half of 2020. We presented final data from our 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 received Breakthrough Therapy designation from the Food and Drug Administration, or FDA, for rilonacept for the treatment of recurrent pericarditis in 2019, and reported final data from our open label Phase 2 proof of concept clinical trial of rilonacept in a range of recurrent pericarditis populations at the American Heart Association Scientific Sessions in November 2019.

Mavrilimumab is a monoclonal antibody that antagonizes granulocyte-macrophage colony stimulating factor. We are evaluating mavrilimumab for the potential treatment of giant cell arteritis, or GCA, a chronic inflammatory disease of the medium-to-large arteries with an estimated U.S. prevalence of approximately 75,000 to 150,000 patients. We are conducting a randomized, double-blind, placebo-controlled, global Phase 2 proof-of-concept trial for the study of mavrilimumab in GCA. We expect top-line data from this trial in the second half of 2020. In December 2019, we entered into a clinical collaboration with Kite Pharma, Inc., a Gilead Company, or Kite, to initiate a Phase 2 clinical trial evaluating the combination of Yescarta® (axicabtagene ciloleucel) and mavrilimumab in relapsed or refractory large B-Cell lymphoma. The objective of the Phase 2 trial is to determine the effect of mavrilimumab on the safety of Yescarta. Treatment related induction of GM-CSF has been identified through clinical, translational and preclinical studies as a potential key signal associated with side effects of chimeric antigen receptor T, or CAR T, cell therapy. Preclinical evidence suggest the potential for interruption of GM-CSF signaling to disrupt CAR T cell mediated inflammation without disrupting anti-tumor activity. Kite will be the sponsor of this study and responsible for its conduct.

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 are conducting a randomized, double-blind, placebo-controlled, Phase 2a clinical trial of KPL-716 in subjects with prurigo nodularis, a chronic inflammatory skin condition with an estimated U.S. prevalence of approximately 300,000 patients. We expect data from this trial by the end of April 2020. We are also conducting an exploratory Phase 2 clinical

trial in diseases characterized by chronic pruritus. This randomized, double-blind, placebo-controlled trial is designed to identify chronic pruritic conditions where signaling through OSMRβ may be playing a role and to investigate the efficacy, safety and tolerability of KPL-716 in reducing the moderate-to-severe pruritus experienced by these subjects. We expect interim data from cohorts of this trial in the first half of 2020. In August 2019, we reported interim data from our 12 week, repeated single dose cohort of the Phase 1b clinical trial in subjects with moderate-to-severe atopic dermatitis experiencing moderate-to-severe pruritus, which was designed to provide safety and exploratory data on disease response markers.

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. In the first quarter of 2019, we acquired 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. In the second half of 2019, we initiated a single-ascending-dose Phase 1 clinical trial of KPL-404 in healthy volunteers. The first-in-human trial is designed to provide safety and pharmacokinetics data as well as data regarding receptor occupancy and T-cell dependent antibody response (TDAR) in these subjects. We expect top-line data in the second half of 2020.

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 a Phase 3 or other pivotal clinical trial, 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.

On May 23, 2018, our registration statement on Form S-1 relating to our initial public offering, or 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 $161.9 million and $103.2 million for the years ended December 31, 2019 and 2018, respectively. As of December 31, 2019 and 2018, we had an accumulated deficit of $356.1 million and $194.2 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 preclinical and clinical development 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 incur significant expenses in connection with our ongoing activities, particularly as we advance the clinical trials, preclinical activities of our product candidates, and prepare for commercial operations. 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.

As a result, until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through public or private securities offerings, debt financings or other sources, which may include licensing, collaborations or other strategic transactions or arrangements. We may be unable to raise additional funds or enter into such other transactions or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such transactions or arrangements 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, 2019 and 2018, we had cash, cash equivalents and short-term investments of $233.4 million and $307.3 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 from the date of filing this Annual Report. 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 any products, and may never be able to develop and commercialize a marketable product. 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 research and development of our product candidates. We expense research and development costs as incurred. These expenses may include:

- expenses incurred to conduct the necessary preclinical 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 preclinical and clinical drug substance and product for our research and development programs for our product candidates;
- other costs related to acquiring and manufacturing preclinical and clinical trial materials, including manufacturing validation batches, as well as investigative sites and consultants that conduct our clinical trials, preclinical studies and other scientific development services;

- payments made in cash or equity securities under third-party licensing, acquisition and other similar 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 preclinical development, process development, manufacturing and clinical development activities. Our direct research and development expenses by program also include fees incurred under license, acquisition and other similar 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 activities as well as for managing our preclinical and clinical development, process development and manufacturing activities.

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

	Years Ended December 31,	
	2019	2018
	(in thousands)	
Rilonacept	$ 25,677	$ 13,446
Mavrilimumab [1]	13,840	15,260
KPL-716 [2]	28,772	25,562
KPL-404 [3]	22,848	5,967
KPL-045	1,947	5,707
Unallocated research and development expenses	41,917	20,655
Total research and development expenses	$ 135,001	$ 86,597

(1) 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, Limited, or MedImmune.

(2) The amount for the year ended December 31, 2019 includes expense of $10.0 million related to a milestone under our asset purchase agreement with Biogen MA, Inc., or Biogen, associated with the achievement of a specified clinical milestone event.

(3) The amount for the year ended December 31, 2019 includes expense of $18.0 million related to our acquisition of the issued and outstanding equity securities of Primatope and milestone achievements, paid in a combination of cash and Class A common shares (inclusive of escrow and holdback amounts) in accordance with the stock purchase option agreement with Primatope, or the Primatope Agreement. The amount for the year ended December 31, 2018 includes expense of $0.8 million related to the extension of the option period under the Primatope Agreement.

Research and development activities are central to our business. Product candidates in later stages of clinical development generally have higher 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 be substantial over the next several years as we conduct our ongoing and planned clinical trials for rilonacept, mavrilimumab, KPL-716 and KPL-404, as well as conduct other preclinical and clinical development including regulatory filings for our product candidates. As a result, 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 other similar 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 preclinical 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 preclinical 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 continue to prepare for potential commercialization activities and increase our headcount to support our business objectives. We also

anticipate that we will continue to incur significant costs associated with being a public company, including accounting, audit, legal, compliance and director and officer insurance costs as well as investor and public relations expenses.

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 are currently available to us for those losses, while our assets remain in Bermuda. Our wholly owned U.S. subsidiary, Kiniksa Pharmaceuticals Corp., or Kiniksa US, and Primatope are subject to federal and state income taxes in the United States. Our wholly owned subsidiary Kiniksa Pharmaceuticals (UK), Ltd., and its wholly owned subsidiaries, Kiniksa Pharmaceuticals (Germany) GmbH and Kiniksa Pharmaceuticals (France) SARL are subject to taxation in their respective countries. Our benefit for income taxes relates to deferred tax attributes and net credits earned against taxable income generated by our wholly owned subsidiaries, primarily, Kiniksa US, under cost-plus arrangements that they have with us.

As of December 31, 2019 and 2018, the Company had federal research and development tax credit carryforwards of approximately $0.2 million and $0 respectively, available to reduce future tax liabilities, which begin to expire in 2039. As of December 31, 2019 and 2018, the Company had state research and development tax credit carryforwards of approximately $0.3 million and $0.1 million respectively, available to reduce future tax liabilities, which begin to expire in 2034.

Results of Operations

Comparison of the Years Ended December 31, 2019 and 2018

The following table summarizes our results of operations for the years ended December 31, 2019 and 2018:

	Years Ended December 31,		
	2019	2018	Change
		(in thousands)	
Operating expenses:			
Research and development	$ 135,001	$ 86,597	$ 48,404
General and administrative	34,962	21,563	13,399
Total operating expenses	169,963	108,160	61,803
Loss from operations	(169,963)	(108,160)	(61,803)
Interest income	6,049	4,719	1,330
Loss before benefit for income taxes	(163,914)	(103,441)	(60,473)
Benefit for income taxes	2,047	214	1,833
Net loss	$ (161,867)	$ (103,227)	$ (58,640)

Research and Development Expenses

	Years Ended December 31,		
	2019	2018	Change
		(in thousands)	
Direct research and development expenses by program:			
Rilonacept	$ 25,677	$ 13,446	$ 12,231
Mavrilimumab	13,840	15,260	(1,420)
KPL-716	28,772	25,562	3,210
KPL-404	22,848	5,967	16,881
KPL-045	1,947	5,707	(3,760)
Unallocated research and development expenses:			
Personnel related (including share-based compensation)	27,194	15,032	12,162
Other	14,723	5,623	9,100
Total research and development expenses	$ 135,001	$ 86,597	$ 48,404

Research and development expenses were $135.0 million for the year ended December 31, 2019, compared to $86.6 million for the year ended December 31, 2018. The increase of $48.4 million was primarily due to a $27.1 million increase in external fees related to our development programs for our product candidates, as well as an increase of $21.2 million in unallocated research and development expenses.

The direct costs for our rilonacept program were $25.7 million for the year ended December 31, 2019, compared to $13.5 million for the year ended December 31, 2018, or an increase of $12.2 million. During the year ended December 31, 2019, expenses incurred primarily related to conducting RHAPSODY, our global, pivotal Phase 3 clinical trial in recurrent pericarditis, including purchases of drug materials under our clinical supply agreement with Regeneron, compared to the year ended December 31, 2018, in which expenses incurred related to clinical research and development for our Phase 2 open label clinical trial and initiation of RHAPSODY.

The direct costs of our mavrilimumab program were $13.8 million for the year ended December 31, 2019, compared to $15.3 million for the year ended December 31, 2018, or a decrease of $1.5 million. During the year ended December 31, 2019, expenses incurred related primarily to our global Phase 2 clinical trial in GCA and manufacturing process development related expenses, compared to the year ended December 31, 2018, in which expenses incurred related to $5.0 million 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 global Phase 2 trial in GCA and manufacturing process development expenses.

The direct costs for our KPL-716 program were $28.8 million for the year ended December 31, 2019, compared to $25.6 million for the year ended December 31, 2018, or an increase of $3.2 million. During the year ended December 31, 2019, expenses incurred related primarily to a milestone payment of $10.0 million under our asset purchase agreement with Biogen associated with the achievement of a specified clinical milestone event as well as expenses incurred for our Phase 2a clinical trial in prurigo nodularis, our exploratory Phase 2 clinical trial in diseases characterized by chronic pruritus, our Phase 1b clinical trial, and manufacturing and development costs for our clinical drug supply, compared to the year ended December 31, 2018, in which expenses incurred related to manufacturing and development costs for our clinical drug supply, our Phase 1a/1b clinical trial, and our LOTUS-PN observational study.

The direct costs for our KPL-404 program were $22.8 million for the year ended December 31, 2019, compared to $6.0 million for the year ended December 31, 2018, or an increase of $16.8 million. During the year ended December 31, 2019, expenses incurred primarily related to $18.0 million of expense related to the acquisition of the issued and outstanding equity securities of Primatope and milestone achievements, comprised of upfront consideration of $10.0 million paid at closing and milestone payments of $5.0 million, which had been achieved as of the closing date, and $3.0 million, which was achieved after the closing during the year ended December 31, 2019, each paid in a combination of cash and Class A common shares (inclusive of escrow and holdback amounts) in accordance with the Primatope Agreement, as well as preclinical expenses and initiation of our Phase 1 trial. 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. The Primatope acquisition was accounted for as an asset acquisition in 2019 as it did not meet the definition of a business. We recorded the upfront payment, milestone payments and the accrued milestone 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.

The direct costs for our former KPL-045 program were $1.9 million during the year ended December 31, 2019, compared to $5.7 million during the year ended December 31, 2018, or a decrease of $3.8 million. In January 2020, we terminated our license agreement with Novo Nordisk and ceased development of KPL-045. During the year ended December 31, 2019, expenses incurred related to preclinical research and development, including manufacturing development costs, compared to the year ended December 31, 2018, in which expenses incurred related primarily to clinical research and development, including manufacturing development costs.

Unallocated research and development expenses were $41.9 million for the year ended December 31, 2019 compared to $20.7 million for the year ended December 31, 2018. The increase of $21.2 million in unallocated research and development expenses was due to an increase of $12.2 million in personnel-related costs, including share-based compensation, and an increase of $9.1 million in other costs, including research costs related to potential future programs and internal lab costs. The increase in personnel-related costs was primarily due to additional personnel in our research and development functions to support our ongoing clinical trials, including development and manufacture of clinical supply and regulatory filings. Personnel-related costs for the year ended December 31, 2019 and 2018 included share-based compensation of $5.7 million and $2.3 million, respectively.

General and Administrative Expenses

General and administrative expenses were $35.0 million for the year ended December 31, 2019 compared to $21.6 million for the year ended December 31, 2018. The increase of $13.4 million was primarily due to increases of $11.7 million in personnel-related costs and $2.1 million in professional fees, partially offset by a decrease of $0.4 million in other general expenses. The increase in personnel-related costs was due to additional personnel in our general and administrative functions, primarily in our commercial and corporate departments, including legal, finance and human resources. Personnel-related costs for the year ended December 31, 2019 and 2018, included share-based compensation of $9.3 million and $3.4 million, respectively. Professional fees increased due to costs related to commercial preparations, including market research as well as legal costs incurred in connection with maintaining and registering worldwide patents and costs associated with our ongoing business operations, as well as expenses for higher recruiting, accounting and other costs incurred as a public company for a full fiscal year.

Interest Income

Interest income was $6.0 million for the year ended December 31, 2019 compared to $4.7 million for the year ended December 31, 2018. The increase was largely due to higher average invested balances.

Benefit for Income Taxes

For the year ended December 31, 2019, we recorded a benefit for income taxes of $2.0 million relating primarily to the impact of the Foreign Derived Intangible Income, or FDII, deduction for 2019 and 2018; and U.S. federal and state research and development tax credits. FDII was enacted as part of the tax reform enacted by the United States in December 2017, generally referred to as the U.S. Tax Cuts and Jobs Act, with additional proposed regulations and guidance issued in 2019. Upon the completion of our assessment we have included the tax impact of FDII in our income tax expense for the year ended December 31, 2019, based on our understanding of the rules available at the time of the filing of this Annual Report. However, the amounts recorded could be impacted in the future when the proposed regulations and guidance are finalized. For the year ended December 31, 2018, we recorded an insignificant benefit for income taxes.

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, 2019, we had cash, cash equivalents and short-term investments of $233.4 million.

Cash Flows

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

	Years Ended December 31,	
	2019	2018
	(in thousands)	
Net cash used in operating activities	$ (158,369)	$ (81,012)
Net cash provided by (used in) investing activities	49,214	(239,198)
Net cash provided by financing activities	84,107	346,736
Net increase (decrease) in cash and cash equivalents and restricted cash	$ (25,048)	$ 26,526

Operating Activities

During the year ended December 31, 2019, operating activities used $158.4 million of cash, primarily resulting from our net loss of $161.9 million and net cash used by changes in our operating assets and liabilities of $17.0 million partially offset by non-cash charges of $20.5 million. Net cash used by changes in our operating assets and liabilities for the year ended December 31, 2019 consisted of a $15.0 million decrease in accrued milestones, a $4.7 million decrease in accounts payable, and a $1.3 million decrease in operating lease liabilities partially offset by a $1.0 million increase in prepaid expenses and other current assets, a $0.3 million increase in other long-term liabilities and a $4.6 million increase in accrued expenses. The decrease in accrued milestones resulted from the payment of outstanding milestones for which the expense was recognized in prior years. The decrease in accounts payable was primarily due to the timing of vendor invoicing and payments. The increase in prepaid expenses and other current assets was due to increases in prepaid insurance expenses and prepaid expenses to CROs related to our clinical trials. The decrease in operating lease liabilities is due to monthly payments for our right-of-use assets.

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.

Investing Activities

During the year ended December 31, 2019, investing activities provided $49.2 million of cash, consisting of $541.2 million from proceeds of maturities of short-term investments partially offset by $488.8 million of purchases of short-term investments and $3.2 million of purchases of property and equipment.

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.

Financing Activities

During the year ended December 31, 2019, financing activities provided $84.1 million of cash, consisting of $83.0 million from proceeds from our issuance and sale of Class A common shares in a follow-on public offering, inclusive of the concurrent issuance and sale of Class A1 common shares in a private placement, and exercise in part of the underwriters' over-allotment option to purchase additional Class A common shares and concurrent issuance after deduction of underwriting commissions and discounts and other offering costs.

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 partially offset by $3.7 million of payments of other offering costs associated with our IPO, inclusive of the over-allotment option exercise.

Funding Requirements

We expect to incur significant expenses in connection with our ongoing activities, particularly as we advance the clinical trials, preclinical activities of our product candidates and prepare for commercial operations. 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. We expect to incur expenses as we:

- continue to conduct our current clinical trials and/or initiate our planned clinical trials, for rilonacept, mavrilimumab, KPL-716 and KPL-404 as applicable;
- manufacture, or have manufactured on our behalf, our preclinical 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, including rilonacept, for which we may obtain marketing approval and intend to commercialize on our own;

- hire additional clinical, quality and research and development personnel;
- expand our operational, financial and management systems and increase personnel globally 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, if we determine to do so.

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 from the date of filing this Annual Report. 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 and technologies or their related businesses. 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 products, 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 preclinical 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 preclinical development efforts and our clinical trials;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, pricing and reimbursement, distribution and compliance, 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;
- 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 licensing, collaboration or other strategic transactions and arrangements on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates, technologies and their related businesses; 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, or other sources, including, licensing, collaboration, marketing, distribution or other strategic transactions or 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 licensing, collaboration, marketing, distribution or other strategic transactions or arrangements with third parties, we may have to relinquish valuable rights to our technologies, product candidates or future revenue streams, or otherwise agree to terms that may not be favorable to us. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs for product candidates, product portfolio expansion or commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of December 31, 2019 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)		
Manufacturing commitments [1]	$ 2,863	$ —	$ —	$ —	$ 2,863
Operating lease commitments [2]	1,821	972	—	—	2,793
Rilonacept long-term incentive plan [3]	—	2,083	—	—	2,083
Total	$ 4,684	$ 3,055	$ —	$ —	$ 7,739

(1) Amounts in the table reflect commitments for costs associated with our external CMOs, which we have engaged to manufacture 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.

(2) 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.

(3) Amounts in the table reflect the cash awards granted to employees under the Rilonacept Long-Term Incentive Plan, or RLTIP, which become payable in such amounts if the performance goals defined in the RLTIP are achieved at target.

Our contracts with CMOs, CROs and other third parties for the manufacture of our product candidates and to support clinical trials and preclinical 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 $57.5 million in aggregate for the first two indications we develop. In addition, we are obligated to make clinical and regulatory milestone payments of up to $15,000 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 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 $315.0 million upon the achievement of specified clinical and regulatory milestones as well as upon the achievement of annual net sales thresholds. 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.

In January 2020, we terminated our license agreement with Novo Nordisk, as such we are no longer obligated to make future milestone payments under the agreement.

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 included elsewhere in this Annual Report, 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 preclinical development activities;
- CROs and investigative sites in connection with preclinical studies and clinical trials;
- third parties in the connection with the achievement of milestones due under license acquisition and other similar agreements; and
- CMOs in connection with drug substance and drug product formulation and manufacturing of materials.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. 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 all share-based awards granted to employees and directors based on their fair value on the date of the grant and recognize compensation expense for those awards over the requisite service period. Forfeitures are accounted for as they occur. We issue share-based awards with both service-based vesting conditions and performance-based vesting conditions. Expense for awards with service-based vesting is recorded using the straight-line method, and expense for awards with performance-based vesting conditions is recognized using the accelerated-attribution method.

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 our Class A common shares and updated assumption inputs in the Black-Scholes option-pricing model.

We classified share-based compensation expense in the 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 was estimated on the date of grant based on the fair value of the Company’s Class A common shares 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 9). Prior to May 2018, we were

a private company and, accordingly, lacked company-specific historical and implied volatility information. Therefore, we estimate our expected share price 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. 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 we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future.

The fair value of each restricted share unit award is based on the closing price of our Class A common shares on the date of grant. Restricted share unit awards with an associated performance condition are evaluated on a regular basis for probability of achievement, to determine the timing of recording share-based compensation expense to include in our consolidated statements of operations and comprehensive loss.

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 included elsewhere of this Annual Report.

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, 2019, 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, 2019.

In designing and evaluating our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under 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.

Management's Report on Internal Control over Financial Reporting for the Fiscal Year Ended December 31, 2019.

Our management is responsible for establishing and maintaining adequate internal control over our financial reporting, as such term is defined in Rule 13a-15(f) under the Securities Exchange Act of 1934, as amended.

Our management conducted an assessment of the effectiveness of our internal control over financial reporting based on the criteria set forth in "Internal Control – Integrated Framework (2013)" issued by the Committee of Sponsoring Organizations of the Treadway Commission.

Based on this assessment, our management concluded that, as of December 31, 2019, our internal control over financial reporting was effective.

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, 2019 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION.

On March 2, 2020, our Board of Directors established that our 2020 Annual Meeting of Shareholders, or our 2020 Annual Meeting, will be held on Tuesday, June 30, 2020. Because the date of our 2020 Annual Meeting differs by more than thirty days from the anniversary date of the Company's 2019 Annual Meeting of Shareholders held on May 29, 2019, the deadlines for shareholder proposals pursuant to Rule 14a-8 under the Exchange Act, and shareholder proposals for director nominations outside of Rule 14a-8, as listed in our 2019 Proxy Statement on Schedule 14A, filed with the SEC on April 17, 2019, are no longer applicable. Pursuant to Rule 14a-5(f) of the Exchange Act and our amended and restated bye-laws (our "Bye-laws"), we are hereby providing notice of the revised deadlines for such proposals within this Item 9B of this Annual Report.

To be considered for inclusion in this year's proxy statement and related materials under Rule 14a-8 for our 2020 Annual Meeting, shareholder proposals must be in writing and be received by us no later than March 15, 2020, and comply with all applicable rules and regulations promulgated by the SEC under the Exchange Act. In addition, shareholders intending to present a proposal at our 2020 Annual Meeting for director nominations, but not include such nomination in this year's proxy statement and related materials for our 2020 Annual Meeting, must give notice thereof to us in writing no later than March 15, 2020 and comply with all applicable provisions of our Bye-laws (including any

additional information specified in therein). The March 15, 2020 deadline will also apply in determining whether notice of a shareholder proposal is timely for purposes of exercising discretionary voting authority with respect to proxies under Rule 14a-4(c)(1) of the Exchange Act.

Any shareholder proposal for inclusion in our proxy statement and related materials or shareholder notice of intention to present a proposal for director nomination outside of our proxy statement and related materials to be brought before the 2020 Annual Meeting should be sent to: our Secretary c/o Kiniksa Pharmaceuticals Corp. at our offices at 100 Hayden Avenue, Lexington, MA 02421.

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 2020 Annual Meeting to be filed with the SEC within 120 days of December 31, 2019, 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 2020 Annual Meeting to be filed with the SEC within 120 days of December 31, 2019, and is incorporated into this Annual Report by reference.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED SHAREHOLDER MATTERS.

The information required to be disclosed by this Item will be set forth in our proxy statement for our 2020 Annual Meeting to be filed with the SEC within 120 days of December 31, 2019, 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 2020 Annual Meeting 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 2020 Annual Meeting 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	
4.3	Description of Kiniksa Pharmaceuticals, Ltd. Securities					*
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†	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	
10.5†	License Agreement, dated September 25, 2017, by and between the Registrant and Regeneron Pharmaceuticals, Inc.	S-1	333-224488	10.7	4/27/18	
10.6†	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	
10.7	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	
10.8	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	
10.9	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	

10.10	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	
10.11	Form of Indemnification Agreement for Non-Fund-Designated Directors	S-1	333-224488	10.11	4/27/18	
10.12	Form of Indemnification Agreement for Fund-Designated Directors	S-1	333-224488	10.12	4/27/18	
10.13	Form of Indemnification Agreement for Officers	S-1	333-224488	10.13	4/27/18	
10.14	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	
10.15	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	
10.16	2018 Employee Share Purchase Plan	S-1/A	333-224488	10.14	5/14/18	
10.17	Offering Document under the 2018 Employee Share Purchase Plan	10-Q	001-38492	10.6	8/6/18	
10.18	Offering Document under the 2018 Employee Share Purchase Plan	S-1	333-229394	10.22	1/28/19	
10.19	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	
10.20	Non-Employee Director Compensation Program	10-Q	001-38492	10.1	11/5/19	
10.21	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	
10.22	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	
10.23	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	10-K	001-38492	10.27	3/12/19	
10.24	Letter Agreement, dated November 13, 2019, between Kiniksa Pharmaceuticals, Ltd. and Stephen Mahoney					*
10.25	Employment agreement, dated August 20, 2018, by and between Kiniksa Pharmaceuticals Corp. and Qasim Rizvi					*
10.26†	Kiniksa Pharmaceuticals, Ltd. Rilonacept Long-Term Incentive Plan	8-K	001-38492	10.1	12/16/19	

10.27	Form of U.S. Performance Restricted Share Unit and Performance Cash Award Grant Notice and Agreement under the Rilonacept Long-Term Incentive Plan	8-K	001-38492	10.2	12/16/19	
10.28	Form of U.S. Restricted Share Unit Grant Notice and Agreement under the Rilonacept Long-Term Incentive Plan	8-K	001-38492	10.3	12/16/19	
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 Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

KINIKSA PHARMACEUTICALS, LTD.

Date: March 5, 2020

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 Sanj K. Patel	Chief Executive Officer and Chairman of the Board of Directors (principal executive officer)	March 5, 2020
/s/ Chris Heberlig Chris Heberlig	Chief Financial Officer (principal financial officer)	March 5, 2020
/s/ Michael R. Megna Michael R. Megna	VP, Finance and Chief Accounting Officer (principal accounting officer)	March 5, 2020
/s/ Felix J. Baker Felix J. Baker	Lead Independent Director	March 5, 2020
/s/ Stephen R. Biggar Stephen R. Biggar	Director	March 5, 2020
/s/ Richard S. Levy Richard S. Levy	Director	March 5, 2020
/s/ Thomas R. Malley Thomas R. Malley	Director	March 5, 2020
/s/ Tracey L. McCain Tracey L. McCain	Director	March 5, 2020
/s/ Kimberly J. Popovits Kimberly J. Popovits	Director	March 5, 2020
/s/ Barry D. Quart Barry D. Quart	Director	March 5, 2020

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INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

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, 2019 and 2018, 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, 2019 and 2018, 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.

Change in Accounting Principle

As discussed in Note 2 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

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 audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud.

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

Emphasis of Matter

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

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
March 5, 2020

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, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 46,928	$ 71,976
Short-term investments	186,452	235,328
Prepaid expenses and other current assets	8,247	6,446
Total current assets	241,627	313,750
Property and equipment, net	6,398	6,356
Operating lease right-of-use assets	1,927	—
Restricted cash	210	210
Deferred offering costs	—	433
Deferred tax assets	4,372	1,216
Total assets	$ 254,534	$ 321,965
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 5,693	$ 10,918
Accrued expenses	20,415	16,418
Accrued milestones	—	15,000
Operating lease liabilities	1,697	—
Other current liabilities	25	218
Total current liabilities	27,830	42,554
Noncurrent liabilities:		
Noncurrent operating lease liabilities	955	—
Other long-term liabilities	326	144
Total liabilities	29,111	42,698
Commitments and contingencies (Note 13)		
Shareholders' equity:		
Class A common shares, par value of $0.000273235 per share; 19,245,201 shares and 15,797,220 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	6	4
Class B common shares, par value of $0.000273235 per share; 4,638,855 shares issued and outstanding as of December 31, 2019 and December 31, 2018	1	1
Class A1 common shares, $0.000273235 par value; 14,995,954 and 12,995,954 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	4	4
Class B1 common shares, $0.000273235 par value; 16,057,618 shares issued and outstanding as of December 31, 2019 and December 31, 2018	4	4
Additional paid-in capital	581,467	473,483
Accumulated other comprehensive income (loss)	33	(4)
Accumulated deficit	(356,092)	(194,225)
Total shareholders' equity	225,423	279,267
Total liabilities and shareholders' equity	$ 254,534	$ 321,965

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,	
	2019	2018
Operating expenses:		
Research and development	$ 135,001	$ 86,597
General and administrative	34,962	21,563
Total operating expenses	169,963	108,160
Loss from operations	(169,963)	(108,160)
Interest income	6,049	4,719
Loss before benefit for income taxes	(163,914)	(103,441)
Benefit for income taxes	2,047	214
Net loss	$ (161,867)	$ (103,227)
Net loss per share attributable to common shareholders—basic and diluted	$ (2.99)	$ (3.49)
Weighted average common shares outstanding—basic and diluted	54,049,477	29,547,427
Comprehensive loss:		
Net loss	$ (161,867)	$ (103,227)
Other comprehensive income (loss):		
Unrealized gain (loss) on short-term investments and currency translation adjustments, net of tax	37	(4)
Total other comprehensive income (loss)	37	(4)
Total comprehensive loss	$ (161,830)	$ (103,231)

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 Other Comprehensive Income (Loss)	Accumulated Deficit	Total Shareholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
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
Issuance of Class A common shares upon completion of follow-on offering, inclusive of the over-allotment option exercise, net of underwriting discounts and commissions and offering costs	—	—	2,816,110	2	48,474	—	—	48,476
Issuance of Class A1 common shares upon completion of private placement, net of underwriting discounts and commissions and offering costs	—	—	2,000,000	—	34,511	—	—	34,511
Class A common shares issued or to be issued in connection with the acquisition of all issued and outstanding equity securities of Primatope Therapeutics, Inc.	—	—	337,008	—	7,000	—	—	7,000
Class A common shares issued or to be issued in connection with a milestone payment due to Primatope Therapeutics, Inc.	—	—	94,284	—	1,800	—	—	1,800
Exercise of options and issuance of shares under the employee share purchase plan	—	—	200,579	—	1,119	—	—	1,119
Share-based compensation expense	—	—	—	—	15,080	—	—	15,080
Unrealized gain on short-term investments and currency translation adjustments	—	—	—	—	—	37	—	37
Net loss	—	—	—	—	—	—	(161,867)	(161,867)
Balances at December 31, 2019	—	$ —	54,937,628	$ 15	$ 581,467	$ 33	$ (356,092)	$ 225,423

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,	
	2019	**2018**
Cash flows from operating activities:		
Net loss	$ (161,867)	$ (103,227)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	2,068	286
Share-based compensation expense	15,080	5,701
Class A common shares issued or to be issued as consideration for Primatope, including milestone payments	8,800	—
Loss on disposal of property and equipment	21	66
Other	—	235
Non-cash lease expense	1,211	—
Accretion of discounts on short-term investments	(3,501)	(1,423)
Deferred income taxes	(3,156)	(978)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,020)	(4,791)
Accounts payable	(4,705)	8,823
Accrued expenses and other liabilities	4,638	9,296
Accrued milestones	(15,000)	5,000
Operating lease liabilities	(1,264)	—
Other long-term liabilities	326	—
Net cash used in operating activities	(158,369)	(81,012)
Cash flows from investing activities:		
Purchases of property and equipment	(3,203)	(5,290)
Purchases of short-term investments	(488,773)	(402,008)
Proceeds from the maturities of short-term investments	541,190	168,100
Net cash provided by (used in) investing activities	49,214	(239,198)
Cash flows from financing activities:		
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
Proceeds from issuance of Class A common shares from follow-on offering, net of underwriting commissions and discounts, inclusive of the over-allotment option exercise	48,595	—
Proceeds from issuance of Class A1 common shares from private placement, net of underwriting commissions and discounts	34,511	—
Payments of offering costs	(118)	(3,657)
Proceeds from exercise of options and employee share purchase plan	1,119	377
Net cash provided by financing activities	84,107	346,736
Net increase (decrease) in cash and cash equivalents and restricted cash	(25,048)	26,526
Cash, cash equivalents, and restricted cash at beginning of period	72,186	45,660
Cash, cash equivalents, and restricted cash at end of period	$ 47,138	$ 72,186
Supplemental information:		
Cash paid for income taxes	$ 1,724	$ 383
Supplemental disclosure of non-cash investing and financing activities:		
Deferred offering costs included in accrued expenses and accounts payable	$ —	$ 404
Property and equipment included in accrued expenses and accounts payable	$ 222	$ 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 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.

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"), Primatope Therapeutics, Inc. ("Primatope") and Kiniksa Pharmaceuticals (UK), Ltd. ("Kiniksa UK") as well as the subsidiaries of Kiniksa UK, Kiniksa Pharmaceuticals (Germany) GmbH ("Kiniksa Germany") and Kiniksa Pharmaceuticals (France) SARL ("Kiniksa France"), after elimination of all significant intercompany accounts and transactions.

In assessing the consolidation requirement for variable interest entities ("VIE"s), 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, 2019 and 2018 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 financial results of the Company's global activities are reported in U.S. dollars ("USD") and its foreign subsidiaries generally utilize their respective local currency to be their functional currency.

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 subsidiaries 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").

Follow-on Offering and Private Placement

On February 4, 2019, the Company completed a follow-on offering of 2,654,984 Class A common shares at a public offering price of $18.26 and a concurrent private placement of 2,000,000 Class A1 common shares at an offering price of $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 a public offering price of $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 $82,988 after deducting underwriting discounts and commissions and other offering costs

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, 2019, the Company had an accumulated deficit of $356,092. During the year ended December 31, 2019, the Company incurred a net loss of $161,867 and used $158,369 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, 2019, the Company had cash, cash equivalents and short-term investments of $233,380.

Based on its current operating plan, the Company expects that its cash, cash equivalents and short-term investments, 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. The Company will need to finance its operations through public or private securities offerings, debt financings or other sources, which may include licensing, collaborations or other strategic transactions or arrangements. 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 for product candidates, 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, 2019 and 2018, 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.

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, cash equivalents and short-term investments. At December 31, 2019 and 2018, substantially all of the Company's cash, cash equivalents and short-term investments 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, cash equivalents and short-term investments and does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

Restricted Cash

In conjunction with the Company's lease agreement entered into in March 2018 (see Note 5), the Company maintains a letter of credit for the benefit of the landlord. As of December 31, 2019 and 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 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 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. As of December 31, 2018, the Company recorded deferred offering costs of $433 related to the follow-on offering of its Class A common shares and private placement of its Class A1 Common Shares. The follow-on offering and concurrent private placement

were finalized in February 2019, and all prior deferred costs were recorded as a reduction to the carrying value. There were no deferred offering costs recorded as of December 31, 2019.

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 and short-term investments, 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.

Leases

In February 2016, the Financial Accounting Standards Board ("FASB") issued ASU No. 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), which set forth the principles for recognition, measurement, presentation and disclosure of lease arrangements to enhance the transparency and comparability of financial reporting related to the arrangements. ASU 2016-02, including subsequently issued amendments, is collectively referred to as Accounting Standards Codification, *Leases (Topic 842)* ("ASC 842"). The Company adopted the new standard on January 1, 2019 using the modified retrospective transition approach as applied to leases existing as of the adoption date. The standard will be applied to all leases entered into after the initial adoption date.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a "lease" as defined by ASC 842. A lease is an arrangement, or part of an arrangement, that conveys the right to control the use of identified property, plant, or equipment (an identified asset) for a period of time in exchange for consideration. The Company determines if the arrangement conveys the right to control the use of an identified asset for a period of time. It assesses throughout the period of use whether the Company has both of the following (1) the right to obtain substantially all of the economic benefits from use of the identified asset and (2) the right to direct the use of the identified asset. This determination is reassessed if the terms of the arrangement are changed. Leases are classified as operating or finance leases based on the terms of the lease agreement and certain characteristics of the identified asset. Right-of-use ("ROU") assets and lease liabilities are recognized at lease commencement date based on the present value of the minimum future lease payments.

Most leases with a term greater than one year are recognized on the balance sheet as ROU assets with corresponding lease liabilities and, if applicable, long-term lease liabilities. The Company has elected not to recognize leases with a term of one year or less on its balance sheet. Operating leases, ROU assets and their corresponding lease liabilities are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the ROU assets may be required for items such as incentives received. The interest rate implicit in lease arrangements is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate, which is the rate incurred to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with the guidance in ASU 2016-02, components of a lease should be split into three categories: lease components (e.g., land, building, etc.), non-lease components (e.g., common area maintenance, consumables, etc.), and non-components (e.g., property taxes, insurance, etc.); then the fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on fair values to the lease components and non-lease components.

Although separation of lease and non-lease components is required, certain practical expedients are available. Companies may elect the practical expedient to not separate lease and non-lease components. In which case, the Company would account for each lease component and the related non-lease component together as a single component. The Company has elected to account for the lease and non-lease components of each of its operating leases as a single lease component and allocate all of the arrangement consideration to the lease component only. The lease component results in an operating right-of-use asset being recorded on the balance sheet and amortized on a straight-line basis as lease expense.

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 preclinical and 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. Milestone and other payments made to third-parties with respect to in-process research and development, in accordance with the Company's license, acquisition and other similar agreements are expensed when determined to be probable and estimable.

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 and development 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. Forfeitures are accounted for as they occur. The Company issues share-based awards with both service-based vesting conditions and performance-based vesting conditions. Expense for awards with service-based vesting is recorded using the straight-line method, and expense for awards with performance-based vesting conditions is recognized using the accelerated-attribution method.

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 common shares 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 9). Prior to May 2018, the Company was a private company and, accordingly, 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.

The fair value of each restricted share unit award is based on the closing price of the Company’s Class A common shares on the date of grant. Restricted share unit awards with an associated performance condition are evaluated on a regular basis for probability of achievement, to determine the timing of recording share-based compensation expense in the Company’s consolidated statements of operations and comprehensive loss.

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, 2019 and 2018, the Company's other comprehensive loss was comprised of unrealized gain (loss) on short-term investments and currency translation adjustments, net of tax.

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 benefit 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.

As an exempted company incorporated under the laws of 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 losses incurred in Bermuda during each reporting period, and no net operating loss carryforwards will be available to the Company for those losses. The Company's wholly owned U.S. subsidiary, Kiniksa US, and Primatope are subject to federal and state income taxes in the United States. The Company's wholly owned subsidiary Kiniksa UK., and its wholly owned subsidiaries, Kiniksa Germany and Kiniksa France are subject to taxation in their respective countries.

The Company's benefit for income taxes primarily relates to net credits earned against taxable income generated by its wholly owned subsidiaries, primarily, Kiniksa US, under cost-plus arrangements that they have with the Company. The benefit for income taxes is impacted by the Foreign Derived Intangible Income, or ("FDII"), deduction and U.S. federal and state research and development tax credits. FDII was enacted as part of the tax reform enacted by the United States in December 2017, generally referred to as the U.S. Tax Cuts and Jobs Act with additional proposed regulations and guidance issued in 2019. Upon the completion of the Company's assessment, the Company has included the tax impact of FDII in its income tax expense for the year ended December 31, 2019, based on its understanding of the rules available at the time of this filing. However, the amounts recorded could be impacted in the future when the proposed regulations and guidance are finalized.

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. In 2019, the Company identified an uncertain tax position related to historic research and development credits as a result of a R&D study performed (see Note 11).

Net Loss per Share

The Company follows the two-class method when computing net loss per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net 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 loss per share attributable to common shareholders is computed by dividing the net loss attributable to common shareholders by the weighted average number of common shares outstanding for the period. Diluted net loss attributable to common shareholders is computed by adjusting net loss attributable to common shareholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share attributable to common shareholders is computed by dividing the diluted net 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.

Recently Adopted Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board ("FASB") issued Account Standards Update ("ASU"), No. 2016-02, *Leases (Topic 842) ("ASU 2016-02")*. The standard, including subsequently issued amendments, collectively referred to as Accounting Standards Codification ("ASC") 842, established principles of recognition, measurement, presentation and disclosure of lease arrangements applicable to lessees and lessors in order to enhance the transparency and compatibility of financial reporting related to the arrangements, including with respect to the amount, timing and uncertainty of cash flows arising from a lease. The Company adopted new accounting guidance regarding the accounting for leases as of January 1, 2019 using a modified retrospective transition approach that was applied to leases existing as of, or entered into prior to, January 1, 2019. See Note 2, Summary of Significant Accounting Policies, "Leases" for a discussion of the Company's policy with respect to this standard and Note 5, "Leases" for a discussion of the Company's adoption of this standard and its impact on its consolidated financial statements and related disclosures.

Upon the adoption of ASC 842, the Company recorded operating lease right-of-use assets of $3,682 and operating lease liabilities of $3,917 for its leases which were in effect and had commenced prior to January 1, 2019 and had original lease terms of more than 12 months.

Recently Issued Accounting Pronouncements

In August 2018, the FASB issued ASU 2018-15, *Intangibles—Goodwill and Other— Internal-Use Software (Subtopic 350-40) ("ASU 2018-15")*, which amends ASC 350-40 to address a customer's accounting for implementation costs incurred in a cloud computing arrangement ("CCA") that is a service contract. ASU 2018-15 aligns the accounting for costs incurred to implement a CCA that is a service contract with the guidance on capitalizing costs associated with developing or obtaining internal-use software. Specifically, the ASU amends ASC 350 to include in its scope implementation costs of a CCA that is a service contract and clarifies that a customer should apply ASC 350-40 to determine which implementation costs should be capitalized in a CCA that is considered a service contract. The standard became effective for the Company beginning on January 1, 2020. The Company is currently evaluating the potential impact that ASC 350-40 may have on its consolidated financial statements and related disclosures .

In June 2016, the FASB, issued ASU 2016-13, *Financial Instruments: Credit Losses (Topic 326)("ASU 2016-13)"*, as clarified in ASU 2019-04 and ASU 2019-05. The objective of the standard is to provide information about expected credit losses on financial instruments at each reporting date and to change how other-than-temporary

(Amounts in thousands, except share and per share amounts)

impairments on investment securities are recorded. The standard became effective for the Company beginning on January 1, 2020. The Company does not anticipate the adoption of this ASU to have a material impact on its consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU 2019-12, *Simplifying the Accounting for Income Taxes (Topic 740)("ASU 2019-12")*. The objective of the standard is to improve areas of GAAP by removing certain exceptions permitted by ASC Topic 740 Income Taxes and clarifying existing guidance to facilitate consistent application. The standard will become effective for the Company beginning on January 1, 2021. The Company is currently evaluating the new standard to determine the potential impact of ASU 2019-12 on its consolidated financial statements and related disclosures.

3. Fair Value of Financial Assets and Liabilities

Short-term investments as of December 31, 2019 and 2018 consisted of U.S. Treasury notes all of which are due within six months. As of December 31, 2019 and 2018, the fair value of short-term investments was $186,452 and $235,328, respectively. As of December 31, 2019, the amortized cost was $186,415 and gross unrealized gain was $37. As of December 31, 2018, the amortized cost was $235,332 and gross unrealized loss was $4.

The following tables present information about the Company's financial instruments 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, 2019 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Restricted cash — money market funds	$ 210	$ —	$ —	$ 210
Cash equivalents — money market funds	25,207	—	—	25,207
Cash equivalents — U.S. Treasury notes	—	10,192	—	10,192
Short-term investments — U.S. Treasury notes	—	186,452	—	186,452
	$ 25,417	$ 196,644	$ —	$ 222,061

	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

During the years ended December 31, 2019 and 2018, 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 and short-term investments as of December 31, 2019 and 2018 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.

(Amounts in thousands, except share and per share amounts)

4. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31, 2019	December 31, 2018
Furniture, fixtures and vehicles	$ 47	$ 91
Computer hardware and software	344	249
Leasehold improvements	3,627	2,676
Lab equipment	4,685	3,107
Construction in progress	20	552
Total property and equipment	8,723	6,675
Less: Accumulated depreciation	(2,325)	(319)
Total property and equipment, net	$ 6,398	$ 6,356

As of December 31, 2019, construction in progress is primarily comprised of lab equipment which the Company anticipates will be placed into service in 2020.

Depreciation expense for the years ended December 31, 2019 and 2018 was $2,068 and $286, respectively.

5. Leases

Kiniksa US leases office and laboratory space under operating leases. Leases with an initial term of 12 months or less are not recorded on the balance sheet; the Company recognizes lease expense for these leases on a straight-line basis over the lease term. For lease agreements entered into or reassessed after the Company's adoption of ASC 842, the Company will combine lease and non-lease components. Kiniksa US's leases have remaining lease terms of less than 2 years.

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 were 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 is $138 per month as of 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 components of lease cost consisted of operating lease costs and variable lease costs were $1,443 and $152 for the year end ended December 31, 2019. As of December 31, 2019, the weighted-average lease term was 1.47 years and the discount rate was 7.16%.

(Amounts in thousands, except share and per share amounts)

Maturities of operating leases liabilities were as follows:

As of December 31,	
2020	$ 1,821
2021	972
2022	—
2023 and thereafter	—
Total future minimum lease payments	$ 2,793
Less imputed interest	(141)
Present value of lease liabilities	$ 2,652

Prior to the adoption of the new lease accounting standard, undiscounted future minimum rents payable as of December 31, 2018 under non-cancelable leases with the initial term exceeding one year were as follows:

As of December 31,	
2019	$ 1,394
2020	1,821
2021	972
2022 and thereafter	—
Total future minimum lease payments	$ 4,187

6. Accrued Expenses

Accrued expenses consisted of the following:

	December 31, 2019	December 31, 2018
Accrued research and development expenses	$ 11,813	$ 9,656
Accrued employee compensation and benefits	7,089	5,678
Accrued legal and professional fees	1,087	994
Other	426	90
	$ 20,415	$ 16,418

7. 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.

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, 2019, no preferred shares were designated or issued.

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 preferred shares were entitled to the number of votes per Series B preferred share equal to the number of whole Class A common shares into which the Series B preferred shares were convertible at the time of such vote (which was one vote for each Class A common share). The holders of Series C preferred shares were entitled to the number of votes per Series C preferred share equal to the number of whole Class A common shares into which the Series C preferred shares were convertible at the time of such vote (which was 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. No cash dividends were declared or paid on the Preferred Shares.

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.

8. 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 a public offering price of $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 in part of their over-allotment option to purchase additional shares at a public offering price of $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 at a public offering price of $18.26 and a concurrent private placement of 2,000,000 Class A1 common shares at an offering price of $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 a public offering price of $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 $82,988 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, transferability 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, 2019, 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, 2019, no preferred shares were designated or issued. Through December 31, 2019, 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 (subject to certain exceptions). Each Class B1 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 B1 common share is convertible into one Class A common share or one Class B common share at the holder's election (subject to certain exceptions). There are no conversion rights associated with the Class A common shares.

9. 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. 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. Upon the effectiveness of the 2018 Plan, the Company ceased granting awards under its 2015 Equity Incentive Plan (as amended, the "2015 Plan" together with the 2018 Plan, the "Plans").

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. In 2019, the board of directors approved an increase of 1,979,586 shares, equal to 4% of the as-converted Class A common shares outstanding on December 31, 2018. 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, 2019, 2,145,907 shares remained available for future grant.

Rilonacept Long-Term Incentive Plan

In December 2019, the compensation committee of the Company's board of directors approved the Company's Rilonacept Long-Term Incentive Plan ("RLTIP") under the Company's 2018 Plan to incentivize eligible employees of the Company or any of its subsidiaries to achieve U.S. Food and Drug Administration ("FDA") approval for the commercial sale and marketing of rilonacept for the treatment of recurrent pericarditis in the United States ("RLTIP Milestone"). The RLTIP provides for the potential to receive a cash award and two grants of restricted share unit ("RSU") awards covering Class A common shares under the 2018 Plan. The target award value for each of the cash award and the two RSU awards will be equal to one-third of a participant's annual target bonus for the year of grant, as determined in accordance with the RLTIP.

Depending on the date-range within which the RLTIP Milestone is achieved (such date the "Achievement Date") occurs, the RLTIP provides for (i) an earnout percentage that can be achieved as to 100%, 50%, 25% or 0% and (ii) an upside earnout percentage that can be achieved as to 50%, 25% or 0%. No awards will be earned or vest, and the second RSU award will not be granted, in the event the Achievement Date does not occur by a specified date.

The cash award is eligible to be earned and vested upon the Achievement Date with respect to an amount determined in accordance with the RLTIP based on the earnout percentage. The number of Class A common shares issuable under the first RSU award ("First RSU Award") as a result of the RLTIP Milestone will be determined in accordance with the RLTIP based on the earnout percentage, and such RSUs will vest on the first anniversary of the Achievement Date, subject to continued employment on such date. The second RSU award will be granted on the Achievement Date with respect to a number of shares determined in accordance with the RLTIP, based on both the earnout percentage and the upside earnout percentage, and will vest on the second anniversary of the Achievement Date, subject to continued employment through such date.

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, 2019, there were 4,125,923 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, 2019 and 2018

During the years ended December 31, 2019 and 2018, the Company granted options to purchase 3,605,388 and 3,114,139 Class A common shares, respectively, to employees and directors. The Company recorded share-based compensation expense for options granted to employees and directors of $13,322 and $5,464 during the years ended December 31, 2019 and 2018, respectively.

During the years ended December 31, 2019 and 2018, the Company granted options to purchase 3,200 and 4,000 Class A common shares, respectively, to non-employees. The Company recorded share-based compensation expense for options granted to non-employees of $94 and $130 during the years ended December 31, 2019 and 2018, 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

(Amounts in thousands, except share and per share amounts)

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, 2019, 598,334 Class A common shares were available for future issuance under the 2018 ESPP. In December 2019, the Company's board of directors determined that the January 1, 2020 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, 2019 and 2018 were as follows, presented on a weighted-average basis:

	Years Ended December 31,	
	2019	2018
Risk-free interest rate	2.07 %	2.82 %
Expected term (in years)	6.22	6.40
Expected volatility	79.14 %	75.04 %
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,	
	2019	2018
Risk-free interest rate	1.38 %	2.91 %
Expected term (in years)	2.77	7.35
Expected volatility	68.17 %	74.18 %
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 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.

(Amounts in thousands, except share and per share amounts)

The following table summarizes option activity for the year ended December 31, 2019:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2018	5,960,939	$ 10.25	8.56	$ 108,352
Granted	3,608,588	$ 13.44		
Exercised	(150,253)	$ 3.93		
Forfeited	(927,540)	$ 15.26		
Outstanding as of December 31, 2019	8,491,734	$ 11.17	7.88	$ 27,217
Options exercisable as of December 31, 2019	3,373,659	$ 7.29	6.24	$ 19,903
Options unvested as of December 31, 2019	5,118,075	$ 13.74	8.96	$ 7,314

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, 2019, option holders exercised 150,253 options for Class A common shares with an intrinsic value of $1,776 for total cash proceeds to the Company of $590. 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.

The weighted-average grant-date fair value per share of options granted during the years ended December 31, 2019 and 2018 was $9.36 and $11.96, respectively.

The total fair value of options vested during the years ended December 31, 2019 and 2018 was $13,997 and $2,255, respectively.

Restricted Shares

Under terms of the Class A and Class B restricted share agreements covering the Class A and Class B common shares, 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 became 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, 2019:

	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, 2018	133,812	$ 0.000273235	743,407	$ 0.000273235
Granted	—	—	—	—
Vested	(133,812)	$ 0.000273235	(743,407)	$ 0.000273235
Unvested restricted shares outstanding as of December 31, 2019	—	—	—	—

The aggregate fair value of restricted shares that vested during the years ended December 31, 2019 and 2018 was $12,154 and $15,182, respectively.

Restricted Share Units

An RSU represents the right to receive one share of the Company's Class A common share upon vesting of the RSU. The fair value of each RSU is based on the closing price of the Company's Class A common shares on the date of grant. In December 2019, the Company granted RSUs with service conditions that vest in one installment provided that the recipient remains employed with the Company ("Time-Based RSUs") through December 31, 2020. During the year ended December 31, 2019, the Company also granted the First RSU Award as part of the RLTIP, which becomes eligible to vest upon the Achievement Date and will vest upon the first anniversary of such date, subject to the recipient's continued employment through that date. In the event the RLTIP Milestone is not achieved, none of the First RSU Award will vest.

The grant-date fair value of the Time-Based RSUs granted in 2019 was $1,250 and will be recognized on a straight-line basis through the vest date for these shares. For the year ended December 31, 2019, the Company recognized $65 in Time-Based RSU expense. The grant-date fair value of the First RSU Award was $2,994 and will be recognized when the RLTIP Milestone is deemed probable of achievement through the date the First RSU Award will vest. During the year ended December 31, 2019, the Company did not recognize compensation expense related to the First RSU Award, as achievement of the RLTIP Milestone was determined to be not probable.

The following table summarizes RSU activity for the year ended December 31, 2019:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested RSUs as of December 31, 2018	—	$ —
Granted	328,296	$ 12.93
Vested	—	$ —
Forfeited	—	$ —
Unvested RSUs as of December 31, 2019	328,296	$ 12.93

(Amounts in thousands, except share and per share amounts)

Share-Based Compensation

Share-based compensation expense was classified in the consolidated statements of operations and comprehensive loss as follows:

	Years Ended December 31,	
	2019	2018
Research and development expenses	$ 5,746	$ 2,285
General and administrative expenses	9,334	3,416
	$ 15,080	$ 5,701

As of December 31, 2019, total unrecognized compensation cost related to the unvested option-based awards was $44,185, which is expected to be recognized over a weighted average remaining period of 3.1 years. As of December 31, 2019, total unrecognized compensation cost related to the unvested Time-Based RSUs was $1,185, which is expected to be recognized over a weighted average remaining period of 1.0 year. As of December 31, 2019, total unrecognized compensation cost related to the First RSU Award was $2,994, which will be recognized when the RLTIP Milestone is deemed probable of achievement through the date the First RSU Award vests.

10. 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 milestone payments of $4,000 and $10,000 paid during the year ended December 31, 2017 and the year ended December 31, 2019, respectively, each payment was 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, 2019 and 2018.

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).

During the year ended December 31, 2019, the Company recorded research and development expense of $10,347 primarily related to milestone payments associated with the achievement of a specified clinical milestone event as well as other payments in connection with the retained contracts due under the Biogen Agreement. 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

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 was 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.

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.

In January 2020, the Company terminated the Novo Nordisk Agreement in accordance with the terms and conditions of the agreement.

Under the Novo Nordisk Agreement, the Company was 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 was recorded in the Company's consolidated statement of operations for the year ended December 31, 2018. In addition, the Company was 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, 2019 and December 31, 2018, 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, 2019 and 2018.

Under the Novo Nordisk Agreement, the Company was 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 Company did not record any research and development expense during the year ended December 31, 2019 in connection with milestone payments due under the Novo Nordisk Agreement. 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.

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 preclinical 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 preclinical work to assess the viability of the asset.

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.

In January 2019, the Company exercised the call option and in March 2019, the Company acquired all of the issued and outstanding equity securities of Primatope (the "Primatope Acquisition") in exchange for $18,000 comprised of upfront consideration of $10,000 at closing and milestone payments of $5,000,which had been achieved as of the closing date, and in June 2019, the Company made the final milestone payment of $3,000, which was achieved during the year ended December 31, 2019 following the closing, each paid in a combination of cash and Class A common shares (inclusive of escrow and holdback amounts) in accordance with the Primatope Agreement. At the closing of the Primatope Acquisition, Primatope became a wholly owned subsidiary of the Company and the acquisition was accounted for as an asset acquisition as it did not meet the definition of a business. The Company recorded the upfront payment and milestone payments 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.

During the year ended December 31, 2019, the Company did not incur any research and development expense directly in connection with milestone or other payments related to the Primatope Acquisition or the Primatope Agreement. During the year ended December 31, 2019, the Company recorded research and development expense of $18,000 related to the Primatope Acquisition. 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.

Beth Israel Deaconess Medical Center License Agreement

As a result of the Primatope Acquisition, the Company acquired the rights to an exclusive license to certain intellectual property rights controlled by Beth Israel Deaconess Medical Center, Inc. ("BIDMC") to make, use, develop and commercialize KPL-404 (the "BIDMC Agreement"). Under the BIDMC 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. Under the BIDMC Agreement, the Company is obligated to pay an insignificant annual maintenance fee as well as clinical and regulatory milestone payments of up to an aggregate of $1,200 to BIDMC. The Company is also obligated to pay a low single-digit royalty on annual net sales of products licensed under the agreement.

During the year ended December 31, 2019, the Company recorded research and development expense of $10 in connection with the BIDMC 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 for all commercial products, 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, 2019 and 2018, the Company recorded research and development expense of $6,854 and $1,835, respectively, related to the purchase of drug materials under this agreement. As of December 31, 2019, the Company has non-cancelable purchase commitments under the clinical supply agreement (see Note 13).

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, 2019 and 2018.

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. During the year ended December 31, 2019, the Company made both the $5,000 and $10,000 previously accrued milestone payments in accordance with the MedImmune Agreement. 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, 2019, the Company did not record research and development expense in connection with the milestone payments due under the MedImmune Agreement. During the year ended December 31, 2018, the Company recorded research and development expense $5,000, related primarily to a pass-through payment due upon the achievement of specified clinical milestone events due under the MedImmune Agreement.

Kite Clinical Collaboration Agreement

In December 2019, we entered into a clinical collaboration (the "Kite Agreement") with Kite Pharma, Inc., a Gilead Company ("Kite"), to initiate a Phase 2 clinical trial evaluating the combination of Yescarta (axicabtagene ciloleucel) and mavrilimumab in relapsed or refractory large B-Cell lymphoma. The objective of the Phase 2 trial is to determine the effect of mavrilimumab on the safety of Yescarta. Treatment related induction of GM-CSF has been identified through clinical, translational and preclinical studies as a potential key signal associated with side effects of chimeric antigen receptor T, or CAR T, cell therapy. Preclinical evidence suggest the potential for interruption of GM-CSF signaling to disrupt CAR T cell mediated inflammation without disrupting anti-tumor activity. Kite will be the

(Amounts in thousands, except share and per share amounts)

sponsor of this study and responsible for its conduct. Under the Kite Agreement, Kite and the Company shall share a portion of the costs incurred in conducting the study.

The Kite Agreement will expire on the one-year anniversary of the date that Kite provides the clinical study report to the Company or unless otherwise terminated in accordance with the Kite Agreement.

The Company evaluated the agreement and determined all costs and expenses will be recognized as incurred as research and development expenses. During the year ended December 31, 2019, the Company did not record any research and development expense in connection with the Kite Agreement.

11. 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. On March 9, 2019, the Company entered into an agreement with Primatope to license certain intellectual property rights related to KPL-404. Kiniksa US and Primatope are subject to tax in the United States for federal and state tax purposes.

The Company has a wholly owned subsidiary in Europe, Kiniksa UK (formed in December 2018). Kiniksa UK has two wholly owned subsidiaries in Europe, Kiniksa Germany (formed in February 2019), and Kiniksa France (formed in June 2019). Each are subject to taxation in their respective countries.

Income (loss) before benefit for income taxes consisted of the following:

	Years Ended December 31,	
	2019	2018
Bermuda	$ (168,053)	$ (105,562)
Foreign (U.S., U.K., Germany, France)	4,139	2,121
	$ (163,914)	$ (103,441)

(Amounts in thousands, except share and per share amounts)

The components of the Company's income tax benefit for the years ended December 31, 2019 and 2018 are as follows:

	Years Ended December 31,	
	2019	2018
Current income tax (provision):		
Bermuda	$ —	$ —
U.S. federal	(567)	(547)
U.S. state	(530)	(217)
Foreign	(12)	—
Total current income tax (provision)	(1,109)	(764)
Deferred income tax benefit:		
Bermuda	—	—
U.S. federal	2,397	542
U.S. state	759	436
Foreign	—	—
Total deferred income tax benefit	3,156	978
Total benefit (provision) for income taxes	$ 2,047	$ 214

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,	
	2019	2018
Bermuda statutory income tax rate	— %	— %
Foreign (U.S.) tax rate differential	(0.5)	(1.0)
Research and development tax credits	2.2	1.5
Share-based compensation	(0.5)	0.1
Permanent differences	(0.1)	—
Change in valuation allowance	(0.1)	—
U.S. state taxes, net of federal	(0.3)	(0.4)
FDII	0.9	—
Uncertain tax positions	(0.3)	—
Effective income tax rate	1.3 %	0.2 %

Net deferred tax assets consisted of the following:

	December 31,	
	2019	2018
Research and development tax credit carryforwards	$ 238	$ 75
Depreciation and amortization	(745)	(639)
Share-based compensation	3,746	1,000
Accrued expenses and other	1,273	829
Net operating losses	190	—
Total deferred tax assets	4,702	1,265
Valuation allowance	(330)	(49)
Net deferred tax assets	$ 4,372	$ 1,216

(Amounts in thousands, except share and per share amounts)

As of December 31, 2019 and 2018, the Company had federal research and development tax credit carryforwards of approximately $228 and $0 respectively, available to reduce future tax liabilities, which begin to expire in 2039. As of December 31, 2019 and 2018, the Company had state research and development tax credit carryforwards of approximately $265 and $95 respectively, available to reduce future tax liabilities, which begin to expire in 2034.

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 $330 and $49 has been established at December 31, 2019 and 2018, respectively.

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 shares of a corporation by more than 50% over a three-year period.

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2019 and 2018 were due primarily to an increase in state research and development tax credits and net operating losses from Primatope which are more likely than not to expire unutilized and were as follows:

	Years Ended December 31,	
	2019	2018
Valuation allowance at beginning of year	$ (49)	$ (27)
Increases recorded to through the balance sheet	(200)	—
Increases recorded to income tax provision	(81)	(22)
Valuation allowance at end of year	$ (330)	$ (49)

The valuation allowance increased by $281 in 2019 primarily as a result of additional California Research and Development (“R&D”) credits and the net operating loss carryovers at Primatope which may not be realized. 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.

The Company recognizes the tax benefit from an uncertain tax position only if it is more-likely-than-not that the tax position will be sustained on examination by taxing authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position are measured based on the largest benefit that has a greater than 50% likelihood of being realized upon ultimate settlement. The amount of unrecognized tax benefits is $528 and $0 as of December 31, 2019 and 2018, respectively. The net increase relates to new tax positions on Research and Development credits as a result of the R&D study performed in 2019.

A roll forward of the Company’s uncertainties in its income tax provision liability is presented below:

	Years Ended December 31,	
	2019	2018
Gross balance at the beginning of year	$ —	$ —
Gross increases based on current period tax positions	528	—
Unrecognized tax benefits at the end of the year	$ 528	$ —

(Amounts in thousands, except share and per share amounts)

The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision. The Company had recorded immaterial interest on the tax positions during the year ended December 31, 2019 and no interest or penalties for the year ended December 31, 2018.

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, 2016 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.

12. 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 8). 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,	
	2019	2018
Numerator:		
Net loss attributable to common shareholders	$ (161,867)	$ (103,227)
Denominator:		
Weighted average common shares outstanding—basic and diluted	54,049,477	29,547,427
Net loss per share attributable to common shareholders— basic and diluted	$ (2.99)	$ (3.49)

The Company's unvested restricted shares and restricted share units 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 unvested RSUs, 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 attributable to common shareholders for the periods indicated because including them would have had an anti-dilutive effect:

	As of December 31,	
	2019	2018
Options to purchase common shares	8,491,734	5,960,939
Unvested restricted shares	—	877,219
Unvested restricted share units	328,296	—
	8,820,030	6,838,158

13. Commitments and Contingencies

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 10).

Manufacturing Commitments

The Company entered into agreements with several contract manufacturing organizations to provide preclinical and clinical trial materials. As of December 31, 2019, the Company had committed to minimum payments under these agreements totaling $2,863 which are due during the year ending December 31, 2020.

Rilonacept Long-Term Incentive Plan

During the year ended December 31, 2019, the Company granted a cash award and the First RSU Award to employees under the RLTIP. The cash award vests upon the achievement of the RLTIP Milestone, subject to the recipient's continued employment. The First RSU Award becomes eligible to vest upon the achievement of the RLTIP Milestone, and will vest upon the first anniversary of such date, subject to the recipient's continued employment through that date. As of December 31, 2019, the Company estimates cash payments of $2,083 under the RLTIP. In the event the RLTIP Milestone is not achieved, the cash award will not be paid and the First RSU Award will not vest.

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, officers and other key personnel 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, officers or other key personnel. 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, 2019 or 2018.

Legal Proceedings

The Company is not a party to any litigation and does not have contingency reserves established for any litigation liabilities.

14. 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, 2019 and 2018, the Company contributed $851 and $315, respectively, to the plan.

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MANAGEMENT TEAM

Sanj K. Patel*
Chief Executive Officer & Chairman of the Board

John F. Paolini, MD, PhD*
Senior Vice President, Chief Medical Officer

Thomas Beetham*
Executive Vice President, Corporate Development & Operations, Chief Legal Officer

Qasim Rizvi*
Senior Vice President, Operations & Chief Commercial Officer

Eben Tessari
Senior Vice President, Chief Business Officer

Dave Nichols
Senior Vice President, Technical Operations

Dana Martin
Senior Vice President, Global Medical Affairs

Martina Struck, PhD
Vice President, Regulatory Affairs

Mike Megna
Vice President, Finance & Chief Accounting Officer

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, MD
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
Former Chief Executive Officer & Chairman of the Board, 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

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, 2019 (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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