Akebia Therapeutics, Inc.
Form 10-K
March 26, 2019

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2018

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
Commission File Number 001-36352

AKEBIA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization)	20-8756903 (I.R.S. Employer Identification No.)
245 First Street, Cambridge, MA (Address of principal executive offices)	02142 (Zip Code)

Registrant’s telephone number, including area code: (617) 871-2098

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

Title of each class	Name of each exchange on which registered
Common Stock, par value $0.00001 per share	The Nasdaq Global Market

Securities registered pursuant to Section 12(g) of the Act: None

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

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

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). YES NO

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

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

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

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

Indicate by check mark whether 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, based on the closing price of the registrant's Common Stock on The Nasdaq Global Market on June 30, 2018, was $534,415,547.

The number of shares of registrant's Common Stock outstanding as of March 15, 2019 was 117,122,262.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Proxy Statement for the registrant's 2019 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K.

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NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements that are being made pursuant to the provisions of the U.S. Private Securities Litigation Reform Act of 1995 with the intention of obtaining the benefits of the "safe harbor" provisions of that Act. These forward-looking statements may be accompanied by words such as "anticipate," "believe," "build," "can," "contemplate," "continue," "could," "should," "designed," "estimate," "project," "expect," "forecast," "intend," "likely," "may," "plan," "possible," "potential," "predict," "strategy," "seek," "target," "will," "would," and other wo similar meaning. These forward-looking statements include, but are not limited to, statements about:

- our expectations with respect to (i) the anticipated financial impact and potential benefits to us related to our merger with Keryx Biopharmaceuticals, Inc., or Keryx, that was completed on December 12, 2018, or the Merger, (ii) integration of the businesses subsequent to the Merger, and (iii) other matters related to the Merger;
- the potential therapeutic applications of the hypoxia inducible factor, or HIF, pathway;
- our pipeline, including its potential, and our research activities;
- the potential therapeutic benefits, safety profile, and effectiveness of our product candidates, including the potential for vadadustat to set a new standard of care in the treatment of anemia due to chronic kidney disease;
- the potential indications and market potential and acceptance of our product and product candidates, including our estimates regarding the potential market opportunity for Auryxia, vadadustat or any other product candidates and the size of eligible patient populations;
- our competitive position, including estimates, developments and projections relating to our competitors and their products and product candidates, and our industry;
- our expectations, projections and estimates regarding our costs, expenses, revenues, capital requirements, need for additional capital, financing our future cash needs, capital resources, cash flows, financial performance, profitability, tax obligations, liquidity, growth, contractual obligations, the period of time our cash resources and collaboration funding will fund our current operating plan, internal control over financial reporting, and disclosure controls and procedures;
- the timing of the availability and disclosure of clinical trial data and results;
- our and our collaborators' strategy, plans and expectations with respect to the development, manufacturing, commercialization, launch, marketing and sale of our product candidates, and the associated timing thereof;
- the designs of our studies, and the type of information and data expected from our studies and the expected benefits thereof;
- the timing of or likelihood of regulatory filings and approvals, including labeling or other restrictions;
- our ability to maintain any marketing authorizations we currently hold or will obtain, including our marketing authorizations for Auryxia and our ability to complete post-marketing requirements with respect thereto;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for Auryxia or any other product candidate that may be approved;
- the targeted timing of enrollment of our clinical trials;
- the timing of initiation of our clinical trials and plans to conduct preclinical and clinical studies in the future;
- the timing and amounts of payments from or to our collaborators and licensees, and the anticipated arrangements and benefits under our collaboration and license agreements, including with respect to milestones and royalties;
- our intellectual property position, including obtaining and maintaining patents; and the timing, outcome and impact of administrative, regulatory, legal and other proceedings relating to our patents and other proprietary and intellectual property rights;
- expected reliance on third parties, including with respect to the development, manufacturing, supply and commercialization of our product and product candidates;
- accounting standards and estimates, their impact, and their expected timing of completion;

- estimated periods of performance of key contracts;
- our facilities, lease commitments, and future availability of facilities;
- cybersecurity;
- insurance coverage;
- our employees, including our management team, employee compensation, employee relations, and our ability to attract and retain high quality employees;
- the implementation of our business model, current operating plan, and strategic plans for our business, product candidates and technology, and business development opportunities including potential collaborations, alliances, mergers, acquisitions or licensing of assets;
- the timing, outcome and impact of current and any future legal proceedings.

These forward-looking statements involve risks and uncertainties, including those that are described in Part I, Item 1A. Risk Factors included in this Annual Report and elsewhere in this Annual Report on Form 10-K, that could cause our actual results, financial condition, performance or achievements to be materially different from those indicated in these forward-looking statements. Given these risks and uncertainties, you should not place undue reliance on these forward-looking statements. Forward-looking statements speak only as of the date of this Annual Report on Form 10-K. Except as required by law, we assume no obligation to publicly update or revise these forward-looking statements for any reason. Unless otherwise stated, our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

This Annual Report on Form 10-K also contains estimates and other information concerning our industry and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Unless otherwise expressly stated, we obtained this industry, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

In this Annual Report on Form 10-K, unless otherwise stated or the context otherwise requires, references to “Akebia,” “we,” “us,” “our,” “the Company,” and similar references refer to Akebia Therapeutics, Inc. and, where appropriate, its subsidiaries, including Keryx. The trademarks, trade names and service marks appearing in this Annual Report on Form 10-K are the property of their respective owners. All website addresses given in this Annual Report on Form 10-K are for information only and are not intended to be an active link or to incorporate any website information into this document.

PART I

Item 1. Business

Overview

We are a biopharmaceutical company focused on the development and commercialization of therapeutics for patients with kidney disease. On December 12, 2018, we completed a merger, or the Merger, with Keryx Biopharmaceuticals, Inc., or Keryx, combining a nephrology-focused commercial organization with our robust development organization. Following the Merger, Keryx is our wholly owned subsidiary, and we are integrating our business and Keryx's business with the goal of positioning Akebia to realize the potential growth opportunities and synergies from the Merger.

We now have a commercial product and a late-stage product candidate:

- Auryxia® (ferric citrate) is approved and marketed in the United States for two indications: (1) the control of serum phosphorus levels in adult patients with chronic kidney disease, or CKD, on dialysis, or DD-CKD, or the Hyperphosphatemia Indication, and (2) the treatment of iron deficiency anemia, or IDA, in adult patients with CKD not on dialysis, or NDD-CKD, or the IDA Indication. Ferric citrate is also approved and marketed in Japan as an oral treatment for the improvement of hyperphosphatemia in patients with CKD, including DD-CKD and NDD-CKD, under the trade name Riona® (ferric citrate hydrate) and approved in the European Union, or the EU, for the control of hyperphosphatemia in adult patients with CKD under the trade name Fexeric® (ferric citrate).
- Vadadustat is an investigational, oral hypoxia-inducible factor prolyl hydroxylase inhibitor, or HIF-PHI, in global Phase 3 development for two indications: (1) anemia due to CKD in adult patients with DD-CKD, and (2) anemia due to CKD in adult patients with NDD-CKD. We believe vadadustat has the potential to set a new oral standard of care for patients with anemia due to CKD, subject to regulatory approval. Vadadustat's proposed mechanism of action is designed to mimic the physiologic effect of altitude on oxygen availability. At higher altitudes, the body responds to lower oxygen availability with increased production of hypoxia-inducible factor, or HIF, which coordinates the interdependent processes of iron mobilization and stimulates endogenous production of erythropoietin, or EPO, to increase red blood cell, or RBC, production and, ultimately, improve oxygen delivery.

We market Auryxia in the United States with our well-established, nephrology-focused commercial organization. Our Japanese sublicensee, Japan Tobacco, Inc., or JT, and its subsidiary, Torii Pharmaceutical Co., Ltd., or Torii, commercialize Riona in Japan. Fexeric is not currently marketed in the EU.

We plan to commercialize vadadustat, subject to U.S. Food and Drug Administration, or FDA, approval, in the United States with our commercial organization, while also leveraging our collaboration with Otsuka Pharmaceutical Co. Ltd., or Otsuka, and its U.S. commercial organization. We also granted Otsuka exclusive rights to commercialize vadadustat in Europe, China and certain other markets, subject to marketing approvals. In Japan and certain other countries in Asia, we granted Mitsubishi Tanabe Pharma Corporation, or MTPC, exclusive rights to commercialize vadadustat, subject to marketing approvals. In addition, we granted Vifor (International) Ltd., or Vifor Pharma, an exclusive license to sell vadadustat solely to Fresenius Kidney Care Group LLC, or FKC, which manages approximately 40% of the dialysis patients in the United States, at its U.S. dialysis clinics, subject to FDA approval of vadadustat, vadadustat's reimbursement under a bundled reimbursement model, and a milestone payment by Vifor Pharma.

Strategy

Our goal is to become a leading biopharmaceutical company focused on the treatment of patients with kidney disease, through the discovery, development and commercialization of innovative therapeutics. The key elements of our strategy are as follows:

- Maximize commercial opportunity for Auryxia. We aim to gain market share in Auryxia's Hyperphosphatemia Indication by leveraging Auryxia's product profile and opportunities for adoption following the release of updated clinical guidelines. We aim to gain market share and grow the market for Auryxia's IDA Indication by offering an alternative to the existing treatment approach.
- Complete global development and commercialization of our late-stage product candidate, vadadustat. We believe vadadustat has the potential to address limitations of injectable erythropoiesis-stimulating agents, or ESAs, and set a new standard of care for the treatment of anemia due to CKD, subject to regulatory approval. We are conducting a global Phase 3 clinical development program for vadadustat, and our collaboration partner, MTPC, is conducting a Phase 3 clinical development program for vadadustat in Japan. We believe we are well positioned to commercialize vadadustat in the United States in partnership with Otsuka and through our agreement with Vifor Pharma, subject to FDA approval. We plan to support Otsuka's and MTPC's commercialization of vadadustat in Europe, China and certain other markets, subject to regulatory approvals. We retained full commercial rights to vadadustat in Latin America, allowing us maximum flexibility in the region.

- Leverage portfolio synergies between our product, Auryxia, and our product candidate, vadadustat, in CKD. We believe there is an opportunity to maximize the U.S. commercial performance of Auryxia and vadadustat, subject to FDA approval and launch, by leveraging our nephrology-focused commercial organization for Auryxia and our relationships and expertise in the renal space. We also plan to explore co-development potential for Auryxia and vadadustat.
- Expand our pipeline and portfolio of renal therapeutics to advance care for patients with kidney disease. We aim to add to our pipeline and portfolio of renal therapeutics through internal discovery and development, and through strategic transactions, such as in-licenses, collaborations and acquisitions. Our pipeline and portfolio expansion efforts will be guided by our vision to improve the health of patients with kidney disease through better disease management and novel therapeutics.

Our management team has extensive experience in developing and commercializing drugs for the treatment of renal and metabolic disorders, a deep understanding of the renal space and biological pathways involved in kidney disease including HIF biology, and broad business development expertise. With this management team, fully integrated capabilities spanning research, manufacturing, development and commercialization, a growing revenue stream and a strong balance sheet, we are well positioned to execute on our strategy.

Kidney Disease

Kidney disease is an area of major unmet need globally, driving massive healthcare costs and with a generally poor prognosis: eventually many patients will progress to a stage where they are dependent on dialysis, with high morbidity and a significant increase in mortality rate.

Kidney disease can be caused by a number of distinct and concomitant factors, including cardiometabolic disorders (primarily diabetes and hypertension), genetic kidney diseases, autoimmune disorders, and aging. Given the prevalence and growth rates of these various underlying conditions, kidney disease prevalence is expected to continue to increase globally. In the United States, kidney disease significantly impacts the U.S. healthcare system, affecting more than 40 million patients and costing Medicare over $110 billion annually in 2016 for the care provided in dialysis clinics, nephrology centers and hospitals. The U.S. Department of Health and Human Services has recognized the national pandemic and partnered with the American Society of Nephrology to found the KidneyX Innovation Accelerator, a public-private partnership to improve the lives of the 850 million people worldwide currently affected by kidney diseases by accelerating innovation in the prevention, diagnosis and treatment of kidney diseases.

Most of the conditions covered by the term “kidney disease” may ultimately lead to dependence on dialysis or kidney transplant for survival, causing renal failure, directly or indirectly, by accelerating the onset of CKD. Dependence on dialysis is associated with a significant increase in mortality and hospitalizations, and a significant reduction in quality of life for patients. It is our vision, in time, to provide or contribute to better alternatives for patients with kidney disease.

As a first step towards our vision, we aim to advance care for patients with CKD, which is the current focus of our pipeline and our FDA-approved product, Auryxia.

CKD is a condition in which the kidneys are progressively damaged to the point that they cannot properly filter the blood circulating in the body. This damage causes waste products to build up in the patient’s blood leading to other health problems, including anemia, cardiovascular disease and bone disease. As illustrated in the table below, CKD patients are categorized in one of five stages based on the degree of their loss of kidney function as measured by the glomerular filtration rate, or GFR, and the level of protein in the urine, referred to as albuminuria. CKD is estimated to affect approximately 37 million adults in the United States.

Stages and Prevalence of Chronic Kidney Disease in the United States

Stage	Description