REGENXBIO Inc.
Form 10-K
March 06, 2018
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2017

or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to

Commission file number: 001-37553

REGENXBIO Inc.

(Exact name of registrant as specified in its charter)

Delaware	47-1851754
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)

9600 Blackwell Road, Suite 210

Rockville, MD 20850

(Address of principal executive offices) (Zip Code)

(240) 552-8181

(Registrant's telephone number, including area code)

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

Common Stock, $0.0001 par value per share	The Nasdaq Stock Market LLC
(Title of each class)	(Name of each exchange on which registered)

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

None

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

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

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) 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 (Do not check if a smaller reporting company) | Smaller reporting company

Emerging growth company

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

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

As of June 30, 2017, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's common stock held by non-affiliates was approximately $505.0 million, based on the closing price of the registrant's common stock on The Nasdaq Global Select Market on June 30, 2017 of $19.75 per share. For purposes of this disclosure, shares of common stock held by each executive officer, director and stockholder known by the registrant to be affiliated with such individuals based on public filings and other information known to the registrant have been excluded since such persons may be deemed affiliates. This determination of executive officer or affiliate status is not necessarily a conclusive determination for other purposes.

As of March 2, 2018, there were 31,655,093 shares of the registrant's common stock, par value $0.0001 per share, issued and outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Specified portions of the registrant's proxy statement with respect to the registrant's 2018 Annual Meeting of Stockholders, which is to be filed pursuant to Regulation 14A within 120 days after the end of the registrant's fiscal year ended December 31, 2017, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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PART I

INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations o by similar expressions. We have based these forward-looking statements on our current expectations and assumptions and analyses made by us in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- the timing of enrollment, commencement and completion of our clinical trials;
- the timing and success of preclinical studies and clinical trials conducted by us and our development partners;
- the timely development and launch of new products;
- the ability to obtain and maintain regulatory approval of our product candidates, and the labeling for any approved products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain and maintain intellectual property protection for our product candidates and technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our product candidates;
- our ability to establish and maintain development partnerships;
- our expectations regarding our expenses and revenue;
- our expectations regarding regulatory developments in the United States and foreign countries; and
- the use or sufficiency of our cash and cash equivalents and needs for additional financing.

You should carefully read the factors discussed in the sections titled "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this Annual Report on Form 10-K and in our other filings with the U.S. Securities and Exchange Commission (SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this report. Except as required by law and the rules of the SEC, we do not undertake any obligation, and specifically decline any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

As used in this Annual Report on Form 10-K, the terms "REGENXBIO," "we," "us," "our" or the "Company" mean REGENXBIO Inc. and its subsidiaries, on a consolidated basis, unless the context indicates otherwise.

NAV, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Annual Report on Form 10-K are the property of their respective holders.

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INDUSTRY AND MARKET DATA

We obtained the industry, market and competitive position data used throughout this Annual Report on Form 10-K from our own internal estimates and research, as well as from industry and general publications, in addition to research, surveys and studies conducted by third parties. Internal estimates are derived from publicly-available information released by industry analysts and third-party sources, our internal research and our industry experience, and are based on assumptions made by us based on such data and our knowledge of our industry and market, which we believe to be reasonable. We have not independently verified industry, market and competitive position data from third-party sources, but we believe the sources of such information to be reliable. While we believe the industry, market and competitive position data included in this Annual Report on Form 10-K is reliable and is based on reasonable assumptions, such data involves risks and uncertainties and are subject to change based on various factors, including those discussed in “Risk Factors.” These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

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ITEM 1. BUSINESS

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our gene therapy product candidates are designed to deliver genes to cells to address genetic defects or to enable cells in the body to produce therapeutic proteins or antibodies that are intended to impact disease. Through a single administration, our gene therapy product candidates are designed to provide long-lasting effects, potentially significantly altering the course of disease and delivering improved patient outcomes.

Our product candidate RGX-314 is for the treatment of wet age-related macular degeneration (wet AMD), a leading cause of total and partial vision loss in the United States, Europe and Japan. We began enrollment in the Phase I clinical trial for RGX-314 for the treatment of wet AMD in May 2017 and have completed dosing of three cohorts of six patients each, a total of 18 patients, in the Phase I clinical trial. We expect to present topline data from the Phase I clinical trial in late 2018.

Our product candidate RGX-501 is for the treatment of homozygous familial hypercholesterolemia (HoFH), a severe genetic disease characterized by premature and aggressive plaque buildup, life threatening coronary artery disease (CAD) and aortic valve disease predominantly due to abnormalities in the function or expression of the low-density lipoprotein receptor (LDLR) gene. We, together with trial sponsor the University of Pennsylvania (Penn), began enrollment in the Phase I/II clinical trial for RGX-501 in March 2017. We have completed dosing of the first cohort of three patients and have dosed two patients in the second cohort. We expect to present topline data from the Phase I/II clinical trial in late 2018.

We are also developing product candidates to address the neurological symptoms of two severe genetic lysosomal storage diseases, Mucopolysaccharidosis Type I (MPS I) and Mucopolysaccharidosis Type II (MPS II). MPS I is caused by deficiency of -l-iduronidase (IDUA) and MPS II is caused by deficiency of iduronate-2-sulfatase (IDS), both of which are enzymes that are responsible for breakdown of cellular waste products. Deficiencies in these enzymes lead to a number of physical symptoms and patients with severe forms of these diseases also exhibit significant cognitive decline. The investigational new drug applications (INDs) filed with the U.S. Food and Drug Administration (the FDA) for RGX-111 and RGX-121, our product candidates for MPS I and MPS II, respectively, are active. We expect to begin enrollment in a Phase I clinical trial for RGX-111 and a Phase I/II clinical trial for RGX-121 in mid-2018.

In addition to our lead product candidates, we have also funded, and plan to continue to fund, preclinical research on potential product candidate programs that may become part of our internal product development pipeline. We have partnered with a number of leading academic institutions and will continue to seek partnerships with innovative institutions to develop novel NAV gene therapy product candidates. We expect to announce an additional lead product candidate in the second half of 2018.

Our gene therapy product candidates deliver genes to cells using adeno-associated virus (AAV) vectors, which are non-replicating viral delivery vehicles that are not known to cause disease. Our product candidates all utilize viral vectors from our proprietary gene delivery platform, which we call our NAV Technology Platform. Our NAV Technology Platform consists of exclusive rights to AAV7, AAV8, AAV9, AAVrh10 and over 100 other novel AAV vectors (NAV Vectors). We currently have exclusive rights to over 100 patents and patent applications worldwide covering our NAV Vectors, including composition of matter claims for AAV7, AAV8, AAV9 and AAVrh10, as well as methods for their manufacture and therapeutic uses. We believe this patent portfolio forms a strong foundation for our current programs and with our ongoing research and development, we expect to continue to expand this robust

patent portfolio.

The foundation of our NAV Technology Platform was discovered in an effort to identify next generation AAV vectors that could overcome the limitations of earlier generation AAV vectors (AAV1 through AAV6). We believe the key benefits of NAV Vectors over earlier generation AAV vectors include:

- higher gene expression;
- longer-term gene expression;
- broad and novel tissue selectivity;
- lower immune response; and
- improved manufacturability.

In addition to our internal product development efforts, we also selectively sublicense our NAV Vectors to other biotechnology companies, which we refer to as NAV Technology Licensees. As of December 31, 2017, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by 10 NAV Technology Licensees.

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Our internal and partnered product development program pipeline is shown below.