REGENXBIO Inc.
Form 10-K
March 03, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

x ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2015

or

o 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)

9712 Medical Center Drive, Suite 100

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 o No x

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 o No x

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 x No o

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 x No o

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. o

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

Large accelerated filer	o	Accelerated filer	o

Non-accelerated filer x (Do not check if a smaller reporting company) Smaller reporting company o
Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes o
No x

As of June 30, 2015, the last business day of the registrant's most recently completed second fiscal quarter, the registrant's common stock was not listed on any exchange or over-the-counter market. The registrant's common stock began trading on The NASDAQ Global Select Market on September 17, 2015. As of December 31, 2015, the aggregate market value of shares of common stock held by non-affiliates of the registrant was $331.8 million based on the number of shares held by non-affiliates as of December 31, 2015 and based on the last reported sale price of the registrant's common stock on December 31, 2015. 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 1, 2016 there were 26,328,584 shares of the registrant's Common Stock issued and outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Specified portions of the registrant's proxy statement with respect to the registrant's 2016 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, 2015, are incorporated by reference into Part III of this Annual Report on Form 10-K.

REGENXBIO INC.

Form 10-K

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

PART I

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains "forward-looking statements" that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Annual Report on Form 10-K, including statements regarding our future results of operations and financial position, strategy and plans, and our expectations for future operations, are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek the negative version of these words and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, strategy, short- and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties, assumptions and other important factors, including those described in the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in this Annual Report on Form 10-K. In light of these risks, uncertainties, assumptions and other factors, the forward-looking events and circumstances discussed in this Annual Report on Form 10-K may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

Forward-looking statements include, but are not limited to, statements about:

- the timing and success of preclinical studies and clinical trials conducted by us and our development partners;
- 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;
- 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 federal, state and foreign regulatory requirements;
- regulatory developments in the United States and foreign countries; and
- our plans for the use of our cash and cash equivalents.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, level of activity, performance or achievements. Any forward-looking statement made by us in this Annual Report on Form 10-K speaks only as of the date of this report. Except as required by law, we disclaim any duty to update any of these forward-looking statements after the date of such statements are made, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

We encourage you to read the discussion and analysis of our financial condition and our financial statements contained in this Annual Report on Form 10-K. We also encourage you to read Item 1A of Part I of this Annual Report on Form 10-K, entitled "Risk Factors," which contains a more complete discussion of the risks and uncertainties associated with our business. In addition to the risks described above and in Item 1A of Part I of this report, other

unknown or unpredictable factors also could affect our results. There can be no assurance that the actual results or developments anticipated by us will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, us. Therefore no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

As used in this Annual Report on Form 10-K, the terms “REGENXBIO,” “Registrant,” “we,” “us,” and “our” mean REGENXBIO Inc. unless the context indicates otherwise.

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. In addition, 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.

ITEM 1. BUSINESS

Overview

We are a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. In AAV gene therapy, the viral genes are removed from the AAV, a small, non-pathogenic virus, creating a biological delivery vehicle called a vector. A therapeutic gene sequence is then inserted, creating a recombinant vector. Our proprietary AAV gene delivery platform (our NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10 (NAV Vectors). Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing gene therapy products administered directly into the body, or in vivo, based on our NAV Technology Platform. We seek to accomplish our mission through a combination of our internal development efforts and the efforts of our third-party licensees (NAV Technology Licensees). As of December 31, 2015, our NAV Technology Platform was being applied in the development of 28 product candidates for a variety of diseases, including five internally developed product candidates and 23 partnered product candidates developed by our NAV Technology Licensees. Most of our NAV Technology Licensees have licensed specific NAV Vectors for the indications they are pursuing. We maintain rights to all unlicensed indications as well as retaining the right to our NAV Technology Platform for unlicensed vectors in disease indications for which we have granted licenses.

We are applying our NAV Technology Platform in an effort to generate a broad pipeline of best-in-class and often first-in-class AAV gene therapy treatments. Our NAV Technology Platform is covered by more than 100 licensed patents and patent applications worldwide. Our product candidates, which are designed for a variety of diseases, incorporate proprietary advances in AAV gene therapy that significantly enhance their profiles as potential therapeutics. The benefits of our NAV Technology Platform have been observed across several clinical trials and studies conducted by our development partners and third-party investigators. Approximately 70% of all AAV gene therapy clinical trials relating to new treatment Investigational New Drug applications (INDs) posted on the United States (U.S.) government clinical trials database from 2012 through 2014 used NAV Vectors.

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.

We believe that gene therapies using our NAV Technology Platform (NAV Gene Therapy) have the potential to transform the treatment paradigm for patients with a wide range of severe diseases with significant unmet medical needs. NAV Vectors have demonstrated stable expression in animals for over eight years. Moreover, AAV8 vectors have demonstrated stable expression for over four years in a clinical trial for the treatment of hemophilia B.

In certain monogenic, recessive diseases, NAV Gene Therapy may provide clinical benefits for patients that are substantially greater than currently available therapies. In other types of diseases, such as hemophilia, NAV Gene Therapy has the potential to replace a lifetime of continuous treatment of standard protein replacement therapy and other treatment approaches with a single treatment, which could reduce health care system costs while also improving patients' quality of life. We believe that the potential efficiency and broad applicability of our NAV Technology Platform may allow us to develop NAV Gene Therapy treatments that are injected or infused into the bloodstream, spinal fluid or directly into the target tissue to treat a wide range of diseases.

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

We currently plan to build internal gene therapy franchises in the metabolic, neurodegenerative and retinal therapeutic areas, and develop multiple product candidates in each area. Our most advanced programs are for the treatment of two severe genetic diseases, homozygous familial hypercholesterolemia (HoFH) and Mucopolysaccharidosis Type I (MPS I). An IND to support a Phase

I/II clinical trial to evaluate the effect of RGX-501 for the treatment of HoFH is active. We expect a Phase I/II clinical trial for RGX-501 to be initiated in the first half of 2016. We expect to file an IND with the U.S. Food and Drug Administration (the FDA) for RGX-111, our program for MPS I, in the first half of 2016 to support a Phase I/II clinical trial, which we expect to initiate in mid-2016. We also have a preclinical program for wet age-related macular degeneration (wet AMD) for which we expect to file an IND with the FDA in the second half of 2016 and a preclinical program for Mucopolysaccharidosis Type II (MPS II) for which we expect to file an IND with the FDA in the first half of 2017.

Our partnered development pipeline benefits from the disease-specific expertise of our NAV Technology Licensees. Our partnering strategy provides us the flexibility to sublicense development of treatments designed to address significant unmet medical needs, while remaining focused on our core programs and therapeutic areas internally, which we believe enables us to achieve maximum value. We believe that the broad applicability of our NAV Technology Platform and any clinical successes of the treatments utilizing NAV Vectors will create new internal and partnered pipeline opportunities.

As an innovator in AAV gene therapy development, our intellectual property strategy is designed to provide us with extensive protection for our product candidates and our NAV Technology Platform. 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 substantial patent portfolio. Our licensed patents not only seek to protect our key assets - our NAV Technology Platform and our internal product candidates - they also form the basis for licensing and partnering arrangements.

Our company was formed from a successful collaboration that began in February 2009 between FoxKiser LLP, the University of Pennsylvania (together with The Trustees of the University of Pennsylvania, Penn) and gene therapy pioneer James Wilson, M.D., Ph.D. We have built on the foundation of this collaboration to produce what we believe to be compelling NAV Gene Therapy product candidates derived from discoveries and research in Dr. Wilson’s lab. As our team has grown, we have continued to build on our scientific foundation, adding depth in gene therapy and biotechnology leadership. Our management team includes leaders who are experienced in building and operating innovative healthcare ventures and have expert knowledge in the development of AAV gene therapy. We believe the strength of our team coupled with the depth of knowledge of our scientific founder and advisors position us to succeed in developing and bringing to market, independently or with our development partners, unique, best-in-class gene therapy treatments for a range of severe diseases with significant unmet medical needs.

Our Strategy

Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing in vivo gene therapy products based on our NAV Technology Platform. We are seeking to develop, manufacture, commercialize and license product candidates across multiple therapeutic areas while continuing to expand our NAV Technology Platform. To achieve our mission, we are pursuing the following strategies:

- Apply our proprietary, next generation AAV vector technology to develop in vivo gene therapies for patients. We believe in vivo gene therapy is an ideal treatment paradigm for many diseases with sub-optimal or non-existent therapies because of its potential to correct an underlying genetic defect, rather than just treating a patient’s symptoms. We believe our NAV Technology Platform will prove to be a significant advancement over earlier AAV vectors. Based on data derived from third-party clinical studies using our NAV Vectors, we believe our NAV Technology Platform possesses unique, beneficial properties that are not seen in earlier generation AAVs. We

believe that our NAV Technology Platform, which underpins our internal development programs and the programs of our NAV Technology Licensees, will enable us and our partners to develop best-in-class gene therapy candidates for a wide range of disease targets due to these unique properties.

- Focus on rapidly advancing our internal lead proprietary development programs in metabolic, neurodegenerative and retinal diseases. Both HoFH and MPS I are diseases with high unmet clinical need and current treatments that are sub-optimal or non-existent. An IND to support a Phase I/II clinical trial to evaluate the effect of RGX-501 for the treatment of HoFH is active. We expect a Phase I/II clinical trial for RGX-501 to be initiated in the first half of 2016. We expect to file an IND for MPS I in the first half of 2016 and expect to initiate a Phase I/II clinical trial for MPS I in mid-2016. If we are successful in achieving proof-of-concept in the Phase I/II clinical trials for these diseases, we will pursue registration trials and commercialization of such product candidates. In addition, we plan to progress our product development programs for wet AMD and MPS II toward clinical trials and expect to file INDs for these programs in the second half of 2016 and the first half of 2017, respectively.

- Establish gene therapy franchises in our current core therapeutic areas of metabolic, neurodegenerative and retinal diseases. After human proof-of-concept is achieved in a disease, we believe we will be able to apply what we have learned and use our NAV Technology Platform to more rapidly develop new product candidates for many similar diseases. Once an appropriate vector and route of administration for a particular disease type have been established, a new gene can be inserted into the appropriate vector and the established route of administration can be used for other similar diseases. We expect to use this approach to further build the foundation for our neurodegenerative disease franchise by filing an IND in the first half of 2017 for our MPS II program and moving to clinical trials thereafter if we are able to demonstrate human proof-of-concept in MPS I. We believe that this approach is also applicable to metabolic and retinal diseases, as well as many other therapeutic areas, and will allow us to efficiently generate product candidates for diseases in and beyond our current areas of therapeutic focus.
- Further grow the pipeline of products based on our NAV Technology Platform through strategic in-licensing and sublicensing of new programs. We also plan to grow the pipeline of commercial product development programs using our NAV Technology Platform through licensing. For example, we plan to pursue in-licensing for programs we deem to be the most promising research programs using our NAV Vectors. We intend to continue to selectively sublicense our NAV Technology Platform for specific vector and indication combinations to additional NAV Technology Licensees. Strategic sublicensing allows us to maintain our internal product development focus in our core disease indications and therapeutic areas while still expanding the NAV Gene Therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform, and creating potential additional revenue.
- Maintain and grow our extensive intellectual property portfolio. We plan to leverage our intellectual property rights and substantial expertise in AAV gene therapy in order to develop and commercialize NAV Gene Therapy treatments. We have licensed exclusive rights to a broad portfolio of certain fundamental AAV gene therapy patents and patent applications. In securing these rights, we have focused on obtaining robust rights for those intellectual property assets we believe will be most important in providing us with a competitive advantage with respect to AAV gene therapy treatments. We plan to continue to seek to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our business.

Our Strengths

We believe our technology, expertise and know-how will allow us to maintain our leadership position in the gene therapy field. Our strengths include the following: