



## REGENXBIO Reports Continued Progress Across Programs in Year-End 2018 Corporate Update

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- Reports further positive interim update from RGX-314 Phase I trial for wet AMD:
  - 50% of subjects treated in Cohort 3 continue to remain free of anti-VEGF injections at nine months
  - Mean RGX-314 intraocular protein expression in recently dosed Cohort 4 was higher than in previously reported Cohort 3 at one month
  - FDA clearance to expand RGX-314 Phase I protocol immediately into a Phase IIa clinical trial received, enabling further characterization of treated subjects in a larger sample; on track to initiate Phase IIb trial in late 2019
  - Plan to expand RGX-314 into additional retinal conditions, with the first such IND submission anticipated in the second half of 2019
  - Confirms analyst and investor event with internationally recognized retina specialists in February 2019
- RGX-121 well-tolerated in first patient dosed at initial eight-week safety assessment; additional recruitment and site activation continues
- Interim trial updates for RGX-121, RGX-111 and RGX-501 anticipated in the second half of 2019
- First anticipated FDA regulatory action for a proprietary NAV<sup>®</sup> Technology-based treatment, Novartis' ZOLGENSMA<sup>®</sup> for the treatment of SMA Type I, expected in May 2019
- More than \$470 million in cash, cash equivalents and marketable securities as of December 31, 2018
- Expects to end 2019 with over \$330 million in cash, cash equivalents and marketable securities, excluding any projected commercial revenue from ZOLGENSMA

REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV Technology Platform, today provided a year-end 2018 corporate update.

"2018 was a pivotal year for the advancement and validation of the NAV Technology Platform across many diseases, as we achieved significant clinical and regulatory progress for our proprietary product candidates, as well as across our licensee network. In 2018, we received over \$200 million from our partners, and we believe our strong financial position will enable us to execute on our corporate goals throughout 2019," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "This year, a key focus will be the acceleration and broadening of our ophthalmology franchise as we build on the promising results observed to date in our ongoing Phase I clinical trial evaluating RGX-314 for wet age-related macular degeneration (wet AMD). Through the immediate expansion of this trial into a Phase IIa clinical trial, we're committed to expediting the development of RGX-314 as a one-time therapy, not only in the hopes of providing a potential long-lasting treatment solution for the wet AMD patient population, but also to potentially address substantial unmet needs in a broad range of other retinal conditions."

Mr. Mills continued: "Another key upcoming milestone for the NAV Technology Platform is the potential approval of Novartis' ZOLGENSMA by the FDA for the treatment of SMA Type I, anticipated in May 2019. This approval would be the first for a NAV Technology-based therapy, and it would provide a post-launch commercial revenue stream to REGENXBIO. In combination with our innovative technology platform, robust internal clinical pipeline and broad licensee network, we are positioned for a transformative 2019 as we accelerate our progress in realizing the curative potential of gene therapy."

### Lead Product Candidate Updates

- RGX-314 for the Treatment of Wet AMD

- As of December 3, 2018, 24 subjects across four dose cohorts have been treated in the Phase I trial of RGX-314. RGX-314 continues to be well-tolerated across all cohorts, with no drug-related serious adverse events (SAEs) reported. In addition, 50% of subjects (3/6) in Cohort 3 continue to remain injection-free at nine months with persistent clinical durability of effect observed on best corrected visual acuity (BCVA) and central retinal thickness (CRT). Mean BCVA improved by +13 letters and mean CRT decreased by -37 microns from baseline in these subjects at nine months.
- As of December 3, 2018, the most common adverse events (AEs) in all dose cohorts were assessed as mild (Grade 1, 83%), and there have been no observed immune responses, drug-related ocular inflammation or post-surgical inflammation beyond what is expected following a routine vitrectomy. Six non-drug-related SAEs were reported among four subjects.<sup>1</sup>
- RGX-314 intraocular protein expression was detected at one month in all subjects in the recently dosed Cohort 4 ( $1.6 \times 10^{11}$  GC/eye). Mean protein expression levels are higher in Cohort 4 than the previously reported Cohort 3 at one month post-RGX-314 administration as measured from aqueous samples by electrochemiluminescence immunoassay (ECL).
- Phase I Trial Expanded into Phase IIa Trial:
  - REGENXBIO today announced that, based on an amendment to the RGX-314 Phase I protocol filed with the U.S. Food and Drug Administration (FDA), it is cleared to proceed immediately to a Phase IIa trial under the current Investigational New Drug (IND) application. With the expansion of the Phase I protocol of RGX-314 for wet AMD to a Phase IIa trial, REGENXBIO now expects to enroll up to a total of 42 subjects. The expanded Phase IIa study, which will include an additional six subjects in Cohort 4 and the initiation of dosing of an additional cohort (12 participants) at a dose of  $2.5 \times 10^{11}$  GC/eye (Cohort 5), has commenced recruitment. This expansion is designed to further characterize RGX-314-treated subjects in a larger sample in order to enhance the design of the Phase IIb trial and accelerate the clinical development of RGX-314.
  - Based on this expanded trial design, REGENXBIO expects to present interim data from the RGX-314 Phase I/IIa clinical trial by the end of 2019.
- Phase IIb Trial Plans:
  - REGENXBIO anticipates initiating a larger, randomized, controlled Phase IIb clinical trial for wet AMD and expects to activate clinical sites in this program in late 2019.
- Extension of RGX-314 Development into Additional Retinal Conditions
  - REGENXBIO plans to develop RGX-314 for the treatment of additional chronic retinal conditions that respond to anti-vascular endothelial growth factor (anti-VEGF) therapy but would benefit from improved, long-term treatment solutions. In the second half of 2019, REGENXBIO plans to file the first such IND for an additional Phase II clinical trial

designed to further evaluate the potential benefit of RGX-314 as a one-time anti-VEGF treatment in such conditions. REGENXBIO expects to provide additional updates on development plans in mid-2019.

- REGENXBIO to Host Upcoming RGX-314 Program Analyst and Investor Event
  - REGENXBIO today announced it will host an analyst and investor event with leading retina specialists and key opinion leaders on Thursday, February 21 at 9:00 a.m. ET in New York.
  - Featured speakers will include:
    - John Pollack, M.D., Partner at Illinois Retina Associates, Assistant Professor of Ophthalmology at Rush University Medical Center and President of the American Society of Retina Specialists (ASRS)
    - Pravin U. Dugel, M.D., Subspecialty Day Board Chairman Emeritus for the American Academy of Ophthalmology (AAO), Board of Directors and Executive Committee of ASRS and Board of Trustees of EuRetina, Clinical Professor at Roski Eye Institute and University of Southern California Keck School of Medicine, Senior and Managing Partner at Retinal Consultants of Arizona, Phoenix
    - Jeffrey Heier, M.D., Co-President, Medical Director and Retina Service Director of Retina Research Ophthalmic Consultants of Boston, Principal Investigator of the RGX-314 Phase I/IIa clinical trial
    - Allen C. Ho, M.D., Executive Committee of the Retina Society, Professor of Ophthalmology at Sidney Kimmel Medical College at Thomas Jefferson University, Director of Retina Research at Wills Eye Hospital, Investigator in the RGX-314 Phase I/IIa clinical trial
  - Analysts and investors are invited to listen to a live webcast of the event, which will be accessible in the Investors section of the REGENXBIO website at [www.regenxbio.com](http://www.regenxbio.com). An archived replay of the webcast will be available on the same website for approximately 30 days following the event.
- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
  - As of December 31, 2018, one subject has been dosed in the first of two expected dose cohorts of the Phase I/II clinical trial evaluating RGX-121 for the treatment of MPS II. The subject recently completed an initial eight-week safety assessment, and RGX-121 has been well-tolerated with no SAEs reported as of December 4, 2018. Additional recruitment and site activation continues. The next program updates are expected in the second half of 2019.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
  - Patient recruitment continues in the Phase I clinical trial evaluating RGX-111 for the treatment of MPS I. Under the current FDA protocol, recruitment is focused on an initial patient over 18 years of age. REGENXBIO is working with the Brazilian Health

Surveillance Agency (ANVISA) to enable initiation of a Phase I/II clinical trial evaluating RGX-111 for the treatment of MPS I in subjects under the age of three. Dosing of the first subject in a clinical trial evaluating RGX-111 is anticipated in mid-2019.

- RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)
  - As of December 31, 2018, a total of six subjects have been enrolled in two dose cohorts of the Phase I/II trial for RGX-501 for the treatment of HoFH. As was previously reported, all three subjects enrolled at the dose of  $7.5 \times 10^{12}$  GC/kg body weight (Cohort 2) experienced elevation in transaminases four to six weeks post-dosing, were asymptomatic and responded rapidly to the initiation of corticosteroids followed by a slow taper, with normalization of the transaminases. No new drug-related SAEs have been reported as of December 31, 2018.
  - In November 2018, REGENXBIO entered into a new clinical trial agreement with the University of Pennsylvania (Penn), the original RGX-501 trial sponsor, which allowed REGENXBIO to become the trial sponsor following the required activities with regulatory authorities to effectuate the transfer of sponsorship. REGENXBIO believes this will allow for enhanced visibility and control over the RGX-501 trial. In the U.S., the RGX-501 IND application was transferred in November 2018. Transfer of the Clinical Trial Applications for all other participating countries is ongoing.
  - In parallel, an amendment to the Phase I/IIa clinical trial protocol has been submitted to health authorities to allow for the enrollment of additional subjects at the Cohort 2 dose using corticosteroid prophylaxis. Trial recruitment has resumed. While REGENXBIO has assumed sponsor responsibilities, Penn will continue to support this study both as a scientific collaborator and as the principal dosing site. Dan Rader, M.D., Seymour Gray Professor of Molecular Medicine, and Marina Cuchel, M.D., Ph.D., Research Associate Professor of Medicine from Penn, will serve as co-global principal investigators for the trial.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
  - REGENXBIO has initiated IND-enabling studies for RGX-181. REGENXBIO plans to submit an IND application for RGX-181 in the second half of 2019.

#### **Anticipated 2019 Milestones**

REGENXBIO expects to meet the following anticipated milestones related to the clinical development of internal lead product candidates in 2019:

- RGX-314 for the Treatment of Wet AMD
  - Complete enrollment of the Phase IIa arm of the clinical trial in the first half of 2019.
  - Present top-line data from the Phase I/IIa clinical trial by the end of 2019.
  - Initiate the Phase IIb clinical trial in late 2019.
- RGX-314 for Additional Ophthalmic Indications
  - File an IND for a Phase II clinical trial of an additional proposed ophthalmic indication in the second half of 2019.
- RGX-121 for the Treatment of MPS II

- Complete enrollment of Cohort 1 and commence enrollment of Cohort 2 in the Phase I/II clinical trial.
- Present an interim data update from the Phase I/II clinical trial in the second half of 2019.
- RGX-111 for the Treatment of MPS I
  - Begin enrollment in the Phase I clinical trial in mid-2019.
  - Present an interim update from the Phase I clinical trial in the second half of 2019.
- RGX-501 for the Treatment of HoFH
  - Resume enrollment in the Phase I/II clinical trial in the first half of 2019.
  - Present interim data from Cohort 2 with steroid prophylaxis from the Phase I/II clinical trial in the second half of 2019.
- RGX-181 for the Treatment of CLN2 Disease
  - File an IND for the first-in-human clinical trial in the second half of 2019.

#### **NAV Technology Licensee Program Highlights**

As of December 31, 2018, REGENXBIO's NAV Technology Platform was being applied in more than 20 partnered product candidates in development by NAV Technology Licensees. Thirteen of these partnered product candidates are in active clinical development, and one partnered product candidate has been submitted for Biologics License Application (BLA) approval with the FDA. Many NAV Technology Licensee advancements and achievements were announced in 2018, including:

##### *Acquisition of AveXis by Novartis*

- In May 2018, AveXis, Inc. was acquired by Novartis AG for approximately \$8.7 billion in cash. In 2018, REGENXBIO received \$180 million under a license agreement with AveXis and is eligible to receive an additional \$80 million in potential future commercial milestone payments, in addition to regulatory milestones and royalties on net sales.

##### *Advancement and Commercialization of NAV Technology Licensee Clinical Programs*

- In December 2018, Novartis announced that the FDA has accepted the BLA for ZOLGENSMA for the treatment of spinal muscular atrophy (SMA) Type I, with regulatory action anticipated in May 2019. ZOLGENSMA uses the NAV AAV9 vector.
- In October 2018, Audentes Therapeutics, Inc. announced updated positive interim data from its Phase I/II clinical trial evaluating AT132 for the treatment of X-linked myotubular myopathy, including meaningful improvements in neuromuscular and respiratory function in all treated subjects, with no new treatment-related SAEs reported since the last scientific update in May 2018. AT132 uses the NAV AAV8 vector.
- In August 2018, Ultragenyx Pharmaceutical Inc. announced that the IND for DTX201 for the treatment of hemophilia A, developed in partnership with Bayer AG, is active. DTX201 uses the NAV AAVhu37 vector.
- In July 2018, Ultragenyx announced that the first subject was dosed in the Phase I/II clinical trial evaluating DTX401 for the treatment of glycogen storage disease type 1a. DTX401 uses the NAV AAV8 vector.
- In February 2018, Audentes announced dosing of the first patient in the Phase I/II clinical trial evaluating AT342 for the treatment of Crigler-Najjar Syndrome. AT342 uses the NAV AAV8 vector.

- In November 2018, REGENXBIO and Abeona Therapeutics Inc. announced a license agreement for the development and commercialization of treatments for mucopolysaccharidosis type IIIA, mucopolysaccharidosis type IIIB, neuronal ceroid lipofuscinosis type 1, also known as infantile Batten disease, and neuronal ceroid lipofuscinosis type 3, also known as juvenile Batten disease, using the NAV AAV9 vector.
- In November 2018, REGENXBIO and Rocket Pharmaceuticals, Inc. announced a license agreement for the development and commercialization of treatments for Danon Disease using the NAV AAV9 vector as well as exclusive options to two additional undisclosed NAV AAV vectors.
- In October 2018, Ultragenyx announced that it had exercised its option with REGENXBIO for the development of treatments for CDKL5 Deficiency Disorder using the NAV Technology Platform, using the NAV AAV9 vector.

#### Financial Guidance

As of December 31, 2018, REGENXBIO had more than \$470 million in cash, cash equivalents and marketable securities. Based on its current operating plan, and excluding any commercial revenue from ZOLGENSMA, REGENXBIO expects that its balance in cash, cash equivalents and marketable securities will be between \$330 million and \$350 million at the end of 2019, which will be used to support the continued development of its lead product candidate programs. Importantly, REGENXBIO anticipates adding commercial revenue from ZOLGENSMA to its existing base of partner revenue this year, pending FDA approval.

REGENXBIO management will present a company overview detailing these goals and guidance at the 37<sup>th</sup> Annual J.P Morgan Healthcare Conference on Wednesday, January 9, 2019 at 4:30 p.m. PT. A live webcast of the presentation can be accessed in the Investors section of the REGENXBIO website at [www.regenxbio.com](http://www.regenxbio.com). An archived replay of the webcast will be available on the same website for approximately 30 days following the presentation.

#### About REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

#### Forward-Looking Statements

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. 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 of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2017 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at [www.sec.gov](http://www.sec.gov). All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. 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 REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

<sup>1</sup> One SAE was a procedure-related peripheral retinal detachment that occurred, was repaired with a scleral buckle and resolved without significant sequelae.

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