REGENXBIO Reports First Quarter 2019 Financial and Operating Results and Additional Positive Interim Phase I/IIa Trial Update for RGX-314 for the Treatment of Wet AMD

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- *Reports positive interim update from RGX-314 Phase I/IIa trial for wet AMD in Cohort 3 over one year*
- *Announces new IND submission for RGX-314 for a Phase II trial in diabetic retinopathy; filing planned for second half 2019*
- *Continuing subject recruitment for RGX-121 Phase I/II trial for MPS II, RGX-111 Phase I trial for MPS I and RGX-501 Phase I/II trial for HoFH; interim updates for all trials expected in second half 2019*
- *On-track to submit IND for first-in-human trial of RGX-181 for CLN2 form of Batten disease in second half 2019*
- *Anticipates FDA approval of NAV Technology licensee Novartis' Zolgensma for SMA Type I*
- *$444 million in cash, cash equivalents and marketable securities as of March 31, 2019*

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 announced financial results for the quarter ended March 31, 2019, and recent operational highlights.

"We continue to be encouraged by the data from Cohort 3 of the RGX-314 Phase I/IIa trial for wet AMD, which demonstrated sustained protein expression and clinical effect on best corrected visual acuity and central retinal thickness measures at one year," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "These results further support the potential of our NAV Technology gene therapy, and we are excited to announce that we will expand clinical development of RGX-314 later this year into diabetic retinopathy, the most common cause of vision loss in people with diabetes, affecting approximately 8 million people in the United States alone."

Mr. Mills added: "This past quarter, we worked to drive significant clinical and regulatory progress across our five internal candidate programs for the treatment of retinal, neurodegenerative and metabolic diseases. We continue to advance our capabilities as a leader in the development and manufacture of NAV product candidates and to enable our NAV Technology licensee network to develop potentially life-changing treatments. As we celebrate the 10th anniversary of REGENXBIO's founding, we remain highly motivated and deeply committed to our mission of improving the lives of patients through the curative potential of gene therapy."

Interim Phase I/IIa Trial Update for RGX-314 for the Treatment of Wet Age-Related Macular Degeneration

REGENXBIO announced today an update to its Phase I/IIa trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), since its previously reported interim results. The interim data update announced today includes safety assessments for all subjects enrolled as of April 18, 2019, and new assessments of efficacy at one year after a single administration of RGX-314 for Cohort 3 (6 x 10^10 GC/eye).

- As of April 18, 2019, 33 subjects across five dose cohorts have been treated in the Phase I/IIa trial of RGX-314. RGX-314 continues to be well-tolerated across all cohorts, with no drug related serious adverse events (SAEs) reported.[1]
- In addition, the interim update announced today includes new assessments of protein expression levels, reduction in anti-vascular endothelial growth factor (VEGF) intravitreal injections, change in central retinal thickness (CRT) by spectral domain optical coherence tomography (SD-OCT), and Best Corrected Visual Acuity (BCVA) at one year after a single administration of RGX-314 for Cohort 3 (n=6). These new data are summarized below.
    - At one year after administration of RGX-314, Cohort 3 subjects continued to demonstrate evidence of sustained RGX-314 intraocular protein expression levels. Mean protein levels were 180.8 ng/ml one year post-treatment.
    - Mean BCVA improved by +5 letters and mean CRT decreased by 39 µm from baseline in

Cohort 3 subjects at one year.
    - Subjects upon enrollment in the study had received, on average, more than 35 anti-VEGF injections since diagnosis, prior to RGX-314 treatment. Cohort 3 subjects received a low number of anti-VEGF injections following the administration of RGX-314, with a mean of 2.3 injections over one year.
    - 50% of subjects (3/6) in Cohort 3 continue to remain injection-free at one year with persistent clinical durability of effect observed on BCVA and CRT. Mean BCVA improved by +10 letters and mean CRT decreased by 59 µm from baseline in these subjects at one year. Evidence of durable protein expression was also observed (see Table 1).

Table 1: RGX-314 Protein Expression Levels (ng/ml) of Cohort 3 Subjects with No Additional Anti-VEGF Injections through One Year (N=3)

Visit	1 month	6 months	1 year
Mean Protein Level (ng/ml)	236.2	274.9	260.5

"The sustained protein expression and durable clinical response observed in Cohort 3 at one year after one-time administration of RGX-314 in these previously treated subjects is promising," said Dr. Jeffrey Heier, Co-President and Director of Retina Research at Ophthalmic Consultants of Boston and primary investigator for the trial. "Most subjects in this study had received frequent anti-VEGF eye injections over five years at entry into the trial. The notable reduction in the number of anti-VEGF injections combined with the sustained clinical effect at one year show the potential of one-time gene therapy with RGX-314 to achieve long-lasting clinical outcomes while alleviating the burden of current treatment in wet AMD patients."

REGENXBIO has completed dosing subjects in Cohort 4 (1.6 x 10^11 GC/eye), is nearing completion of Cohort 5 (2.5 x 10^11 GC/eye) enrollment and expects to present top-line data from the Phase I/IIa clinical trial by the end of 2019. The company is on track to initiate a Phase IIb trial for wet AMD in late 2019.

Expansion of RGX-314 Program for the Treatment of Diabetic Retinopathy

Today, REGENXBIO announced it expects to file a new IND for a Phase II trial evaluating RGX-314 in subjects with diabetic retinopathy (DR) in the second half of 2019.

DR is the leading cause of vision loss in the working-age population and affects approximately 8 million people in the United States. DR is a complication of diabetes and is a progressive retinopathy, the severity of which ranges from mild non-proliferative diabetic retinopathy to a more advanced proliferative diabetic retinopathy (PDR). The main causes of vision loss secondary to DR are the vision-threatening complications of PDR, marked by the growth of new abnormal blood vessels onto the surface of the retina and vitreous cavity causing severe vision loss and diabetic macular edema (DME) leading to visual impairment. DME can occur at any stage of DR as the blood vessels in the retina become increasingly fragile and leak fluid.

"Diabetic retinopathy progression and its complications leading to vision impairment are largely driven by excess intraocular VEGF," said Dr. Stephen Pakola, Senior Vice President and Chief Medical Officer of REGENXBIO. "The standard of care for vision-threatening complications of diabetic eye disease is frequent intraocular injections of drugs that target VEGF on a long-term basis or panretinal laser treatment depending on the stage of the disease. We are excited at the potential applications of RGX-314 as a one-time therapy to address significant unmet needs in diabetic eye disease."

"Diabetic retinopathy is a serious public health concern affecting our growing diabetic patient populations, who often present with vision loss at a younger age than patients with macular degeneration," said Dr. Allen Ho, Wills Eye Hospital Attending Surgeon and Director of Retina Research and study investigator. "The opportunity to administer a one-time therapy to potentially halt disease progression and/or treat vision-threatening complications may offer important and efficient treatment options for patients who are otherwise burdened by numerous specialist visits and interventions for their diabetes."

For the Phase II trial evaluating RGX-314 in subjects with DR, REGENXBIO expects to administer the treatment using the same subretinal approach as in the current trial of RGX-314 for treatment of wet AMD. Safety and other data from the ongoing Phase I/IIa trial of RGX-314 in wet AMD will also be used to support dose selection. REGENXBIO anticipates many of the existing clinical sites from the Phase I/IIa trial will participate in the DR study, plus additional sites to be selected.

Manufacturing of Phase II product continues to be on track to support enrollment in all trials of RGX-314 for wet AMD and DR. REGENXBIO expects that its current manufacturing process is capable of meeting future clinical program demand and anticipated potential commercial demand.

Other Recent Operational Highlights

- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
    - At a recent 28-week safety assessment, RGX-121 continues to be well-tolerated with no serious adverse events (SAEs) reported. Additional recruitment and site activations are ongoing in the Phase I/II clinical trial evaluating RGX-121 for the treatment of MPS II.
    - REGENXBIO expects to present an interim data update from the Phase I/II clinical trial in the second half of 2019.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
    - Recruitment and additional site activations are ongoing in the Phase I clinical trial evaluating RGX-111 for the treatment of MPS I.
    - REGENXBIO expects to present an interim data update from the Phase I clinical trial in

the second half of 2019.

- RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)
  - The protocol has been amended and screening has re-initiated in the Phase I/II clinical trial evaluating RGX-501 for the treatment of HoFH.
  - REGENXBIO expects to report interim data from Cohort 2 with corticosteroid prophylaxis from the Phase I/II clinical trial in the second half of 2019.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
  - REGENXBIO expects to file an IND or foreign equivalent for the first-in-human clinical trial evaluating RGX-181 for the treatment of CLN2 in the second half of 2019.

NAV Technology Licensee Program Highlights

As of March 31, 2019, REGENXBIO's NAV Technology Platform was being applied in more than 20 partnered product candidates in development by NAV Technology Licensees. Fourteen 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. Over 200 subjects have been treated in clinical trials sponsored by NAV Technology Licensees. REGENXBIO's NAV Technology Licensees are advancing product candidates in a broad range of therapeutic areas and disease indications. Recent updates from NAV Technology Licensees include:

- In April 2019, Novartis announced that interim data from its Phase III STR1VE trial of Zolgensma in SMA type I showed prolonged event-free survival, an early and rapid increase in CHOP-INTEND scores and significant milestone achievement compared to untreated natural history, consistent with data from the pivotal Phase I START trial. Novartis has reiterated that they remain on track to launch Zolgensma in the United States and Japan in first half of 2019 and Europe in second half of 2019 for the treatment of spinal muscular atrophy (SMA) Type I, pending approval by the FDA. REGENXBIO is eligible to receive $80 million in potential future commercial milestone payments, in addition to regulatory milestones and royalties on net sales of Zolgensma. Zolgensma uses the NAV AAV9 vector.
- Earlier this week, Novartis presented interim data from their STRONG Phase I study of Zolgensma in Type 2 SMA and SPR1NT Phase III study, designed to evaluate Zolgensma in pre-symptomatic SMA newborns. Intrathecal administration of Zolgensma in SMA Type 2 subjects led to improvement of motor function, as assessed by HFMSE, and achievement of motor milestones following treatment. Initial results of the SPR1NT study in pre-symptomatic SMA newborns are encouraging, with subjects demonstrating improvement in CHOP-INTEND scores.
- In May 2019, Audentes Therapeutics, Inc. announced new positive data from ASPIRO, the Phase I/II Clinical Trial of AT132 for X-linked Myotubular Myopathy. The new data included up to 48 weeks of follow-up for nine treated subjects in two dose cohorts and reported significant and sustained improvements in neuromuscular and respiratory function in both dose cohorts, with corresponding achievement of clinically meaningful milestones. Audentes continues to plan for interactions in the second half of 2019 to gain further alignment on the license application submission pathways for AT132 in the United States and Europe. AT132 uses the NAV AAV8 vector.

Financial Results

Cash, cash equivalents and marketable securities were $444.3 million as of March 31, 2019, compared to $470.6 million as of December 31, 2018. The decrease was primarily attributable to net cash flows used in operating activities of $29.3 million.

Revenues were $0.9 million for the three months ended March 31, 2019, compared to $132.4 million for the three months ended March 31, 2018. The decrease was primarily attributable to $132.1 million of non-recurring revenue recognized during the three months ended March 31, 2018 under our amended license agreement with AveXis for the development and commercialization of treatments for SMA.

Research and development expenses were $25.2 million for the three months ended March 31, 2019, compared to $19.6 million for the three months ended March 31, 2018. The increase was primarily attributable to personnel costs as a result of increased headcount, laboratory and facilities costs and external expenses associated with conducting clinical trials and manufacturing-related services.

General and administrative expenses were $11.6 million for the three months ended March 31, 2019, compared to $8.4 million for the three months ended March 31, 2018. The increase was primarily attributable to personnel costs as a result of increased headcount and professional fees for

advisory and other services.

Net loss was $32.2 million, or $0.89 basic and diluted net loss per share, for the three months ended March 31, 2019, compared to net income of $104.2 million, or $3.30 basic and $3.04 diluted net income per share, for the three months ended March 31, 2018.

Financial Guidance

Upon regulatory approval and product launch, Zolgensma is expected to provide REGENXBIO with its first revenue stream from royalties on commercial product sales, adding commercial revenue to its existing base of licensee revenue this year. Based on REGENXBIO's current operating plan, and excluding any royalties from Zolgensma, REGENXBIO reiterates that it expects its balance in cash, cash equivalents and marketable securities to be between $330 million and $350 million as of December 31, 2019, which will be used to support the continued development of its lead product candidate programs.

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.

About Diabetic Retinopathy

Diabetic retinopathy (DR) is the leading cause of vision loss in adults between 25 and 74 years of age worldwide. DR affects approximately 8 million people in the United States alone. The spectrum of DR encompasses nonproliferative diabetic retinopathy and proliferative diabetic retinopathy, marked by the absence or presence of retinal neovascularization, respectively. Macular edema, defined as retinal thickening and edema involving the macula, can occur at any stage of DR and is a significant complication.

Current treatment options include anti-VEGF injections and/or panretinal laser treatment, depending on the stage of DR with and without macular edema. Anti-VEGF therapy, however, requires repetitive and inconvenient intraocular injections, typically ranging from every four to eight weeks in frequency, to maintain efficacy. Panretinal laser treatment cauterizes and destroys diseased retina to preserve healthy retina. Side-effects of panretinal laser include permanent decrease in peripheral and color vision. One-time administration of RGX-314 can potentially provide lasting treatment effect and halt progression of disease in DR by preventing and treating vision threatening complications of the disease.

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, 2018, 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. 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.

1. As previously reported, one SAE was a procedure-related peripheral retinal detachment that occurred, was repaired with a scleral buckle and resolved without significant sequelae.

REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except per share data)

	March 31, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	$ 55,852	$ 75,561
Marketable securities	229,373	244,200
Accounts receivable	8,372	8,587

Prepaid expenses	6,292	5,734
Other current assets	3,995	3,831
Total current assets	303,884	337,913
Marketable securities	159,083	150,819
Accounts receivable	22,758	23,012
Property and equipment, net	23,140	28,702
Operating lease right-of-use assets	6,858	—
Restricted cash	1,053	1,053
Other assets	2,255	2,315
Total assets	$ 519,031	$ 543,814
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 4,204	$ 4,412
Accrued expenses and other current liabilities	14,189	17,164
Deferred revenue	600	600
Operating lease liabilities	2,397	—
Total current liabilities	21,390	22,176
Deferred revenue	3,333	3,333
Operating lease liabilities	5,483	—
Deferred rent	—	1,098
Financing lease obligations	—	5,854
Other liabilities	1,772	2,505
Total liabilities	31,978	34,966
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2019 and December 31, 2018	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at March 31, 2019 and December 31, 2018; 36,611 and 36,120 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	602,425	592,580
Accumulated other comprehensive loss	(59)	(720)
Accumulated deficit	(115,317)	(83,016)
Total stockholders' equity	487,053	508,848
Total liabilities and stockholders' equity	$ 519,031	$ 543,814

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)
(unaudited)
(in thousands, except per share data)

	Three Months Ended March 31,	
	2019	2018
Revenues		
License revenue	$ 884	$ 132,391
Total revenues	884	132,391
Operating Expenses		
Costs of revenues		
Licensing costs	29	2,408
Research and development	25,203	19,550
General and administrative	11,558	8,380
Other operating expenses	—	28
Total operating expenses	36,790	30,366
Income (loss) from operations	(35,906)	102,025
Other Income		
Interest income from licensing	613	1,355
Investment income	2,995	859
Total other income	3,608	2,214
Income (loss) before income taxes	(32,298)	104,239
Income Tax Benefit	70	—
Net income (loss)	$ (32,228)	$ 104,239
Other Comprehensive Income (Loss)		
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	621	(188)
Total other comprehensive income (loss)	621	(188)
Comprehensive income (loss)	$ (31,607)	$ 104,051
Net income (loss) per share:		

Basic	$ (0.89)	$ 3.30
Diluted	$ (0.89)	$ 3.04
Weighted-average common shares outstanding:		
Basic	36,366	31,632
Diluted	36,366	34,275

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