REGENXBIO Reports First Quarter 2021 Financial Results and Operational Highlights

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ROCKVILLE, Md., May 5, 2021 /PRNewswire/ --

- *Enrollment ongoing in ATMOSPHERE™, the first of two planned pivotal trials for the subretinal delivery of RGX-314 for the treatment of wet AMD*
- *Continued progress and expansion of Phase II AAVIATE® trial of RGX-314 utilizing in-office suprachoroidal delivery for the treatment of wet AMD*
- *Began dosing in Phase I/II trial of RGX-121, a one-time gene therapy for MPS II, in pediatric patients over 5 years old*
- *Completed dosing in Cohort 1 of ongoing trial of RGX-111, a one-time gene therapy for MPS I*
- *IND filing expected in mid-2021 for RGX-202, a novel, advanced microdystrophin gene therapy for treatment of Duchenne Muscular Dystrophy*
- *$657 million in cash, cash equivalents and marketable securities as of March 31, 2021*
- *Conference call Wednesday, May 5th at 4:30 p.m. ET*

REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the first quarter ended March 31, 2021, and recent operational highlights.

"In the first quarter of 2021, we were able to build upon the important clinical advancements we made in 2020, including continued enrollment of patients in the Company's first pivotal trial for the treatment of wet AMD," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Additionally, we are pleased to announce the advancement of our Phase II AAVIATE® trial for the suprachoroidal delivery of RGX-314 for wet AMD where we have completed enrollment in the second cohort of patients and expanded the trial to evaluate suprachoroidal delivery of RGX-314 in patients who are positive for neutralizing antibodies. This cohort of patients may provide us additional information about the effects of RGX-314 in neutralizing antibody positive patients, potentially broadening the patient population that could be treated with this in-office delivery approach."

Mr. Mills added: "Beyond RGX-314, we have continued to advance our MPS II clinical program, enrolling additional patients in our ongoing Phase I/II trial of RGX-121 in younger patients, as well as dosing the first patient in our Phase I/II trial of RGX-121 in older patients. The ongoing Phase I/II trial of RGX-111 in patients with MPS I also continues to advance, having completed dosing in Cohort 1. We are also on track to file an IND for RGX-202 in mid-2021. We look forward to providing additional updates from our programs in 2021."

Recent Operational Highlights

Gene Therapy Using NAV® Vectors for AAV-Mediated Antibody Delivery

- Pivotal Program for RGX-314 for the Treatment of Wet Age-related Macular Degeneration (wet AMD)
  - Enrollment is ongoing in ATMOSPHERE™, the first of two planned pivotal trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach.
    - The randomized, well-controlled trial will enroll approximately 300 patients across two RGX-314 dose arms versus ranibizumab. The primary endpoint of the trial is non-inferiority to ranibizumab based on change from baseline in Best Corrected Visual Acuity (BCVA) at one year.
  - The second pivotal trial is expected to be similar in design to ATMOSPHERE and REGENXBIO plans to initiate the trial in the second half of 2021.
  - The manufacturing bridging study is now active to align with plans to incorporate REGENXBIO's scalable suspension cell culture manufacturing process to support potential future commercialization of RGX-314.
    - The open-label study will enroll approximately 60 patients to evaluate two

manufacturing process formulations of RGX-314. The primary endpoint of the trial is RGX-314 protein concentration in the aqueous humor at six months.

- Suprachoroidal Delivery of RGX-314 for the Treatment of Wet AMD
  - REGENXBIO plans to report interim data from Cohort 1 of AAVIATE, a Phase II trial of RGX-314 for the treatment of wet AMD, in the third quarter of 2021.
  - REGENXBIO has completed dosing of patients in Cohort 2 of AAVIATE and expects to report interim data from Cohort 2 in the second half of 2021.
  - In addition, REGENXBIO has expanded AAVIATE, and began dosing in a third cohort of patients.
    - Cohort 3 will evaluate the efficacy, safety and tolerability of RGX-314 in up to 20 patients who are neutralizing antibody (NAb) positive.
    - The same dose evaluated in Cohort 2, $5.0x10^{11}$ genomic copies per eye (GC/eye) of RGX-314, will be delivered to patients in Cohort 3 via a single injection. As with Cohorts 1 and 2, patients in Cohort 3 will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

- Suprachoroidal Delivery of RGX-314 for the Treatment of Diabetic Retinopathy (DR)
  - REGENXBIO continues to enroll patients in Cohort 1 in ALTITUDE™, a Phase II trial for the treatment of DR, and expects to report initial data in 2021.

- Research Program for the Treatment of Hereditary Angioedema (HAE)
  - REGENXBIO expects to provide a program update in 2021.

- Research Program for the Treatment of Neurodegenerative Diseases
  - REGENXBIO continues to collaborate with Neurimmune AG on research programs targeting both alpha synuclein and tau and expects to provide a program update in 2021.

Gene Therapy Using NAV Vectors for Rare Genetic Diseases

- RGX-202 for the Treatment of Duchenne Muscular Dystrophy (DMD)
  - REGENXBIO expects to submit an Investigational New Drug application (IND) to the FDA for RGX-202 for the treatment of DMD in mid-2021.

- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
  - In April 2021, REGENXBIO announced that the first patient had been dosed in Cohort 3 of the ongoing Phase I/II trial of RGX-121 for the treatment of MPS II in patients up to 5 years old. Patients in the third cohort will receive a dose of $2.0x10^{11}$ GC/g of brain mass of RGX-121, delivered directly to the cerebrospinal fluid (CSF).
  - In addition, the first patient has been dosed in a second Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of 5 years old.
    - Up to six patients are expected to be enrolled in the multicenter, open-label trial, and RGX-121 will be administered at a dose level of $6.5x10^{10}$ GC/g of brain mass, delivered directly to the CSF.

- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
  - REGENXBIO has completed dosing of patients in Cohort 1 of the Phase I/II trial of RGX-111 for the treatment of MPS I.

- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)

Disease
    - An IND was submitted to the U.S. Food and Drug Administration (FDA), after which the FDA notified REGENXBIO in a letter that its proposed trial had been placed on clinical hold and the agency requested more information to support the initial dose selection and certain study drug administration procedures. REGENXBIO is evaluating the FDA's requests and plans to provide an update on the program in the second half of 2021.

- RGX-381 for the Treatment of Ocular Manifestations of CLN2 Disease
    - Based on communication with the FDA and the update from the RGX-181 program, REGENXBIO now expects to provide a program update for RGX-381 in the second half of 2021.

Operational Updates

- Current Good Manufacturing Practice (cGMP) Manufacturing Facility
    - REGENXBIO expects to begin utilizing its new headquarters in Rockville, Maryland in the first half of 2021, and the cGMP facility which is expected to allow for production of NAV vectors at scales up to 2,000 liters using REGENXBIO's platform suspension cell culture process is on track to be operational starting in the first half of 2022.

NAV Technology Licensee Program Highlights

As of March 31, 2021, REGENXBIO's NAV Technology Platform was being applied in one marketed product and multiple clinical stage programs, with over 20 partnered programs in total. 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 January 2021, Ultragenyx Pharmaceutical Inc. announced FDA Clearance of an IND for UX701, a gene therapy for the treatment of Wilson Disease. Ultragenyx is expected to begin enrolling patients in a single-protocol Phase 1/2/3 study in the second half of 2021. UX701 uses the NAV AAV9 vector.

- In April 2021, Astellas Gene Therapies, formerly Audentes Therapeutics, Inc., announced that the first patient has been dosed in its Phase I/II FORTIS trial evaluating AT845 in patients with late-onset Pompe disease. AT845 uses the NAV AAV8 vector.

Marketed NAV Technology Products

REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma®. On April 27, 2021, Novartis AG reported first quarter 2021 global Zolgensma sales revenue of $319 million.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $656.5 million as of March 31, 2021, compared to $522.5 million as of December 31, 2020. The increase was primarily attributable to $216.1 million of aggregate net proceeds received from the Company's follow-on public offering of common stock completed in January 2021, including the full exercise of the underwriters' option to purchase additional shares in connection with the offering. The increase was partially offset by net cash used in operating activities of $41.1 million, cash used to purchase property and equipment of $31.0 million, and Zolgensma royalties paid to Healthcare Royalty Management, LLC of $9.5 million during the three months ended March 31, 2021.

Revenues: Revenues were $18.9 million for the three months ended March 31, 2021, compared to $17.6 million for the three months ended March 31, 2020. The increase was primarily attributable to Zolgensma royalty revenues, which increased by $8.3 million, from $10.0 million for the first quarter of 2020 to $18.3 million for the first quarter of 2021. As reported by Novartis, sales of Zolgensma for the first quarter of 2021 increased by 88% as compared to the first quarter of 2020. The increase in revenues was partially offset by non-recurring revenue recognized during the three months ended March 31, 2020 related to new licenses to the NAV Technology Platform granted by REGENXBIO during the period.

Research and Development Expenses: Research and development expenses were $39.7 million for the three months ended March 31, 2021, compared to $37.0 million for the three months ended March 31, 2020. The increase was primarily attributable to personnel costs as a result of increased headcount, laboratory and facilities costs, and clinical trial expenses for our lead product candidates.

General and Administrative Expenses: General and administrative expenses were $17.8 million for the three months ended March 31, 2021, compared to $14.8 million for the three months ended March 31, 2020. The increase was primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services.

Net Loss: Net loss was $50.1 million, or $1.20 basic and diluted net loss per share, for the three months March 31, 2021, compared to net loss of $40.0 million, or $1.08 basic and diluted net loss per share, for the three months ended March 31, 2020.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $656.5 million as of March 31, 2021, to fund its operations, including the completion of its internal manufacturing capabilities and clinical advancement of its product candidates, into the second half of 2023.

Conference Call

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 4:30 p.m. ET. To access the live call by phone, dial (855) 422-8964 (domestic) or (210) 229-8819 (international) and enter the passcode 7044809. To access a live or recorded webcast of the call, please visit the "Investors" section of the REGENXBIO website at www.regenxbio.com. The recorded webcast will be available for approximately 30 days following the call.

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," "assume," "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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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, 2020, 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. Except as required by law, 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.

Zolgensma® is a registered trademark of Novartis Gene Therapies. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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

	March 31, 2021	December 31, 2020
Assets		
Current assets		
Cash and cash equivalents	$ 291,482	$ 338,426
Marketable securities	149,398	137,314
Accounts receivable, net	41,039	42,999
Prepaid expenses	13,839	10,505
Other current assets	2,880	1,953
Total current assets	498,638	531,197
Marketable securities	215,598	46,809
Accounts receivable, net	2,859	3,267
Property and equipment, net	89,342	56,467
Operating lease right-of-use assets	62,607	63,815
Restricted cash	1,330	1,330
Other assets	9,068	5,279
Total assets	$ 879,442	$ 708,164
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 11,311	$ 10,622
Accrued expenses and other current liabilities	41,605	49,082
Deferred revenue	395	449

Operating lease liabilities	1,843	2,500
Liability related to sale of future royalties	28,807	18,794
Total current liabilities	83,961	81,447
Deferred revenue	3,729	3,783
Operating lease liabilities	75,078	70,153
Liability related to sale of future royalties	161,722	174,504
Other liabilities	448	524
Total liabilities	324,938	330,411
Stockholders' equity		
Preferred stock; no shares issued and outstanding at March 31, 2021 and December 31, 2020	—	—
Common stock; 42,505 and 37,476 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	4	4
Additional paid-in capital	895,079	667,181
Accumulated other comprehensive loss	(1,368)	(360)
Accumulated deficit	(339,211)	(289,072)
Total stockholders' equity	554,504	377,753
Total liabilities and stockholders' equity	$ 879,442	$ 708,164

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

	Three Months Ended March 31,	
	2021	2020
Revenues		
License and royalty revenue	$ 18,884	$ 17,644
Total revenues	18,884	17,644
Operating Expenses		
Cost of revenues	4,851	3,409
Research and development	39,722	37,035
General and administrative	17,838	14,833
Provision for credit losses and other	515	67
Total operating expenses	62,926	55,344
Loss from operations	(44,042)	(37,700)
Other Income (Expense)		
Interest income from licensing	29	848
Investment income (loss)	580	(3,186)
Interest expense	(6,702)	—
Total other income (expense)	(6,093)	(2,338)
Loss before income taxes	(50,135)	(40,038)
Income Tax Expense	(4)	—
Net loss	$ (50,139)	$ (40,038)
Other Comprehensive Loss		
Unrealized loss on available-for-sale securities, net	(1,008)	(785)
Total other comprehensive loss	(1,008)	(785)
Comprehensive loss	$ (51,147)	$ (40,823)
Net loss per share, basic and diluted	$ (1.20)	$ (1.08)
Weighted-average common shares outstanding, basic and diluted	41,819	37,104

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