REGENXBIO Reports First Quarter 2020 Financial Results and Operational Highlights

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- Positive long-term and interim data update from RGX-314 Phase I/IIa trial for wet AMD recently announced

- RGX-314 clinical trials for treatment of wet AMD and diabetic retinopathy to advance in 2020 using subretinal and suprachoroidal delivery approaches

- Additional interim data from RGX-121 Phase I/II trial for MPS II expected in 2020

- Revenue includes $10 million in royalties from the Q1 2020 sales of Novartis' Zolgensma®; Zolgensma has recorded total net sales of over $530 million since launch

- $357 million in cash, cash equivalents and marketable securities as of March 31, 2020

- Conference call Thursday, May 7th, at 4:30 p.m. ET

ROCKVILLE, Md., May 7, 2020 /PRNewswire/ -- 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, 2020, and recent operational highlights.

"Our overall focus at REGENXBIO during the COVID-19 pandemic is on the health and safety of our people and our communities, as well as the important work that we need to continue in order to help patients," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We recently reported additional positive data from our RGX-314 Phase I/IIa wet AMD trial which demonstrated long-term, sustained clinical outcomes in patients after a one-time administration of RGX-314 gene therapy. We look forward to initiating a pivotal program for the subretinal delivery of RGX-314 for wet AMD as well as additional studies using the suprachoroidal delivery approach in both wet AMD and diabetic retinopathy in 2020. We also anticipate providing updates throughout 2020 from our other programs, including the Phase I/II trials of RGX-121 for the treatment of MPS II and RGX-501 for the treatment of HoFH."

Mr. Mills added: "We continue to track the positive progress of Novartis' Zolgensma which uses the NAV AAV9 vector for the treatment of pediatric patients with spinal muscular atrophy (SMA). We are encouraged by the recent regulatory updates from Japan and Europe, and we look forward to continued sales growth for Zolgensma. After more than a decade of steadfast effort and focus, we remain dedicated and committed to improving lives through the curative potential of gene therapy."

Recent Operational Highlights

Gene Therapy Using NAV Vectors for AAV-Mediated Antibody Delivery

- RGX-314 for the Treatment of Wet AMD
  - In April 2020, REGENXBIO reported positive additional data from the RGX-314 Phase I/IIa trial using subretinal delivery. As of April 6, 2020:
    - RGX-314 continued to be well-tolerated at all dose levels.
    - Long-term, durable treatment effect was demonstrated in Cohort 3:
      - Mean improvement in vision (+14 letters) and stable retinal thickness (+2 µm) observed at two years.
      - 50% of patients (3/6) remained anti-VEGF injection-free over two years.
      - 67% of patients (4/6) were anti-VEGF injection-free from nine months to two years.
      - Stable intraocular RGX-314 protein expression observed over two years.
    - 73% of patients (8/11) in Cohort 5 were anti-VEGF injection-free over nine months.
    - Intraocular RGX-314 protein levels at six months demonstrated dose-dependent expression across cohorts.
  - REGENXBIO expects to initiate a pivotal program for the subretinal delivery of RGX-314 for the treatment of wet AMD in the second half of 2020.
    - REGENXBIO plans to finalize the design of the trial based on the one-year assessment of patients in Cohorts 4 and 5 in the Phase I/IIa trial and expects to

begin dosing patients in the trial in the second half of 2020.
    - REGENXBIO plans to initiate the Phase II trial for the suprachoroidal delivery of RGX-314 using the SCS Microinjector™ for the treatment of wet AMD in the first half of 2020.
        - Interim data is expected from the first cohort by the end of 2020.
- RGX-314 for the Treatment of Diabetic Retinopathy (DR)
    - REGENXBIO plans to evaluate RGX-314 using the SCS Microinjector for the treatment of DR, and expects to submit an investigational new drug (IND) application for a Phase II trial in mid-2020.
        - REGENXBIO plans to initiate the trial in the second half of 2020 and interim data is expected in 2021.
- Research Program for the Treatment of Hereditary Angioedema (HAE)
    - Lead product candidate selection is expected in the first half of 2020 and REGENXBIO expects to provide a program update in the second half of 2020.
- Research Program for the Treatment of Neurodegenerative Diseases
    - REGENXBIO previously announced the expansion of the exclusive collaboration with Neurimmune AG to design and develop vectorized antibody therapies targeting both alpha synuclein and tau. REGENXBIO expects to provide a program update in the second half of 2020.

Gene Therapy Using NAV Vectors for Rare Genetic Diseases

- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
    - Initial data from Cohort 1 of the Phase I/II trial were presented at the WORLD*Symposium* conference in February 2020 and REGENXBIO expects to provide additional data from this cohort in mid-2020.
    - REGENXBIO expects to complete enrollment of patients in Cohort 2 in the first half of 2020, with interim data anticipated in the second half of 2020.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
    - Recruitment, screening and additional site activations are ongoing in the Phase I/II trial.
    - REGENXBIO expects to provide a program update in the second half of 2020.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
    - REGENXBIO is conducting ongoing preclinical development of RGX-181, including assessment of unmet clinical needs such as neurologic and ophthalmologic manifestations of the disease. REGENXBIO expects to provide a program update in mid-2020 and submit an IND for a first-in-human trial in the second half of 2020.
- RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)
    - As previously announced, REGENXBIO completed dosing of an expanded Cohort 2 in the Phase I/II trial and plans to assess low-density lipoprotein (LDL-C) levels after all patients have completed steroid prophylaxis treatment. REGENXBIO expects to provide a program update in the first half of 2020.
- Research Program for the Treatment of Neuromuscular Disorders
    - REGENXBIO expects to announce plans for clinical development of a potential treatment for a neuromuscular disorder using NAV AAV8 in the second half of 2020.

Operational Updates

- Current Good Manufacturing Practice (cGMP) Manufacturing Facility
    - Construction of a new corporate, research and manufacturing headquarters in Rockville, Maryland continues, with plans to begin utilizing the new headquarters in late 2020.

- The new cGMP production facility is expected to allow for production of NAV vectors at scales up to 2,000 liters using REGENXBIO's platform suspension cell culture process, which will complement REGENXBIO's current external manufacturing network and capabilities. The cGMP facility is expected to be operational starting in 2021.

NAV Technology Licensee Program Highlights

As of March 31, 2020, REGENXBIO's NAV Technology Platform was being applied in one marketed product, and the clinical development of 15 partnered product candidates, 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:

- On March 31, 2020, REGENXBIO and Ultragenyx Pharmaceutical Inc. announced a new exclusive, worldwide license agreement to REGENXBIO's NAV AAV8 and AAV9 vectors for the treatment of a rare metabolic disorder, extending the companies' existing gene therapy partnership. In return for these rights, REGENXBIO received an upfront payment of $7 million, and will receive ongoing fees, milestone payments, and royalties on net sales of products incorporating the licensed intellectual property.

Marketed NAV Technology Product Highlights

- On April 28, 2020, Novartis AG reported first quarter 2020 Zolgensma® sales revenue of $170 million, and REGENXBIO recognized royalty revenue of $10.0 million in the first quarter of 2020 as a result of these sales. Since launch in May 2019, Zolgensma has recorded total net sales of over $530 million.
- On March 27, 2020, Novartis announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending conditional marketing authorization of Zolgensma.
- On March 19, 2020, Novartis announced that Zolgensma received approval from the Japanese Ministry of Health, Labour and Welfare (MHLW) for the treatment of patients under the age of two with spinal muscular atrophy (SMA), including patients who are pre-symptomatic at diagnosis.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $356.6 million as of March 31, 2020, compared to $400.0 million as of December 31, 2019. The decrease was primarily attributable to net cash used in operating activities of $35.6 million and cash used to purchase property and equipment of $4.6 million.

Revenues: Revenues were $17.6 million for the three months ended March 31, 2020, compared to $0.9 million for the three months ended March 31, 2019. The increase was primarily attributable to $10.0 million of royalty revenue recognized on net sales of Zolgensma in the first quarter of 2020, as well as $7.2 million of license revenue recognized from a new license granted to Ultragenyx Pharmaceutical Inc. during the period. Commercial sales of Zolgensma commenced in the second quarter of 2019, and REGENXBIO is eligible to receive a milestone payment of $80.0 million from AveXis, Inc. upon the achievement of $1.0 billion in cumulative net sales of Zolgensma.

Research and Development Expenses: Research and development expenses were $37.0 million for the three months ended March 31, 2020, compared to $25.2 million for the three months ended March 31, 2019. The increase was primarily attributable to personnel-related costs as a result of increased headcount, laboratory and facilities costs, expenses associated with conducting clinical trials for our lead product candidates and externally sourced services for preclinical, regulatory and manufacturing-related activities.

General and Administrative Expenses: General and administrative expenses were $14.8 million for the three months ended March 31, 2020, compared to $11.6 million for the three months ended March 31, 2019. The increase was primarily attributable to personnel-related costs as a result of increased headcount and professional fees for advisory and other services.

Net Loss: Net loss was $40.0 million, or $1.08 basic and diluted net loss per share, for the three months March 31, 2020, compared to net loss of $32.2 million, or $0.89 basic and diluted net loss per share, for the three months ended March 31, 2019.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $356.6 million to fund the completion of its internal manufacturing capabilities and clinical advancement of its product candidates into 2022.

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 3893881. To access a live or recorded webcast of the call and accompanying slides, 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," "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, 2019, 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.

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

	March 31, 2020	December 31, 2019
Assets		
Current assets		
Cash and cash equivalents	$ 69,411	$ 69,514
Marketable securities	209,846	226,696
Accounts receivable	44,522	38,148
Prepaid expenses	7,422	6,475
Other current assets	7,032	4,199
Total current assets	338,233	345,032
Marketable securities	77,361	103,785
Accounts receivable	4,373	4,155
Property and equipment, net	30,414	28,973
Operating lease right-of-use assets	9,375	10,078
Restricted cash	1,330	1,330
Other assets	2,850	4,555
Total assets	$ 463,936	$ 497,908
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 9,008	$ 6,409
Accrued expenses and other current liabilities	20,327	24,846
Operating lease liabilities	2,454	2,421
Total current liabilities	31,789	33,676
Deferred revenue	3,333	3,333
Operating lease liabilities	7,990	8,874
Other liabilities	672	1,828
Total liabilities	43,784	47,711
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2020 and December 31, 2019	—	—

Common stock; $0.0001 par value; 100,000 shares authorized at March 31, 2020 and December 31, 2019; 37,190 and 36,992 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	638,588	627,810
Accumulated other comprehensive income (loss)	(580)	205
Accumulated deficit	(217,860)	(177,822)
Total stockholders' equity	420,152	450,197
Total liabilities and stockholders' equity	$ 463,936	$ 497,908

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

	Three Months Ended March 31,	
	2020	**2019**
Revenues		
License and royalty revenue	$ 17,644	$ 884
Total revenues	17,644	884
Operating Expenses		
Cost of revenues	3,409	29
Research and development	37,035	25,203
General and administrative	14,833	11,558
Other operating expenses	67	—
Total operating expenses	55,344	36,790
Loss from operations	(37,700)	(35,906)
Other Income (Loss)		
Interest income from licensing	848	613
Investment income (loss)	(3,186)	2,995
Total other income (loss)	(2,338)	3,608
Loss before income taxes	(40,038)	(32,298)
Income Tax Benefit	—	70
Net loss	$ (40,038)	$ (32,228)
Other Comprehensive Income (Loss)		
Unrealized gain (loss) on available-for-sale securities, net	(785)	621
Total other comprehensive income (loss)	(785)	621
Comprehensive loss	$ (40,823)	$ (31,607)
Basic and diluted net loss per share	$ (1.08)	$ (0.89)
Weighted-average basic and diluted common shares outstanding	37,104	36,366

Contacts:

Tricia Truehart
Investor Relations and Corporate Communications
347-926-7709
ttruehart@regenxbio.com

Investors:
Heather Savelle, 212-600-1902
heather@argotpartners.com

Media:
David Rosen, 212-600-1902
david.rosen@argotpartners.com

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