REGENXBIO Reports Fourth Quarter and Full-Year 2019 Financial Results and Operational Highlights

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- *Additional data from RGX-314 Phase I/IIa trial for wet AMD expected in 1H 2020*
- *RGX-314 clinical trials for treatment of wet AMD and diabetic retinopathy expected 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*
- *Plans to expand rare disease pipeline into neuromuscular disorders in 2H 2020*
- *$400 million in cash, cash equivalents and marketable securities as of December 31, 2019*
- *Conference call Wednesday, February 26th at 4:30 p.m. ET*

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 fourth quarter and full year ended December 31, 2019, and recent operational highlights.

"In 2020, we are broadening our internal gene therapy pipeline using our proprietary NAV Technology Platform, and advancing key programs including RGX-314 for the treatment of retinal diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "As we drive the RGX-314 program forward with new trials in wet AMD and diabetic retinopathy planned for later this year, we are also advancing our RGX-121 program in MPS II based on encouraging data from the first cohort of patients. We are pleased with the advancement of the RGX-111 and RGX-501 programs, and look forward to submitting an IND for RGX-181 and providing updates for our research programs in HAE and neurodegenerative diseases later this year."

Mr. Mills added: "Based on our in-house research and development team's work, we also plan to expand our rare disease platform to include a potential treatment for a neuromuscular disorder using NAV AAV8, which we believe has the potential to treat patients who lack treatments or who are currently underserved by existing therapies. I look forward to providing further updates on this work in the second half of the year."

Recent Operational Highlights

Gene Therapy Using NAV Vectors for AAV-Mediated Antibody Delivery

- RGX-314 for the Treatment of Wet AMD
  - REGENXBIO expects to report additional data from the RGX-314 Phase I/IIa trial using subretinal delivery in 2020
    - One-year data from Cohorts 4 and 5 is expected in mid-2020. As announced in January 2020, six-month data from Cohort 5 demonstrated meaningful reductions in anti-vascular endothelial growth factor (anti-VEGF) treatment burden following one-time administration of RGX-314. Eight out of 11 (73%) patients remained anti-VEGF injection-free, and demonstrated a mean improvement in vision of +5 ETDRS letters and a mean improvement in retinal thickness of -83 microns.
    - REGENXBIO expects to provide two-year data from Cohorts 1-3 in the first quarter of 2020.
  - 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 Cohort 5 in the Phase I/IIa trial and expects to begin dosing patients in a pivotal 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 expects to submit an IND for a Phase II trial for the treatment of DR in the first half of 2020. This trial will evaluate RGX-314 using the SCS Microinjector to deliver RGX-314 to the suprachoroidal space.
        - The trial is expected to begin in the second half of 2020 and enrollment of the first cohort is expected to be complete by the end of 2020, with interim data 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 will provide a program update in the second half of 2020.

- Research Program for the Treatment of Neurodegenerative Diseases
    - REGENXBIO recently 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 was presented at the WORLD*Symposium* ™ conference in February 2020. As of January 27, 2020, RGX-121 was well-tolerated with no drug-related or procedure-related serious adverse events assessed. As reported in December 2019, patients had demonstrated consistent and sustained reduction in heparan sulfate (HS) in the cerebral spinal fluid (CSF) and available data supported early signs of neurocognitive stability.
    - REGENXBIO expects to provide additional data from Cohort 1 in mid-2020.
    - The first patient of Cohort 2 has been dosed at an increased dose level and REGENXBIO expects to complete enrollment of the cohort in the first half of 2020, with interim data available in 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 clinical trial evaluating RGX-111 for the treatment of MPS I. Recruitment in this trial was previously focused on an initial patient over 18 years of age; however, the protocol has recently been amended to include patients 4 months of age and older, given REGENXBIO's clinical experience with the direct-to-CNS delivery platform and accumulating data from the ongong Phase I/II trial of RGX-121.
    - 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 of RGX-501 and plans to assess low-density lipoprotein (LDL-C) levels after all patients have completed steroid prophylaxis treatment. REGENXBIO expects to provide interim data 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 and Anticipated Milestones in 2020

- 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 fully operational starting in 2021.

NAV Technology Licensee Program Highlights

As of December 31, 2019, REGENXBIO's NAV Technology Platform was being applied in one marketed product and more than 20 partnered product candidates in development. Fifteen of these partnered product candidates are in active clinical development. 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:

Marketed NAV Technology Products

- On January 29, 2020, Novartis AG reported fourth quarter 2019 U.S. Zolgensma® sales revenue of $186 million, and REGENXBIO recognized royalty revenue of $10.7 million in the fourth quarter of 2019 as a result of these sales.
- Novartis has reported that Zolgensma is currently under regulatory review in Europe with an anticipated regulatory decision in the first quarter of 2020 and in Japan with an anticipated regulatory decision in the first half of 2020. Zolgensma uses the NAV AAV9 vector.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $400.0 million as of December 31, 2019, compared to $470.6 million as of December 31, 2018. The decrease was primarily attributable to $107.7 million of net cash used in operating activities during 2019, partially offset by realized and unrealized gains of $37.8 million related to our marketable equity securities of Prevail Therapeutics Inc.

Revenues: Revenues were $11.8 million and $35.2 million for the three months and year ended December 31, 2019, respectively, compared to $40.8 million and $218.5 million for the three months and year ended December 31, 2018, respectively. The decreases were primarily attributable to $176.1 million of non-recurring revenue recognized in 2018 under REGENXBIO's March 2014 license agreement with AveXis, Inc., as amended, as well as $35.6 million of non-recurring revenue recognized in the fourth quarter of 2018 under REGENXBIO's license agreement with Abeona Therapeutics Inc. The decreases in revenue were partially offset by $10.7 million and $20.8 million of royalty revenue recognized during the three months and year ended December 31, 2019, respectively, related to net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019, and we are eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma.

Research and Development Expenses: Research and development expenses were $33.8 million and $124.2 million for the three months and year ended December 31, 2019, respectively, compared to $24.3 million and $83.9 million for the three months and year ended December 31, 2018, respectively. The increases were primarily attributable to personnel 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 $14.5 million and $51.8 million for the three months and year ended December 31, 2019, respectively compared to $11.1 million and $36.9 million for the three months and year ended December 31, 2018, respectively. The increases were primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services.

Net Loss: Net loss was $26.5 million, or $0.72 basic and diluted net loss per share, and $94.7 million, or $2.58 basic and diluted net loss per share, for the three months and year ended December 31, 2019, respectively, compared to net income of $4.3 million, or $0.12 basic and $0.11 diluted net income per share, and $99.9 million, or $2.99 basic and $2.73 diluted net income per share, for the three months and year ended December 31, 2018, respectively.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $400 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 2439067. 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, 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, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2020, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the 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)

	December 31, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	$ 69,514	$ 75,561
Marketable securities	226,696	244,200
Accounts receivable	38,148	8,587
Prepaid expenses	6,475	5,734
Other current assets	4,199	3,831
Total current assets	345,032	337,913
Marketable securities	103,785	150,819
Accounts receivable	4,155	23,012
Property and equipment, net	28,973	28,702
Operating lease right-of-use assets	10,078	—
Restricted cash	1,330	1,053
Other assets	4,555	2,315
Total assets	$ 497,908	$ 543,814
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 6,409	$ 4,412
Accrued expenses and other current liabilities	24,846	17,164
Deferred revenue	—	600
Operating lease liabilities	2,421	—
Total current liabilities	33,676	22,176

Deferred revenue	3,333	3,333
Operating lease liabilities	8,874	—
Deferred rent	—	1,098
Financing lease obligations	—	5,854
Other liabilities	1,828	2,505
Total liabilities	47,711	34,966
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at December 31, 2019 and December 31, 2018	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at December 31, 2019 and December 31, 2018; 36,992 and 36,120 shares issued and outstanding at December 31, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	627,810	592,580
Accumulated other comprehensive income (loss)	205	(720)
Accumulated deficit	(177,822)	(83,016)
Total stockholders' equity	450,197	508,848
Total liabilities and stockholders' equity	$ 497,908	$ 543,814

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

	Three Months Ended December 31,		Years Ended December 31,	
	2019	2018	2019	2018
Revenues				
License and royalty revenue	$ 11,768	$ 40,777	$ 35,233	$ 218,505
Total revenues	11,768	40,777	35,233	218,505
Operating Expenses				
Cost of revenues	3,791	2,843	8,241	9,640
Research and development	33,807	24,329	124,185	83,873
General and administrative	14,450	11,144	51,815	36,850
Other operating expenses (income)	44	11	(10)	42
Total operating expenses	52,092	38,327	184,231	130,405
Income (loss) from operations	(40,324)	2,450	(148,998)	88,100
Other Income				
Interest income from licensing	860	584	2,951	8,946
Investment income	10,609	2,893	48,559	7,070
Total other income	11,469	3,477	51,510	16,016
Income (loss) before income taxes	(28,855)	5,927	(97,488)	104,116
Income Tax Benefit (Expense)	2,391	(1,621)	2,755	(4,179)
Net income (loss)	$ (26,464)	$ 4,306	$ (94,733)	$ 99,937
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(158)	154	885	(5)
Total other comprehensive income (loss)	(158)	154	885	(5)
Comprehensive income (loss)	$ (26,622)	$ 4,460	$ (93,848)	$ 99,932
Net income (loss) per share:				
Basic	$ (0.72)	$ 0.12	$ (2.58)	$ 2.99
Diluted	$ (0.72)	$ 0.11	$ (2.58)	$ 2.73
Weighted-average common shares outstanding:				
Basic	36,905	35,951	36,690	33,427
Diluted	36,905	38,933	36,690	36,648

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