REGENXBIO Reports Fourth Quarter and Full-Year 2018 Financial Results and Recent Operational Highlights

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- *Announces updates from recently expanded RGX-314 Phase I/IIa trial for wet AMD*
  - *Completed dosing of six additional subjects in the fourth cohort for a total of 12 subjects; 30 subjects dosed across four cohorts in the trial*
  - *On-track to initiate Phase IIb trial for wet AMD in late 2019*
  - *Continues to advance development of RGX-314 for other chronic retinal diseases; new IND submission for a Phase II trial in an additional retinal condition planned in second half 2019*
  - *Hosted an Analyst and Investor Day on February 21, 2019, with leading retina specialists who provided their perspectives on the potential advantages of one-time gene therapy as a foundational anti-VEGF treatment for wet AMD and other retinal diseases*
- *Continuing additional site activation and subject recruitment in RGX-121 Phase I/II trial for MPS II; recruitment continues in RGX-111 Phase I trial for MPS I*
- *Resumed recruitment in the RGX-501 Phase I/II trial for HoFH*
- *IND-enabling studies progressing for RGX-181 for CLN2 form of Batten disease; on-track to submit IND for first-in-human trial in second half 2019*
- *First anticipated commercial launch for a proprietary NAV® Technology-based treatment, Novartis' ZOLGENSMA® for the treatment of SMA Type I, expected in first half 2019 in United States and Japan; European launch expected in second half 2019*
- *$471 million in cash, cash equivalents and marketable securities as of December 31, 2018*
- *Conference call Wednesday, February 27th 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, 2018, and recent operational highlights.

"2018 was a pivotal year for REGENXBIO. Significant clinical and regulatory progress was made in advancing our NAV Technology Platform across 18 diverse clinical stage programs, four of which are in our own expanding pipeline of gene therapy product candidates for the treatment of retinal, neurodegenerative and metabolic diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "In 2019, we are focused on advancing and expanding our ophthalmology franchise with RGX-314, our lead product candidate for subjects with wet AMD, by extending development of this asset as a one-time anti-VEGF treatment in another chronic retinal condition. In addition, we anticipate the launch of the first NAV Technology-based treatment, Novartis' ZOLGENSMA for the treatment of SMA Type I, in the United States, Japan and Europe in 2019. This would be a major commercial milestone for our NAV Technology Platform and represent a post-launch commercial revenue stream for REGENXBIO."

Mr. Mills added: "Our mission is to improve lives through the curative potential of gene therapy. We continue to execute on our strategic priorities including the expansion of RGX-314 development into additional retinal conditions, advancing our current clinical programs with RGX-314, RGX-121, RGX-111 and RGX-501 and enabling our NAV Technology licensee network to develop potentially life-changing treatments. We believe we are well-positioned for a transformative 2019 as we build on last year's pivotal achievements and broaden our NAV Technology footprint."

Recent Operational Highlights

- RGX-314 for the Treatment of Wet Age-Related Macular Degeneration (wet AMD)
  - In January 2019, REGENXBIO announced that, based on an amendment to the Phase I protocol filed with the U.S. Food and Drug Administration (FDA), RGX-314 was cleared to proceed to a Phase IIa trial under the current Investigational New Drug (IND)

application. 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.
    - Dosing of an additional six subjects has been completed in Cohort 4, for a total of 12 subjects at a dose of 1.6x10^11 GC/eye.
    - A total of 30 subjects have been dosed in the RGX-314 Phase I/IIa trial. An additional cohort (12 subjects) at a dose of 2.5 x 10^11 GC/eye (Cohort 5) is currently recruiting.
    - REGENXBIO expects to present top-line data from the Phase I/IIa clinical trial by the end of 2019 and is on track to initiate a Phase IIb trial for wet AMD in late 2019.
    - REGENXBIO continues to advance development of RGX-314 in additional chronic retinal conditions that respond to anti-vascular endothelial growth factor (anti-VEGF) therapy; the company is on track to file a new IND for a Phase II trial in an additional retinal condition in the second half of 2019.
- RGX-314 Analyst and Investor Day
    - On February 21, 2019, REGENXBIO hosted an RGX-314 Analyst and Investor Day with leading retina specialists, who provided their perspectives on the potential advantages to one-time gene therapy, if approved, as a foundational anti-VEGF treatment for wet AMD and other retinal diseases. Key takeaways and opinions shared at the event included the following:
        - Real life patients lose vision over time due to an unsustainable treatment burden of current anti-VEGF injections;
        - Sustained treatment strategies that close the gap between randomized clinical trials and real-world outcomes are urgently needed; and
        - A one-time gene therapy has the potential to offer a significant benefit for many wet AMD patients, as consistent anti-VEGF expression offers the potential to sustain clinical outcomes while alleviating the treatment burden over the long-term.
    - Furthermore, the specialists shared the American Society of Retina Specialists (ASRS) 2018 Preferences and Trends (PAT) Membership Survey conducted with over one thousand respondents, which highlighted that the vast majority (88%) of retinal specialists are surgeons, and shared their opinion that they believe retinal specialists would be willing to incorporate a surgical procedure readily into their treatment strategy for wet AMD.
- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
    - As of December 31, 2018, one subject had been dosed in the first of two expected dose cohorts in the Phase I/II clinical trial evaluating RGX-121 for the treatment of MPS II. At the eight-week safety assessment, RGX-121 had been well-tolerated with no serious adverse events (SAEs) reported as of December 4, 2018. Additional recruitment and site activation are ongoing.
    - REGENXBIO expects to present an interim data update from the Phase I/II clinical trial evaluating RGX-121 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 approved protocol, recruitment is focused on an initial subject over 18 years of age.
    - REGENXBIO continues to work 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)
    - An amendment to the Phase I/II clinical trial protocol to allow for the enrollment of

additional subjects at the Cohort 2 dose using corticosteroid prophylaxis was submitted to the FDA and patient screening has resumed.
    - REGENXBIO expects to present interim data from Cohort 2 with corticosteroid prophylaxis from the Phase I/II clinical trial evaluating RGX-501 in the second half of 2019.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
    - REGENXBIO initiated IND-enabling studies for RGX-181 and expects to file an IND for the first-in-human clinical trial evaluating RGX-181 in the second half of 2019.
    - In November 2018, REGENXBIO announced that the FDA had granted Orphan Drug Designation to RGX-181 for the treatment of the CLN2 form of Batten disease. Additionally, in January 2019, REGENXBIO announced that the FDA had granted Rare Pediatric Disease Designation to RGX-181.

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. 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 100 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, including two clinical trials started in 2018 in hemophilia A sponsored by Takeda Pharmaceutical Company Limited and Bayer AG. Recent updates from NAV Technology Licensees include:

- In February 2019, Ultragenyx announced improved glucose control and increased time to hypoglycemia during fasting at 24 weeks in all three subjects in the first dose cohort of the Phase I/II clinical trial for DTX401 for the treatment of glycogen storage disease type Ia (GSDIa), with two subjects demonstrating a clinically meaningful improvement in time to hypoglycemia during a controlled fasting challenge. DTX401 uses the NAV AAV8 vector.
- In February 2019, Lysogene and Sarepta Therapeutics, Inc. announced that the first patient has been dosed in AAVance, a global Phase II/III clinical trial of LYS-SAF302 for the treatment of MPS IIIA. LYS-SAF302 uses the NAV AAVrh10 vector.
- In January 2019, Novartis announced that the company is 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. ZOLGENSMA uses the NAV AAV9 vector. 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.
- In January 2019, Audentes announced that optimal dose selection in the Phase I/II clinical trial for AT132 for the treatment of X-linked myotubular myopathy is expected to occur in the second quarter of 2019. Subsequent to the determination of the optimal dose, Audentes plans to provide an updated data package to FDA to facilitate final agreement on the path to BLA submission. AT132 uses the NAV AAV8 vector.
- In January 2019, Rocket Pharmaceuticals, Inc. announced clearance by the FDA of the IND for RP-A501 for the treatment of Danon disease. RP-A501 uses the NAV AAV9 vector.

Financial Results

Cash, cash equivalents and marketable securities were $470.6 million as of December 31, 2018, compared to $176.4 million as of December 31, 2017. Cash, cash equivalents and marketable securities as of December 31, 2018 include $180.0 million received in 2018 in connection with the amendment to the license agreement with AveXis, Inc. for the development and commercialization of treatments for SMA, as well as $189.1 million of aggregate net proceeds from a follow-on public offering of common stock completed in August 2018.

Revenues were $40.8 million and $218.5 million for the three months and year ended December 31, 2018, respectively, compared to $2.0 million and $10.4 million for the three months and year ended December 31, 2017, respectively. The increases in revenue were primarily attributable to $176.1 million of revenue recognized in 2018 under the amended license agreement with AveXis for the development and commercialization of treatments for SMA, as well as $35.6 million of revenue recognized in the fourth quarter of 2018 under the license agreement with Abeona for the development and commercialization of treatments for various diseases.

Research and development expenses were $24.3 million and $83.9 million for the three months and year ended December 31, 2018, respectively, compared to $14.2 million and $57.2 million for the three months and year ended December 31, 2017, respectively. The increases were primarily

attributable to personnel costs as a result of increased headcount, laboratory and facilities costs and expenses associated with conducting clinical trials and externally sourced manufacturing-related services.

General and administrative expenses were $11.1 million and $36.9 million for the three months and year ended December 31, 2018, respectively, compared to $4.8 million and $27.2 million for the three months and year ended December 31, 2017, respectively. The increases were primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services.

Net income was $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, compared to net losses of $16.0 million, or $0.51 basic and diluted net loss per share, and $73.2 million, or $2.45 basic and diluted net loss per share, for the three months and year ended December 31, 2017, respectively.

Financial Guidance

Based on its current operating plan, and excluding any commercial revenue from Novartis' ZOLGENSMA, subject to approval by regulatory authorities, 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. Importantly, REGENXBIO anticipates adding commercial revenue from ZOLGENSMA to its existing base of partner revenue this year, pending approval by regulatory authorities.

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 1564206. 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, 2018, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2019, 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, 2018	December 31, 2017
Assets		
Current assets		
Cash and cash equivalents	$ 75,561	$ 46,656
Marketable securities	244,200	114,122
Accounts receivable	8,587	473
Prepaid expenses	5,734	5,334
Other current assets	3,831	1,412
Total current assets	337,913	167,997
Marketable securities	150,819	15,616
Accounts receivable	23,012	—

Property and equipment, net	28,702	13,977
Restricted cash	1,053	225
Other assets	2,315	862
Total assets	$ 543,814	$ 198,677
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 4,412	$ 4,832
Accrued expenses and other current liabilities	17,164	9,605
Deferred revenue	600	—
Total current liabilities	22,176	14,437
Deferred revenue	3,333	—
Deferred rent, net of current portion	1,098	1,211
Financing lease obligation	5,854	—
Other liabilities	2,505	—
Total liabilities	34,966	15,648
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at December 31, 2018 and December 31, 2017	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at December 31, 2018 and December 31, 2017; 36,120 and 31,295 shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively	4	3
Additional paid-in capital	592,580	371,497
Accumulated other comprehensive loss	(720)	(715)
Accumulated deficit	(83,016)	(187,756)
Total stockholders' equity	508,848	183,029
Total liabilities and stockholders' equity	$ 543,814	$ 198,677

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,	
	2018	2017	2018	2017
Revenues				
License revenue	$ 40,777	$ 2,040	$ 218,505	$ 10,385
Other revenues	—	—	—	8
Total revenues	40,777	2,040	218,505	10,393
Operating Expenses				
Costs of revenues				
Licensing costs	2,843	(382)	9,640	1,703
Other	—	—	—	6
Research and development	24,329	14,170	83,873	57,224
General and administrative	11,144	4,808	36,850	27,229
Other operating expenses	11	42	42	116
Total operating expenses	38,327	18,638	130,405	86,278
Income (loss) from operations	2,450	(16,598)	88,100	(75,885)
Other Income				
Interest income from licensing	584	—	8,946	—
Investment income	2,893	601	7,070	2,716
Total other income	3,477	601	16,016	2,716
Income (loss) before income taxes	5,927	(15,997)	104,116	(73,169)
Income Tax Expense	(1,621)	—	(4,179)	—
Net income (loss)	$ 4,306	$ (15,997)	$ 99,937	$ (73,169)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	154	(161)	(5)	(682)
Total other comprehensive income (loss)	154	(161)	(5)	(682)
Comprehensive income (loss)	$ 4,460	$ (16,158)	$ 99,932	$ (73,851)
Net income (loss) per share:				
Basic	$ 0.12	$ (0.51)	$ 2.99	$ (2.45)
Diluted	$ 0.11	$ (0.51)	$ 2.73	$ (2.45)
Weighted-average common shares outstanding:				
Basic	35,951	31,178	33,427	29,878
Diluted	38,933	31,178	36,648	29,878

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