REGENXBIO Reports Third Quarter 2018 Financial and Operating Results and Announces Initiation of Phase I/II Clinical Trial for RGX-121 for the Treatment of MPS II

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- Announces first subject dosed in RGX-121 Phase I/II clinical trial for MPS II
- Completed Cohort 4 dosing in RGX-314 Phase I clinical trial for wet AMD
- Sustained RGX-314 protein levels at six months in Cohort 3 reported at AAO 2018
- Global submissions by Novartis of AVXS-101 for type 1 SMA using NAV AAV9; expected launch in mid-2019
- Announces 132,000 RSF build-to-suit lease for new corporate, manufacturing and research headquarters in Rockville, MD
- $475 million in cash, cash equivalents and marketable securities as of September 30, 2018
- Conference call Wednesday, November 7th at 4:30 p.m. ET

ROCKVILLE, Md., Nov. 7, 2018 /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 September 30, 2018 and recent operational highlights, including updates from its ongoing clinical trials.

"We are pleased to have recently treated the first subject in the Phase I/II study of RGX-121 from our neurodegenerative disease franchise and to have presented a continuing positive profile for our potential one-time treatment of RGX-314 for wet AMD, demonstrating sustained protein expression levels at six months. Furthermore, a major milestone was recently achieved through Novartis' announcement of the first worldwide marketing applications filed for a REGENXBIO NAV gene therapy product candidate," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "The adoption and application of NAV Technology continues to expand. As we near the end of 2018, our work and commitment to our mission intensifies. We are looking for 2019 to be another transformative year for REGENXBIO and one that will be uniquely marked by the start of more studies for innovative treatments, moving RGX-314 into later-stages of development, and the expected approval of AVXS-101 which has the potential to become an important new treatment for SMA type 1 patients. We are proud of the ongoing efforts and contributions by REGENXBIO and our partners focused on translating the hope of gene therapy into a reality for many patients and families."

Lead Product Candidate Updates

RGX-314 for the Treatment of Wet Age-Related Macular Degeneration (wet AMD)

- In September 2018, REGENXBIO announced the completion of dosing of an additional cohort (six subjects) at a higher dose of 1.6 x 10^11 genome copies (GC)/eye (Cohort 4) in the Phase I clinical trial of RGX-314 for the treatment of wet AMD. To date, 24 subjects have been treated with RGX-314.
- In October 2018, updated interim study results demonstrating that protein expression levels from the 6 x 10^10 GC/eye dose cohort (Cohort 3) in the Phase I clinical trial of RGX-314 for the treatment wet AMD were sustained at six months were presented by Jeffrey Heier, M.D., Co-President and Director of Retina Research at Ophthalmic Consultants of Boston, and primary investigator for the trial, during the Retina Subspecialty Day program at the American Association of Ophthalmology (AAO) 2018 Annual Meeting.
- REGENXBIO plans to proceed to a Phase II clinical trial as soon as possible and the next program updates are expected in early 2019.

RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)

- Today, REGENXBIO announced that the first subject was dosed at the Children's Hospital of Pittsburgh by a medical team led by Dr. Maria Escolar, Associate Professor of Pediatrics, Director of Program for the Study of Neurodevelopment in Rare Disorders at the Children's Hospital of Pittsburgh and primary investigator for the Phase I/II clinical trial of RGX-121 for the

treatment of MPS II. Additional subject recruitment and site activation continues. The next program updates are expected in 2019.

RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)

- In October 2018, updated interim results were presented by Dr. Marina Cuchel, Research Associate Professor of Medicine in the Institute for Translational Medicine and Therapeutics at the University of Pennsylvania Perelman School of Medicine, and primary investigator for the RGX-501 Phase I/II clinical trial for the treatment of HoFH, during a poster session at the American Society of Human Genetics 2018 Annual Meeting. The current clinical trial protocol is expected to be amended to enroll additional subjects using steroid prophylaxis and the next program updates are expected in early 2019.

RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)

- Subject recruitment continues in the Phase I clinical trial evaluating RGX-111 for the treatment of MPS I. Under the current U.S. Food and Drug Administration (FDA) protocol, recruitment is focused on an initial subject over 18 years of age. Today, REGENXBIO also announced that it has submitted an application to the Brazilian Health Surveillance Agency (ANVISA) to proceed with a Phase I clinical trial evaluating RGX-111 for treatment of MPS I in patients under the age of 3 which it expects will allow the company to activate an initial site in Brazil during the first half of 2019. Dosing of the first subject in a clinical trial evaluating RGX-111 is now expected to occur in the first half of 2019.

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

- In August 2018, REGENXBIO announced a new lead product candidate program called RGX-181. The program aims to develop a one-time treatment to halt progression of CLN2 disease, one of the most common forms of Batten disease, a rare, pediatric, neurodegenerative disease. REGENXBIO plans to advance RGX-181 to clinical trials with submission of an Investigational New Drug application expected in 2019.

Other Recent Operational Highlights

REGENXBIO's NAV Technology Platform is currently being applied in the development of more than 20 partnered product candidates by our NAV Technology Licensees. Fourteen of these partnered product candidates are in active clinical development and one partnered product candidate has been submitted for BLA approval. Recent highlights include:

- In November 2018, REGENXBIO and Abeona Therapeutics Inc. announced a license agreement for the development and commercialization of treatments for mucopolysaccharidosis type IIIA, mucopolysaccharidosis type IIIB, neuronal ceroid lipofuscinosis type 1, also known as infantile Batten disease, and neuronal ceroid lipofuscinosis type 3, also known as juvenile Batten disease, using the NAV AAV9 vector. REGENXBIO could receive up to $180 million under the license agreement, including a guaranteed $20 million upfront fee, $100 million in annual fees, $20 million of which is guaranteed, and potential commercial milestone payments of up to $60 million in addition to low double-digit royalties on net sales of products incorporating the licensed technology.

- In October 2018, Novartis AG announced the simultaneous global submissions in the United States, European Union and Japan for type 1 SMA based on the phase I data and select data from the ongoing phase III STR1VE study. Novartis is planning to launch AVXS-101 in the middle of 2019. AVXS-101 uses the NAV AAV9 vector.

- In October 2018, Audentes Therapeutics, Inc. announced an additional update to its positive

interim data from its Phase I/II clinical trial for AT132 for the treatment of X-linked myotubular myopathy, including that all treated patients continue to show meaningful improvements in neuromuscular and respiratory function, with no new treatment-related SAEs reported since the last scientific update in May 2018. AT132 uses the NAV AAV8 vector.

- In October 2018, Ultragenyx Pharmaceutical Inc. announced it has exercised its option with REGENXBIO to develop a gene therapy to treat patients with CDKL5 Deficiency Disorder utilizing the NAV Technology Platform, including AAV9.

Today, REGENXBIO announced that it has entered into a 132,000 RSF build-to-suit lease for a new office, laboratory and manufacturing headquarters in Rockville, Maryland. REGENXBIO will also have options on the remainder of the 175,000 square foot facility, to be constructed at 9800 Medical Center Drive by Alexandria Real Estate Equities. The project will provide REGENXBIO with space for collocated corporate headquarters, research and development and commercial-scale cGMP manufacturing. The facility is expected to be fully operational in 2020.

"The recent clinical progress and data presented across our internal and partnered development programs continues to reinforce the strength and benefits of our NAV Technology platform for patients," said Mr. Mills. "The relocation of our headquarters to 9800 Medical Center Drive will enable us to continue to meet our needs for growth in our talent and capabilities – including the potential for installing our own cGMP production facility – in order to support the development and launch of NAV Technology-based treatments. We are fortunate to secure a new location that keeps us in Rockville and supports our continued evolution as a company."

Management Update

Today, REGENXBIO announced that Stephen Yoo, M.D., REGENXBIO's Chief Medical Officer, has advised the company of his intent to resign on December 31, 2018 for personal reasons. REGENXBIO has initiated a retained search for this role. Dr. Yoo will continue to serve as a consultant to REGENXBIO during the transition.

"Since joining REGENXBIO over four years ago, Stephen has played an integral role in advancing our lead product candidates and leading our medical activities," said Mr. Mills. "Stephen has contributed to establishing REGENXBIO as a leader in gene therapy and we have confidence in the ability of the strong clinical team that he has assembled to continue to advance our product candidates. We wish Stephen all the best in his future endeavors."

Financial Results

Cash, cash equivalents and marketable securities were $474.9 million as of September 30, 2018, compared to $176.4 million as of December 31, 2017. Cash, cash equivalents and marketable securities as of September 30, 2018 include $180.0 million received in 2018 in connection with the amendment to our license agreement with AveXis for the development and commercialization of treatments for SMA, as well as $189.1 million of aggregate net proceeds from our follow-on public offering of common stock completed in August 2018.

Revenues were $5.3 million for the three months ended September 30, 2018, compared to $1.3 million for the three months ended September 30, 2017. The increase is primarily attributable to $4.0 million of license revenue recognized during the three months ended September 30, 2018 as a result of development milestones achieved, or deemed probable of achievement, by licensees during the period.

Research and development expenses were $18.5 million for the three months ended September 30, 2018, compared to $12.5 million for the three months ended September 30, 2017. The increase was 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 $9.0 million for the three months ended September 30, 2018, compared to $9.4 million for the three months ended September 30, 2017. The decrease was primarily attributable to a reduction in professional fees related to advisory services incurred in 2017 and was partially offset by an increase in personnel costs as a result of increased headcount.

Net loss was $19.2 million, or $0.56 basic and diluted net loss per share, for the three months ended September 30, 2018, compared to a net loss of $20.7 million, or $0.67 basic and diluted net loss per share, for the three months ended September 30, 2017.

Financial Guidance

Based on its current operating plan, REGENXBIO expects that its balance in cash, cash equivalents and marketable securities will be between $460 million and $470 million as of December 31, 2018, which will be used to support the continued development of its lead product candidate programs.

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 8283205. 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, 2017 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)

	September 30, 2018	December 31, 2017
Assets		
Current assets		
Cash and cash equivalents	$ 142,423	$ 46,656
Marketable securities	230,166	114,122
Accounts receivable	2,626	473
Prepaid expenses	6,308	5,334
Other current assets	4,834	1,412
Total current assets	386,357	167,997
Marketable securities	102,296	15,616
Accounts receivable	4,600	—
Property and equipment, net	19,856	13,977
Restricted cash	225	225
Other assets	1,650	862
Total assets	$ 514,984	$ 198,677
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 5,214	$ 4,832
Accrued expenses and other current liabilities	13,306	9,605
Deferred revenue	600	—
Total current liabilities	19,120	14,437
Deferred rent, net of current portion	1,116	1,211
Other liabilities	691	—
Total liabilities	20,927	15,648
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2018 and December 31, 2017	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at September 30, 2018 and December 31, 2017; 35,704 and 31,295 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	4	3
Additional paid-in capital	582,249	371,497
Accumulated other comprehensive loss	(874)	(715)
Accumulated deficit	(87,322)	(187,756)
Total stockholders' equity	494,057	183,029
Total liabilities and stockholders' equity	$ 514,984	$ 198,677

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenues				

License revenue	$ 5,306	$ 1,335	$ 177,728	$ 8,345
Other revenues	—	1	—	8
Total revenues	5,306	1,336	177,728	8,353
Expenses				
Costs of revenues				
Licensing costs	517	683	6,797	2,085
Other	—	—	—	6
Research and development	18,508	12,518	59,544	43,054
General and administrative	9,008	9,444	25,706	22,421
Other operating expenses (income)	(2)	—	31	74
Total operating expenses	28,031	22,645	92,078	67,640
Income (loss) from operations	(22,725)	(21,309)	85,650	(59,287)
Other Income				
Interest income from licensing	109	—	8,362	—
Investment income	2,122	603	4,177	2,115
Total other income	2,231	603	12,539	2,115
Income (loss) before income taxes	(20,494)	(20,706)	98,189	(57,172)
Income Tax Benefit (Expense)	1,292	—	(2,558)	—
Net income (loss)	$ (19,202)	$ (20,706)	$ 95,631	$ (57,172)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	(103)	93	(159)	(521)
Total other comprehensive income (loss)	(103)	93	(159)	(521)
Comprehensive income (loss)	$ (19,305)	$ (20,613)	$ 95,472	$ (57,693)
Net income (loss) applicable to common stockholders	$ (19,202)	$ (20,706)	$ 95,631	$ (57,172)
Net income (loss) per share:				
Basic	$ (0.56)	$ (0.67)	$ 2.94	$ (1.94)
Diluted	$ (0.56)	$ (0.67)	$ 2.67	$ (1.94)
Weighted-average common shares outstanding:				
Basic	33,988	30,940	32,576	29,440
Diluted	33,988	30,940	35,875	29,440

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