REGENXBIO Reports Fourth Quarter and Full-Year 2017 Financial Results and Recent Operational Highlights

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- *Completed dosing of third cohort in RGX-314 Phase I clinical trial for wet AMD*
- *Continuing dosing of second cohort in RGX-501 Phase I/II clinical trial for HoFH*
- *Anticipate presenting topline data from RGX-314 and RGX-501 clinical trials in late 2018*
- *Expect to initiate dosing in clinical trials for RGX-111 for MPS I and RGX-121 for MPS II in mid-2018*
- *$176 million in cash, cash equivalents and marketable securities as of December 31, 2017*

ROCKVILLE, Md., March 06, 2018 (GLOBE NEWSWIRE) -- 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, 2017 and recent operational highlights.

"In 2017, we significantly advanced our AAV gene therapy pipeline, which now consists of 12 active clinical stage programs, including four internal programs, as we seek to improve treatment options using our NAV Technology Platform," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We believe 2018 will be a transformative year for REGENXBIO, as we advance our mission of improving lives through the curative potential of gene therapy and expand our leading AAV gene therapy pipeline with our internal lead product candidates and our NAV Technology Licensees' programs. We look forward to providing updates on our lead product candidates and our NAV Technology Licensees' programs throughout the year, and are on track to report topline trial data for RGX-314 for wet AMD and RGX-501 for HoFH by year-end."

Recent Operational Highlights

- In February 2018, REGENXBIO announced the completion of dosing of the third cohort in the Phase I clinical trial for RGX-314 for the treatment of wet age-related macular degeneration (wet AMD). A total of 18 patients have been treated in the clinical trial to date. REGENXBIO expects to present topline data from the RGX-314 clinical trial in late 2018, which will include both primary and secondary endpoint data.
- In February 2018, REGENXBIO dosed the second patient in the second cohort, and fifth patient overall, with a single administration of RGX-501 in the Phase I/II clinical trial for the treatment of homozygous familial hypercholesterolemia (HoFH). REGENXBIO expects to present topline data from the RGX-501 clinical trial in late 2018, which will include both primary and secondary endpoint data.
- Site activation is continuing in the Phase I clinical trial evaluating RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I). Patient recruitment is anticipated to begin in the first quarter of 2018, with the first patient expected to be dosed in mid-2018.
- Site activation is continuing in the Phase I/II clinical trial evaluating RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II). Patient recruitment is anticipated to begin in the first quarter of 2018, with the first patient expected to be dosed in mid-2018.
- In January 2018, REGENXBIO and AveXis, Inc. announced an amendment to their license agreement for the development and commercialization of treatments for spinal muscular atrophy (SMA). Under the terms of the amended agreement, REGENXBIO could receive up to $260 million, including an upfront payment of $80 million, $60 million in additional guaranteed annual payments and potential commercial milestone payments of up to $120 million, and AveXis acquired exclusive rights to the entire NAV Technology Platform for the development of treatments for SMA. Additionally, the amended agreement permits assignment by AveXis upon a change of control without REGENXBIO's consent.

- In January 2018, REGENXBIO entered into an agreement with FUJIFILM Diosynth Biotechnologies which secures access to dedicated cGMP suite capacity and resources capable of manufacturing REGENXBIO's lead product candidates at up to 2,000L scale in support of global development and commercialization.

As of December 31, 2017, REGENXBIO's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by 10 NAV Technology Licensees. Eight of these partnered product candidates are in active clinical development. Three recent updates from NAV Technology Licensee active clinical programs include:

- In January 2018, AveXis announced that it will initiate screening for remaining patients in the pivotal trial of AVXS-101 for SMA Type I following review of preliminary data from the first three patients. AVXS-101 uses the NAV AAV9 vector.
- In January 2018, Audentes Therapeutics, Inc. announced positive interim data from the first dose cohort in the Phase I/II clinical trial evaluating AT132 for the treatment of X-linked myotubular myopathy. AT132 uses the NAV AAV8 vector.
- In February 2018, Audentes Therapeutics announced dosing of the first patient in the Phase I/II clinical trial evaluating AT342 for the treatment of Crigler-Najjar Syndrome. AT342 uses the NAV AAV8 vector.

Financial Results

Cash, cash equivalents and marketable securities were $176.4 million as of December 31, 2017, compared to $159.0 million as of December 31, 2016. Cash, cash equivalents and marketable securities as of December 31, 2017 exclude the $80 million received from AveXis in connection with the previously announced amendment to the license agreement in January 2018 for the development and commercialization of treatments for SMA.

Revenues were $2.0 million and $10.4 million for the three months and year ended December 31, 2017, respectively, compared to $1.7 million and $4.6 million for the three months and year ended December 31, 2016, respectively.

Total operating expenses were $18.6 million and $86.3 million for the three months and year ended December 31, 2017, respectively, compared to $22.2 million and $69.9 million for the three months and year ended December 31, 2016, respectively.

Net loss was $16.0 million and $73.2 million, or $0.51 and $2.45 net loss per basic and diluted common share, for the three months and year ended December 31, 2017, respectively, compared to $19.6 million and $63.0 million, or $0.74 and $2.38 net loss per basic and diluted share, for the three months and year ended December 31, 2016, respectively.

Financial Guidance

REGENXBIO reiterates that it expects full-year 2018 cash burn to be between $85 million and $95 million, which will support the continued development of its lead product candidate programs. Full-year 2018 cash burn guidance excludes the effect of the upfront payment of $80 million and any other potential consideration that may be received from AveXis in connection with the previously announced amendment to the license agreement in January 2018 for the development and commercialization of treatments for SMA. Subject to this exclusion, full-year 2018 cash burn will be measured as the decrease in cash, cash equivalents and marketable securities from December 31, 2017 to December 31, 2018.

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 of REGENXBIO's clinical trials; the timing and success of preclinical studies and clinical trials 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, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2018, 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, 2017	December 31, 2016
Assets		
Current assets		
Cash and cash equivalents	$ 46,656	$ 24,840
Marketable securities	114,122	64,714
Accounts receivable	473	1,032
Prepaid expenses	5,334	1,775
Other current assets	1,412	1,010
Total current assets	167,997	93,371
Marketable securities	15,616	69,412
Property and equipment, net	13,977	9,324
Restricted cash	225	225
Other assets	862	400
Total assets	$ 198,677	$ 172,732
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 4,832	$ 1,543
Accrued expenses and other current liabilities	9,605	8,126
Total current liabilities	14,437	9,669
Deferred rent, net of current portion	1,211	1,326
Total liabilities	15,648	10,995
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at December 31, 2017 and December 31, 2016	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at December 31, 2017 and December 31, 2016; 31,295 and 26,477 shares issued and outstanding at December 31, 2017 and December 31, 2016, respectively	3	3
Additional paid-in capital	371,497	276,354
Accumulated other comprehensive loss	(715)	(33)
Accumulated deficit	(187,756)	(114,587)
Total stockholders' equity	183,029	161,737
Total liabilities and stockholders' equity	$ 198,677	$ 172,732

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

	Three Months Ended December 31,		Years Ended December 31,	
	2017	2016	2017	2016
Revenues				
License revenue	$ 2,040	$ 1,665	$ 10,385	$ 4,303
Reagent sales	—	—	—	213

Grant revenue	—	31	8	73
Total revenues	2,040	1,696	10,393	4,589
Expenses				
Costs of revenues				
Licensing costs	(382)	330	1,703	861
Costs of reagent sales	—	—	6	98
Research and development	14,170	16,059	57,224	45,482
General and administrative	4,808	5,742	27,229	23,590
Other operating expenses (income)	42	34	116	(102)
Total operating expenses	18,638	22,165	86,278	69,929
Loss from operations	(16,598)	(20,469)	(75,885)	(65,340)
Other Income				
Investment income	601	426	2,716	1,938
Total other income	601	426	2,716	1,938
Loss before income taxes	(15,997)	(20,043)	(73,169)	(63,402)
Income Tax Benefit	—	435	—	435
Net loss	$ (15,997)	$ (19,608)	$ (73,169)	$ (62,967)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	(161)	(886)	(682)	686
Total other comprehensive income (loss)	(161)	(886)	(682)	686
Comprehensive loss	$ (16,158)	$ (20,494)	$ (73,851)	$ (62,281)
Basic and diluted net loss per common share	$ (0.51)	$ (0.74)	$ (2.45)	$ (2.38)
Weighted-average basic and diluted common shares	31,178	26,476	29,878	26,409

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