



REGENXBIO Reports First Quarter 2017 Financial Results and Recent Operational Highlights

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- *On track to initiate dosing of patients in RGX-314 Phase I trial for wet AMD by mid-2017 and continuing enrollment of patients in RGX-501 Phase I/II trial for HoFH*
- *Interim trial updates for RGX-314 and RGX-501 anticipated by year-end 2017*
- *\$210 million in cash, cash equivalents and marketable securities as of March 31, 2017*

ROCKVILLE, Md., May 09, 2017 (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 first quarter ended March 31, 2017 and recent operational highlights.

"We have had a productive start to 2017 in advancing the development of our lead product candidates, RGX-314 for wet AMD and RGX-501 for HoFH. Additionally, we enhanced and expanded our team with the appointment of Dr. Olivier Danos as Chief Scientific Officer," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "For the remainder of the year we will continue to focus our energy on achieving our upcoming clinical milestones and building a robust clinical pipeline of gene therapy product candidates with the goal of improving treatment options in many diseases. We look forward to sharing interim updates in the RGX-314 Phase I trial for wet AMD and the RGX-501 Phase I/II trial for HoFH by year-end."

Recent Operational Highlights

- In February 2017, REGENXBIO announced that the Investigational New Drug application (IND) for RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) is active. Site activation is underway at six leading retinal surgery centers in the United States. RGX-314 has been released to sites to support dosing the first patient in the RGX-314 Phase I clinical trial by mid-2017 and REGENXBIO is on track to provide an interim update by the end of 2017.
- In March 2017, dosing was initiated for the Phase I/II clinical trial evaluating RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH). Patient recruitment and enrollment is continuing and REGENXBIO is on track to provide an interim update from the RGX-501 clinical trial in late 2017.
- REGENXBIO plans to file an IND for the Phase I/II clinical trial of RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I) in mid-2017. Enrollment in the RGX-111 clinical trial is on track to commence in the second half of 2017.
- REGENXBIO plans to file an IND for RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) in mid-2017, once the IND for the Phase I/II clinical trial of RGX-111 is active.
- In March 2017, REGENXBIO further strengthened its management team with the appointment of Olivier Danos, Ph.D., as Chief Scientific Officer. Dr. Danos brings significant expertise in the gene therapy industry, most recently serving as Senior Vice President, Cell and Gene Therapy, at Biogen Inc.
- As of March 31, 2017, REGENXBIO's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by nine NAV Technology Licensees. Seven of these partnered product candidates have advanced to clinical stages of

development. Since March 31, 2017, two NAV Technology Licensees have provided updates on active clinical programs:

- AveXis, Inc. reported additional Phase I clinical data on AVXS-101 for the treatment of spinal muscular atrophy (SMA) Type 1 as presented at the 2017 Annual Meeting of the American Academy of Neurology, demonstrating a sustained efficacy and safety profile. AveXis, Inc. anticipates initiating the pivotal trial of AVXS-101 in the U.S. in the second quarter of 2017. AVXS-101 uses the NAV AAV9 vector.
- Audentes Therapeutics, Inc. announced that the IND is active for the Phase I/II clinical trial of AT132 for the treatment of X-linked Myotubular Myopathy and that preliminary data are expected to be available by the end of 2017. AT132 uses the NAV AAV8 vector.

Financial Results

Cash, cash equivalents and marketable securities were \$209.6 million as of March 31, 2017, compared to \$159.0 million as of December 31, 2016.

Revenues were \$0.5 million for the three months ended March 31, 2017, compared to \$0.4 million for the three months ended March 31, 2016.

Total operating expenses were \$23.4 million for the three months ended March 31, 2017, compared to \$11.6 million for the three months ended March 31, 2016.

Net loss was \$22.0 million, or \$0.82 net loss per basic and diluted common share, for the three months ended March 31, 2017, compared to \$10.8 million, or \$0.41 net loss per basic and diluted share, for the three months ended March 31, 2016.

Financial Guidance

REGENXBIO reiterates that it expects full-year 2017 cash burn to be between \$75 million and \$85 million, which will support the continued development of its lead product candidate programs. Full-year 2017 cash burn guidance excludes the effect of REGENXBIO's previously announced underwritten public offering of common stock in March 2017 and the underwriters' exercise of their option to purchase additional shares in April 2017, which resulted in aggregate net proceeds to REGENXBIO of approximately \$81.5 million, after deducting underwriting discounts and commissions and estimated offering expenses.

Conference Call Information

In connection with the earnings release, REGENXBIO will host a conference call 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 13743533. To access a live or recorded webcast of the call, 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 Licensees are applying the NAV Technology Platform in the development of a broad pipeline of product candidates in multiple therapeutic areas.

Forward Looking Statements

This press release contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995, regarding, among other things, REGENXBIO's research, development and regulatory plans in connection with its NAV Technology Platform and gene therapy treatments. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could cause actual results to differ materially from those projected by such forward-looking statements. All of REGENXBIO's development timelines could be subject to adjustment depending on recruitment rate, regulatory agency review and other factors that could delay the initiation and completion of clinical trials. Meaningful factors which could cause actual results to differ include, but are not limited to, 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 ability to obtain and maintain regulatory approval of REGENXBIO's product candidates, and the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing REGENXBIO's product candidates; REGENXBIO's ability to obtain and maintain intellectual property protection for REGENXBIO's product candidates and technology; REGENXBIO's growth strategies; REGENXBIO's competition; trends and challenges in REGENXBIO's business and the markets in which REGENXBIO operates; REGENXBIO's ability to attract or retain key personnel; the size and growth of the potential markets for REGENXBIO's product candidates and the ability to serve those markets; the rate and degree of market acceptance of any of REGENXBIO's product candidates; REGENXBIO's ability to establish and maintain development partnerships; REGENXBIO's expenses and revenue; regulatory developments in the United States and foreign countries; the sufficiency of REGENXBIO's cash resources and needs for additional financing; and other factors discussed in 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. Additional factors may be set forth in those sections of REGENXBIO's Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, to be filed with the Securities and Exchange Commission (SEC) in the second quarter of 2017. In addition to the risks described above and in REGENXBIO's filings with the SEC, other unknown or unpredictable factors also could affect REGENXBIO's results. There can be no assurance that the actual results or developments anticipated by REGENXBIO will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, REGENXBIO. Therefore, no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

All forward-looking statements contained in this press release are expressly qualified by the cautionary statements contained or referred to herein. REGENXBIO cautions investors not to rely too heavily on the forward-looking statements REGENXBIO makes or that are made on its behalf.

These forward-looking statements speak only as of the date of this press release (unless another date is indicated). REGENXBIO undertakes no obligation, and specifically declines any obligation, to publicly update or revise any such forward-looking statements, whether as a result of new information, future events or otherwise.

REGENXBIO INC.
BALANCE SHEETS
(unaudited)
(in thousands)

	<u>March 31,</u> <u>2017</u>	<u>December 31,</u> <u>2016</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 82,045	\$ 24,840
Marketable securities	63,764	64,714
Accounts receivable	228	1,032
Prepaid expenses	1,843	1,775
Other current assets	922	1,010
Total current assets	148,802	93,371
Marketable securities	63,742	69,412
Property and equipment, net	11,061	9,324
Restricted cash	225	225
Other assets	297	400
Total assets	<u>\$ 224,127</u>	<u>\$ 172,732</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 4,129	\$ 1,543
Accrued expenses and other current liabilities	5,829	8,126
Total current liabilities	9,958	9,669
Deferred rent, net of current portion	1,271	1,326
Total liabilities	11,229	10,995
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2017 and December 31, 2016	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at March 31, 2017 and December 31, 2016; 30,244 and 26,477 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	3	3
Additional paid-in capital	350,047	276,354
Accumulated other comprehensive loss	(572)	(33)
Accumulated deficit	(136,580)	(114,587)
Total stockholders' equity	212,898	161,737
Total liabilities and stockholders' equity	<u>\$ 224,127</u>	<u>\$ 172,732</u>

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

	<u>Three Months Ended March</u> <u>31,</u>	
	<u>2017</u>	<u>2016</u>
Revenues		
License revenue	\$ 455	\$ 328
Reagent sales	—	59

Grant revenue	—	6
Total revenues	<u>455</u>	<u>393</u>
Expenses		
Costs of revenues		
Licensing costs	91	66
Costs of reagent sales	—	30
Research and development	16,619	6,183
General and administrative	6,622	5,479
Other operating expenses (income)	<u>45</u>	<u>(114)</u>
Total operating expenses	<u>23,377</u>	<u>11,644</u>
Loss from operations	(22,922)	(11,251)
Other Income		
Investment income	<u>929</u>	<u>483</u>
Total other income	<u>929</u>	<u>483</u>
Net loss	<u>\$ (21,993)</u>	<u>\$ (10,768)</u>
Other Comprehensive Income (Loss)		
Unrealized gain (loss) on available-for-sale securities, net of reclassifications of \$480 for the three months ended March 31, 2017	<u>(539)</u>	<u>994</u>
Total other comprehensive income (loss)	<u>(539)</u>	<u>994</u>
Comprehensive loss	<u>\$ (22,532)</u>	<u>\$ (9,774)</u>
Basic and diluted net loss per common share	<u>\$ (0.82)</u>	<u>\$ (0.41)</u>
Weighted-average basic and diluted common shares	<u>26,673</u>	<u>26,327</u>

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