



## **REGENXBIO Reports Third Quarter 2017 Financial Results and Recent Operational Highlights**

November 8, 2017 9:07 PM EST

- *Completed second cohort dosing in RGX-314 Phase I clinical trial for wet AMD*
- *Completed first cohort dosing in RGX-501 Phase I/II clinical trial for HoFH*
- *Interim trial updates to be included in the Company's year-end 2017 corporate update scheduled to be released during the first week of January 2018*
- *Initiated build-out of new 15,000 square foot, state-of-the-art Research and Development facility in Rockville, MD*
- *\$191 million in cash, cash equivalents and marketable securities as of September 30, 2017*

ROCKVILLE, Md., Nov. 08, 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<sup>®</sup> Technology Platform, today announced financial results for the third quarter ended September 30, 2017 and recent operational highlights.

"We continue to make meaningful progress in the clinical development of our lead product candidates, most notably with the recent completion of dosing of the second cohort in our Phase I clinical trial evaluating RGX-314 for the treatment of wet AMD and completion of dosing in the first cohort in our Phase I/II clinical trial evaluating RGX-501 for the treatment of HoFH," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We remain committed to advancing our internal and partnered gene therapy product candidates, leveraging our proprietary NAV Technology Platform to evaluate the curative potential of one-time administrations of gene therapy for patients with significant unmet need. We look forward to providing additional trial details on our lead product candidates, including interim updates from the RGX-314 Phase I clinical trial and the RGX-501 Phase I/II clinical trial, as part of our year-end 2017 corporate update."

### **Recent Operational Highlights**

- REGENXBIO completed dosing of the second cohort in the Phase I clinical trial evaluating RGX-314 for the treatment of wet age-related macular degeneration (wet AMD). A total of 12 patients, from four different sites, have now been treated with RGX-314 in the Phase I clinical trial. Based on the 12 patients dosed, we are encouraged by the preliminary safety and tolerability profile observed with RGX-314. Following a scheduled review by an independent Data Safety and Monitoring Board (DSMB), the Company expects to initiate dosing of the third cohort in the Phase I clinical trial by the first quarter of 2018. REGENXBIO expects to provide further details regarding the RGX-314 clinical trial, including updates on enrollment, procedural implementation and preliminary safety and tolerability, in the Company's year-end 2017 corporate update scheduled to be released during the first week of January 2018.
- Dosing of the first cohort of three patients was completed in the Phase I/II clinical trial evaluating RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH). Review of data from the first cohort has been conducted by an independent DSMB, who has granted clearance to proceed to the next dosing cohort based on their assessment of the safety and tolerability data. The Company anticipates dosing of the second cohort in the trial to be initiated prior to year-end 2017. REGENXBIO expects to provide further details regarding the RGX-501 clinical trial, including updates on enrollment and preliminary safety and tolerability, in the Company's year-end 2017 corporate update.
- REGENXBIO's Investigational New Drug (IND) application is now active for the Phase I clinical trial of RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I). Site activation in

the planned multi-center, open-label, multiple-cohort, dose-escalation trial is underway to support recruitment and patient enrollment, with the first patient expected to be dosed in the first half of 2018.

- REGENXBIO plans to file an IND application for RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) in the fourth quarter of 2017, which will incorporate feedback received from the U.S. Food and Drug Administration (FDA) in its review of the IND application for RGX-111.
- In September 2017, REGENXBIO further strengthened its management team with the appointment of Shiva Fritsch as Senior Vice President, Human Resources. Ms. Fritsch brings significant expertise in the biotechnology industry, previously serving in human resources leadership roles at Human Genome Sciences, Inc., the Howard Hughes Medical Institute and, most recently, at Novavax, Inc.
- In October 2017, REGENXBIO initiated the build-out of a state-of-the-art Research and Development facility and currently expects to complete the build-out in 2018. The new 15,000 square foot facility supports the expansion of REGENXBIO's internal gene therapy research capabilities for the creation of new gene therapy technologies and the origination of new lead product candidates and will be adjacent to REGENXBIO's advanced manufacturing and analytics lab located in Rockville, Maryland.
- As of September 30, 2017, REGENXBIO's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by 10 NAV Technology Licensees. Seven of these partnered product candidates are in active clinical development.
  - In October 2017, Shire plc announced the FDA had awarded Orphan Drug Designation to its gene therapy candidate SHP654 for the treatment of hemophilia A. SHP654 uses the NAV AAV8 vector.
  - In September 2017, AveXis, Inc. announced that it has commenced the pivotal trial of AVXS-101 for the treatment of spinal muscular atrophy (SMA) Type 1. AVXS-101 uses the NAV AAV9 vector.
  - Also in September 2017, Audentes Therapeutics, Inc. announced dosing of the first patient in the Phase I/II clinical trial evaluating AT132 for the treatment of X-linked myotubular myopathy. AT132 uses the NAV AAV8 vector.
  - In August 2017, Dimension Therapeutics, Inc., which was subsequently acquired by Ultragenyx Pharmaceutical Inc., announced dosing of the first patient in the Phase I/II clinical trial of DTX301 for the treatment of ornithine transcarbamylase (OTC) deficiency. DTX301 uses the NAV AAV8 vector.

#### **Financial Results**

Cash, cash equivalents and marketable securities were \$191.1 million as of September 30, 2017, compared to \$159.0 million as of December 31, 2016.

Revenues were \$1.3 million for the three months ended September 30, 2017, compared to \$0.1 million for the three months ended September 30, 2016.

Total operating expenses were \$22.6 million for the three months ended September 30, 2017, compared to \$18.8 million for the three months ended September 30, 2016.

Net loss was \$20.7 million, or \$0.67 net loss per basic and diluted common share, for the three months ended September 30, 2017, compared to \$18.2 million, or \$0.69 net loss per basic and diluted share, for the three months ended September 30, 2016.

## Financial Guidance

REGENXBIO continues to expect full-year 2017 cash burn to be between \$75 million and \$80 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, which resulted in aggregate net proceeds to REGENXBIO of approximately \$81.5 million, after deducting underwriting discounts and commissions and 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 96009722. To access a live or recorded webcast of the call, please visit the "Investors" section of the REGENXBIO website at [www.regenxbio.com](http://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<sup>®</sup> 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, statements about REGENXBIO's future operations, 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, 2016 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](http://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)**

	<u>September 30, 2017</u>	<u>December 31, 2016</u>
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 59,884	\$ 24,840
Marketable securities	106,778	64,714
Accounts receivable	884	1,032
Prepaid expenses	3,225	1,775
Other current assets	1,390	1,010
Total current assets	<u>172,161</u>	<u>93,371</u>
Marketable securities	24,485	69,412
Property and equipment, net	11,548	9,324
Restricted cash	225	225
Other assets	836	400
Total assets	<u>\$ 209,255</u>	<u>\$ 172,732</u>

**Liabilities and Stockholders' Equity**

## Current liabilities

Accounts payable	\$	2,594	\$	1,543
Accrued expenses and other current liabilities		10,805		8,126
Total current liabilities		13,399		9,669

## Deferred rent, net of current portion

		1,205		1,326
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## Total liabilities

		14,604		10,995
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## Stockholders' equity

Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2017 and December 31, 2016

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Common stock; \$0.0001 par value; 100,000 shares authorized at September 30, 2017 and December 31, 2016; 31,109 and 26,477 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively

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Additional paid-in capital

		366,960		276,354
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Accumulated other comprehensive loss

		(553)		(33)
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Accumulated deficit

		(171,759)		(114,587)
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Total stockholders' equity

		194,651		161,737
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Total liabilities and stockholders' equity

	\$	209,255	\$	172,732
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**REGENXBIO INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
(unaudited)  
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
<b>Revenues</b>				
License revenue	\$ 1,335	\$ 65	\$ 8,345	\$ 2,638
Reagent sales	—	47	—	213
Grant revenue	1	13	8	42
Total revenues	1,336	125	8,353	2,893
<b>Expenses</b>				
Costs of revenues				
Licensing costs	683	13	2,085	528
Costs of reagent sales	—	22	6	101
Research and development	12,518	12,560	43,054	29,423
General and administrative	9,444	6,200	22,421	17,848
Other operating expenses (income)	—	(2)	74	(136)
Total operating expenses	22,645	18,793	67,640	47,764
Loss from operations	(21,309)	(18,668)	(59,287)	(44,871)
<b>Other Income</b>				
Investment income	603	514	2,115	1,512
Total other income	603	514	2,115	1,512
Net loss	\$ (20,706)	\$ (18,154)	\$ (57,172)	\$ (43,359)
<b>Other Comprehensive Income (Loss)</b>				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications of \$479 and \$20 for the nine months ended September 30, 2017 and 2016, respectively	93	332	(521)	1,572
Total other comprehensive income (loss)	93	332	(521)	1,572
Comprehensive loss	\$ (20,613)	\$ (17,822)	\$ (57,693)	\$ (41,787)
Basic and diluted net loss per common share	\$ (0.67)	\$ (0.69)	\$ (1.94)	\$ (1.64)

Weighted-average basic and diluted common shares

30,940      26,469      29,440      26,386

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REGENXBIO Inc.