REGENXBIO Reports Third Quarter 2016 Financial Results and Recent Operational Highlights

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- *On track to dose first patient for Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia by the end of 2016*
- *Initiated manufacturing of material for Phase I clinical trial of RGX-314 for the treatment of wet age-related macular degeneration; IND filing planned for early 2017*
- *Anticipate completion of new, advanced manufacturing and analytics lab by the end of 2016*
- *$184.9 million in cash, cash equivalents and marketable securities as of September 30, 2016*

ROCKVILLE, Md., Nov. 09, 2016 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (Nasdaq:RGNX), a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated (AAV) gene therapy based on its proprietary NAV® Technology Platform (NAV), today announced financial results for the quarter ended September 30, 2016 and recent operational highlights.

“During the third quarter, we continued to advance our lead programs, with ongoing patient screening for the Phase I/II clinical trial of RGX-501, and the initiation of manufacturing in preparation for the Phase I clinical trial of RGX-314,” said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. “In addition, we made steady progress in expanding our capabilities with the establishment of an advanced manufacturing and analytics lab, and we continued to see validation of our NAV gene therapy pipeline with the publication of positive preclinical data in neurodegenerative diseases and compelling results from our NAV licensees, including what we believe to be promising interim Phase I data and FDA support of a single-arm pivotal study design for AveXis’ AVXS-101, which uses the NAV AAV9 vector to treat spinal muscular atrophy.”

Recent Operational Highlights

- REGENXBIO and trial sponsor the University of Pennsylvania continue to screen and schedule eligible patients for dosing in the Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH). REGENXBIO anticipates dosing the first patient by the end of 2016.
- REGENXBIO initiated the manufacturing of material for the anticipated Phase I clinical trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD). Preclinical studies of RGX-314 are nearing completion, and REGENXBIO plans to file an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) in the first quarter of 2017.
- In July 2016, preclinical data on RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I) were published in Molecular Genetics and Metabolism. The data demonstrate disease correction from a single administration of NAV AAV9 in a canine model, and is expected to inform the minimum effective dose for planned human studies. REGENXBIO intends to file an IND with the FDA and a Clinical Trial Application (CTA) with Health Canada for a Phase I/II clinical trial of RGX-111 in the first half of 2017.
- In August 2016, preclinical data on RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) were published in Human Gene Therapy. RGX-121 demonstrated disease correction and improvement in evaluable long-term memory from a single administration of

NAV AAV9 in a mouse model. REGENXBIO plans to file an IND with the FDA for a Phase I/II clinical trial of RGX-121 in the first half of 2017.

- REGENXBIO initiated and currently expects to complete build-out of an advanced manufacturing and analytics lab by the end of 2016.

- REGENXBIO strengthened its leadership team with the appointment of two new members to its board of directors and the addition of a key executive:

    - Daniel Abdun-Nabi was appointed to the board of directors. Mr. Abdun-Nabi brings more than 25 years of success in key strategic, operational, legal and board roles to REGENXBIO, including his current role as President and Chief Executive Officer of Emergent BioSolutions.

    - Daniel Tassé was appointed to the board of directors. Mr. Tassé has more than 20 years of biopharmaceutical leadership experience, including his current role as Chairman and Chief Executive Officer of Alcresta Therapeutics.

    - Patrick Christmas was named Senior Vice President, General Counsel. Mr. Christmas brings extensive biopharmaceutical industry experience to REGENXBIO, having previously served as General Counsel at Lumara Health and the Wellstat Group of Companies.

- In November 2016, REGENXBIO NAV licensee AveXis, Inc. announced that, based on its receipt of the minutes following a Type B meeting with the FDA, AveXis' planned pivotal study of AVXS-101 in spinal muscular atrophy Type 1 will reflect a single-arm design. Additionally, AveXis announced that the FDA strongly recommended that AveXis request an end-of-Phase I meeting at the completion of its Phase I study of AVXS-101, which is expected to occur in the first half of 2017, that would include a discussion of whether the data from the Phase I study might provide the substantial evidence necessary to support a marketing application.

Financial Results

- Cash, cash equivalents and marketable securities as of September 30, 2016 were $184.9 million, compared to $216.4 million as of December 31, 2015.

- Revenues were $0.1 million for the quarter ended September 30, 2016, compared to $1.1 million for the quarter ended September 30, 2015.

- Total operating expenses were $18.8 million for the quarter ended September 30, 2016, compared to $8.5 million for the quarter ended September 30, 2015.

- Net loss was $18.2 million, or $0.69 net loss per basic and diluted common share, for the quarter ended September 30, 2016, compared to $7.3 million, or $1.52 net loss per basic and diluted share, for the quarter ended September 30, 2015.

Financial Guidance

- REGENXBIO updated its expected full-year 2016 cash burn (change in cash, cash equivalents and marketable securities) guidance to between $55 million and $60 million from previous guidance of between $60 million and $70 million.

About REGENXBIO

REGENXBIO is a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. REGENXBIO's NAV® Technology Platform, a proprietary AAV gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO's mission is to transform the lives of patients suffering from severe diseases with significant unmet medical need by developing and commercializing in vivo gene therapy products based on REGENXBIO's NAV Technology Platform. REGENXBIO seeks to accomplish this mission through a combination of internal development efforts and third-party NAV Technology Platform licensees. As of September 30, 2016, REGENXBIO's NAV Technology Platform was being applied in the development of 29 product candidates for a variety of diseases, including five internally developed candidates and 24 partnered candidates developed by REGENXBIO's licensees.

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 for RGX-111, RGX-121, RGX-314, RGX-501 and other gene therapies and the research, development and regulatory plans of REGENXBIO's NAV Technology Licensees. 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, its development partners and its NAV Technology Licensees; the ability to obtain and maintain regulatory approval to conduct clinical trials and to commercialize 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; 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, including those with NAV Technology Licensees; REGENXBIO's expenses and revenue, the sufficiency of REGENXBIO's cash resources and needs for additional financing, regulatory developments in the United States and foreign countries, as well as 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, 2015 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, which are available on the SEC's website at www.sec.gov. Additional factors may be set forth in those sections of REGENXBIO's Form 10-Q for the quarter ended September 30, 2016, to be filed with the SEC in the fourth quarter of 2016. 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)

	September 30, 2016	December 31, 2015
Assets		
Current assets		
Cash and cash equivalents	$ 28,108	$ 54,116
Marketable securities	63,662	60,025
Accounts receivable	679	2,136
Prepaid expenses	2,171	1,020
Other current assets	2,000	851
Total current assets	96,620	118,148
Marketable securities	93,087	102,226
Property and equipment, net	5,804	538
Cost method investments	—	300
Restricted cash	225	—
Other assets	239	168
Total assets	$ 195,975	$ 221,380
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 5,376	$ 1,014
Accrued expenses and other current liabilities	9,006	3,198
Advance payments	—	127

Total current liabilities	14,382	4,339
Deferred rent, net of current portion	1,367	233
Total liabilities	15,749	4,572
Stockholders' equity	180,226	216,808
Total liabilities and stockholders' equity	$ 195,975	$ 221,380

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Revenues				
License revenue	$ 65	$ 65	$ 2,638	$ 635
License revenue from related party	—	1,000	—	2,000
Reagent sales	47	61	213	200
Grant revenue	13	14	42	305
Total revenues	125	1,140	2,893	3,140
Expenses				
Costs of revenues				
Licensing costs (including amounts to related parties)	13	213	528	527
Costs of reagent sales (including amounts to related parties)	22	44	101	94
Research and development (including amounts to related parties)	12,560	5,664	29,423	12,471
General and administrative (including amounts to related parties)	6,200	2,567	17,848	7,671
Other operating expenses (income)	(2)	(1)	(136)	15
Total operating expenses	18,793	8,487	47,764	20,778
Loss from operations	(18,668)	(7,347)	(44,871)	(17,638)
Other Income (Expense)				
Investment income	514	15	1,512	23
Interest expense	—	—	—	(20)
Total other income (expense)	514	15	1,512	3
Net loss	$ (18,154)	$ (7,332)	$ (43,359)	$ (17,635)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities	332	(26)	1,572	(26)
Total other comprehensive income (loss)	332	(26)	1,572	(26)
Comprehensive loss	$ (17,822)	$ (7,358)	$ (41,787)	$ (17,661)
Reconciliation of net loss to net loss applicable to common stockholders				
Net loss	$ (18,154)	$ (7,332)	$ (43,359)	$ (17,635)
Net accretion and dividends on convertible preferred stock	—	—	—	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders	$ (18,154)	$ (7,332)	$ (43,359)	$ (18,623)
Basic and diluted net loss per common share	$ (0.69)	$ (1.52)	$ (1.64)	$ (5.48)
Weighted-average basic and diluted common shares	26,469	4,809	26,386	3,397

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