REGENXBIO Reports Second Quarter 2016 Financial Results and Recent Operational Highlights

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- *On track to enroll first patients for Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia in the second half of 2016*
- *Received FDA Rare Pediatric Disease Designation for RGX-121 for the treatment of Mucopolysaccharidosis Type II*
- *Entered into an exclusive license agreement with Biogen for the development of gene therapies for rare genetic vision disorders*
- *$198.7 million in cash, cash equivalents and marketable securities as of June 30, 2016*

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

“This quarter has seen great progress in the advancement and expansion of the pipeline of gene therapies based on our NAV Technology Platform through our internal development efforts and the activities of our NAV Technology Licensees,” said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. “We look forward to enrolling the first patients in the RGX-501 Phase I/II clinical trial in the coming months, and expect to file INDs for our other three lead programs in 2017. Our NAV Technology Licensees continued their work to advance NAV-based gene therapies, with new progress reported by AveXis during the second quarter of 2016 in the treatment of spinal muscular atrophy using the NAV AAV9 vector, and the addition of our ninth licensee, Biogen, in a partnership that is focused on treatments for inherited blindness using NAV AAV8 and AAV9 vectors.”

Recent Operational Highlights

- REGENXBIO and trial sponsor the University of Pennsylvania are actively recruiting and screening participants in the Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), and remain on track to enroll the first patients in the second half of 2016.

- REGENXBIO is in the process of completing preclinical studies for RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) and plans to file an Investigational New Drug application (IND) with the U.S. Food and Drug Administration (FDA) for a Phase I clinical trial in the first quarter of 2017. REGENXBIO plans to begin production to support this planned clinical trial in the third quarter of 2016.

- REGENXBIO is conducting dose-ranging pre-clinical studies of RGX-111 in animals for the treatment of Mucopolysaccharidosis Type I (MPS I) to inform an optimal initial dosing plan for a first-in-human Phase I/II clinical trial. REGENXBIO plans to file an IND with the FDA in the U.S. and a Clinical Trial Application (CTA) with Health Canada for the Phase I/II clinical trial in the first half of 2017.

- Rare Pediatric Disease Designation was granted to RGX-121 by the FDA in July 2016. REGENXBIO plans to file an IND with the FDA for a Phase I/II clinical trial in the first half of 2017 for the treatment of Mucopolysaccharidosis Type II (MPS II).

- REGENXBIO entered into an exclusive license agreement with Biogen in May 2016 for the development of gene therapy treatments for rare genetic vision disorders. REGENXBIO

received an undisclosed upfront payment and will receive ongoing fees, milestone payments and royalties on net sales of products incorporating the licensed intellectual property.

- REGENXBIO expanded the organization to 81 full-time employees as of August 5, 2016, including an additional key executive dedicated to advancing its lead programs:
    - Sherri Van Everen, Pharm.D., Vice President, Medical Affairs
        - More than 20 years of experience in medical affairs, including medical affairs strategy and initiatives, health economics and outcomes research.
        - Previous positions include leading medical affairs activities as Executive Director of Scientific Collaborations at Avalanche Biotechnologies, Inc., and supportive medical affairs roles at Genentech, Inc., and Eli Lilly and Company.

Financial Results

- Cash, cash equivalents and marketable securities as of June 30, 2016, were $198.7 million, compared to $216.4 million as of December 31, 2015.
- Revenues were $2.4 million for the quarter ended June 30, 2016, compared to $1.4 million for the quarter ended June 30, 2015.
- Total operating expenses were $17.3 million for the quarter ended June 30, 2016, compared to $7.7 million for the quarter ended June 30, 2015.
- Net loss was $14.4 million, or $0.55 net loss per basic and diluted common share, for the quarter ended June 30, 2016, compared to $6.3 million, or $3.24 net loss per basic and diluted common share, for the quarter ended June 30, 2015.

Financial Guidance

- REGENXBIO continues to expect full-year 2016 cash burn to be 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 June 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. 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 our 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 March 31, 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 June 30, 2016, to be filed with the SEC in the third 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)

	June 30, 2016	December 31, 2015
Assets		
Current assets		
Cash and cash equivalents	$ 30,083	$ 54,116
Marketable securities	64,124	60,025
Accounts receivable	783	2,136
Prepaid expenses	1,480	1,020
Other current assets	1,437	851
Total current assets	97,907	118,148
Marketable securities	104,510	102,226
Property and equipment, net	2,864	538
Cost method investments	300	300
Restricted cash	225	—
Other assets	266	168
Total assets	$ 206,072	$ 221,380
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 2,076	$ 1,014
Accrued expenses and other current liabilities	7,073	3,198
Advance payments	—	127
Total current liabilities	9,149	4,339
Deferred rent, net of current portion	748	233
Total liabilities	9,897	4,572
Stockholders' equity	196,175	216,808
Total liabilities and stockholders' equity	$ 206,072	$ 221,380

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Revenues				
License revenue	$ 2,245	$ 470	$ 2,573	$ 570
License revenue from related party	—	1,000	—	1,000

Reagent sales	107	44	166	148
Grant revenue	23	(151)	29	289
Total revenues	2,375	1,363	2,768	2,007
Expenses				
Costs of revenues				
Licensing costs (including amounts to related parties)	449	294	515	314
Costs of reagent sales (including amounts to related parties)	49	16	79	49
Research and development (including amounts to related parties)	10,680	4,012	16,863	6,803
General and administrative (including amounts to related parties)	6,169	3,397	11,648	5,113
Other operating expenses (income)	(20)	(60)	(134)	17
Total operating expenses	17,327	7,659	28,971	12,296
Loss from operations	(14,952)	(6,296)	(26,203)	(10,289)
Other Income (Expense)				
Investment income	515	6	998	8
Interest expense	—	—	—	(20)
Total other income (expense)	515	6	998	(12)
Net loss	$ (14,437)	$ (6,290)	$ (25,205)	$ (10,301)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities	246	—	1,240	—
Total other comprehensive income	246	—	1,240	—
Comprehensive loss	$ (14,191)	$ (6,290)	$ (23,965)	$ (10,301)
Reconciliation of net loss to net loss applicable to common stockholders				
Net loss	$ (14,437)	$ (6,290)	$ (25,205)	$ (10,301)
Net accretion and dividends on convertible preferred stock	—	(2,502)	—	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders	$ (14,437)	$ (8,792)	$ (25,205)	$ (11,289)
Basic and diluted net loss per common share	$ (0.55)	$ (3.24)	$ (0.96)	$ (4.21)
Weighted-average basic and diluted common shares	26,362	2,712	26,344	2,679

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