REGENXBIO Reports Fourth Quarter and Full-Year 2015 Financial Results and Recent Operational Progress

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- *Entered 2016 in strong financial position with more than $215 million in cash, cash equivalents and marketable securities; on track to advance multiple lead gene therapy product candidate programs into clinic*
- *Active IND for RGX-501 for the treatment of homozygous familial hypercholesterolemia*
- *Received FDA orphan drug designation for RGX-121 for the treatment of mucopolysaccharidosis Type II; IND planned for first half of 2017*

ROCKVILLE, Md., March 03, 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 reported financial results for the fourth quarter and full year ended December 31, 2015. REGENXBIO also provided updates on lead program progress and reiterated its full-year 2016 cash burn guidance.

"REGENXBIO has made significant progress over the past year toward our goal of changing the lives of people with severe diseases through gene therapy. During 2015, we strengthened our leadership team, built out our manufacturing capabilities and raised more than $240 million in capital to advance our pipeline. At the end of 2015, in collaboration with our development partners at the University of Pennsylvania, an IND for our lead program, RGX-501, became active. We believe all of our lead product candidate programs remain on track based on our previous guidance and I am also excited to announce that we intend to file an IND application for our fourth lead program, RGX-121 for the treatment of mucopolysaccharidosis Type II, in the first half of 2017," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "In 2016, we plan to focus on advancing our lead gene therapy product candidates into the clinic, expanding our pipeline and continuing to grow the REGENXBIO team with a goal of delivering important gene therapy treatments in areas of unmet patient need."

Fourth Quarter and Full-Year 2015 Financial Results

- Cash, cash equivalents and marketable securities as of December 31, 2015 were $216.4 million, compared to $1.1 million as of December 31, 2014.
- Revenues were $4.4 million for the quarter ended December 31, 2015, compared to $1.2 million for the quarter ended December 31, 2014. Full-year 2015 revenues were $7.6 million, compared to $6.1 million in 2014.
- Total operating expenses were $9.9 million for the quarter ended December 31, 2015, compared to $3.4 million for the quarter ended December 31, 2014. Full-year 2015 operating expenses were $30.7 million, compared to $9.8 million in 2014.
- Net loss was $5.2 million, or $0.20 net loss per basic and diluted share, for the quarter ended December 31, 2015, compared to $2.3 million, or $0.97 net loss per basic and diluted share, for the quarter ended December 31, 2014. Full-year 2015 net loss was $22.8 million, or $2.59 net loss per basic and diluted share, compared to a net loss of $4.0 million, or $1.82 net loss per basic and diluted share, in 2014.

2016 Financial Guidance

REGENXBIO's balance sheet was strengthened during 2015, with aggregate gross proceeds of $100.5 million raised from two private financings as well as $145.2 million raised from its initial public offering, net of underwriting discounts and commissions and offering expenses. REGENXBIO ended 2015 with $216.4 million in cash, cash equivalents and marketable securities. In 2016, REGENXBIO expects its cash burn to be between $60 million and $70 million, which will support the continuing development of its lead product candidate programs and the anticipated growth of its internal pipeline.

Pipeline Update

- **RGX-501:** An Investigational New Drug (IND) application is active. REGENXBIO is on track to initiate a Phase I/II clinical trial to evaluate the effect of RGX-501 in the treatment of

homozygous familial hypercholesterolemia (HoFH) in the first half of 2016 with the University of Pennsylvania.

- **RGX-111:** Orphan drug designation and rare pediatric disease designation were granted by the U.S. Food and Drug Administration (FDA) to RGX-111 for the treatment of mucopolysaccharidosis Type I (MPS I) in 2015. The U.S. National Institutes of Health Office of Biotechnology Activities' Recombinant DNA Advisory Committee endorsed REGENXBIO's RGX-111 protocol in 2015. REGENXBIO expects to file an IND application for RGX-111 for the treatment of MPS I with the FDA in the first half of 2016 and to initiate a Phase I/II clinical trial mid-2016.
- **RGX-314:** REGENXBIO plans to file an IND application for RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) with the FDA in the second half of 2016.
- **RGX-121:** Orphan drug designation was granted by the FDA to RGX-121 for the treatment of mucopolysaccharidosis Type II (MPS II), or Hunter syndrome, at the end of 2015. REGENXBIO expects to file an IND application for RGX-121 for the treatment of MPS II with the FDA in the first half of 2017.
- **NAV Technology Licensees:** REGENXBIO granted eight new commercial sublicenses to existing NAV Technology Licensees for the development of gene therapy products based on its NAV Technology Platform in 2015. As of December 31, 2015, the NAV Technology Platform is being applied in the development of 28 product candidates, including five internal and 23 partnered product candidates developed by third-party licensees. Since January 1, 2016, two NAV Technology Licensees have provided updates on active clinical trials:
    - AveXis, Inc., announced the completion of enrollment for a Phase I clinical trial of AVXS-101, which uses AAV9 in the treatment of patients with spinal muscular atrophy (SMA) Type 1.
    - Dimension Therapeutics, Inc., announced the initiation of a Phase I/II clinical trial of DTX101, which uses AAVrh10 in the treatment of patients with hemophilia B.

Additional Corporate Updates

- In June 2015, the Company entered into an exclusive strategic manufacturing collaboration with WuXi AppTec, Inc. (Wuxi) to establish efficient, scalable manufacturing processes within current Good Manufacturing Practice guidelines for the production of AAV gene therapy treatments incorporating REGENXBIO's NAV Technology Platform. REGENXBIO and Wuxi are currently engaged in the production of RGX-111 to support the planned Phase I/II clinical trial.
- REGENXBIO has grown to 56 full-time employees as of March 1, 2016, including the appointment of Faraz Ali as Chief Business Officer.

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 December 31, 2015, REGENXBIO's NAV Technology Platform is being applied in the development of 28 product candidates for a variety of diseases, including five internally developed candidates and 23 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 expected cash burn for 2016, 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 delay, divert or change any of them, and could cause actual results to differ materially from those projected in its forward-looking statements. 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 its product candidates and technology; REGENXBIO's ability to establish and maintain development partnerships, including those with NAV Technology Licensees; REGENXBIO's expectations regarding REGENXBIO's expenses and revenue, the sufficiency of REGENXBIO's cash resources and needs for additional financing, REGENXBIO's expectations regarding federal, state and foreign regulatory requirements; 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2015, available on the SEC's website at www.sec.gov. Additional factors may be described in those sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2015, to be filed with the SEC in the first quarter of 2016. In addition to the risks described above and in Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other 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)

	As of December 31,	
	2015	**2014**
Assets		
Current assets		
Cash and cash equivalents	$ 54,116	$ 1,121
Marketable securities	60,025	—
Accounts receivable		
Trade receivables	2,136	805
Related party receivables	—	750
Unbilled receivables	—	327
Prepaid expenses	1,020	28
Other current assets	851	—
Total current assets	118,148	3,031
Marketable securities	102,226	—
Property and equipment, net	538	—
Cost method investments	300	303
Deferred issuance costs	—	157
Other assets	168	—
Total assets	$ 221,380	$ 3,491
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities		
Accounts payable	$ 1,014	$ 334
Accrued expenses and other current liabilities	3,198	1,115
Due to related party under services agreement	—	1,423
Related party promissory notes	—	2,403
Other related party payables	—	3,761
Advance payments	127	153
Total current liabilities	4,339	9,189
Deferred rent, net of current portion	233	—
Total liabilities	4,572	9,189
Convertible preferred stock	—	12,593
Stockholders' equity (deficit)	216,808	(18,291)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 221,380	$ 3,491

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

	Three Months Ended December 31,		Years Ended December 31,	
	2015	**2014**	**2015**	**2014**
Revenues				
License revenue	$ 4,390	$ 650	$ 5,025	$ 4,355
License revenue from related party	—	$ 70	2,000	220
Reagent sales	48	$ 12	257	326
Grant revenue	3	$ 419	306	1,219
Total revenues	4,441	$ 1,151	7,588	6,120
Expenses				
Costs of revenues				
Licensing costs (including amounts to related parties)	878	$ 144	1,405	885
Costs of reagent sales (including amounts to related parties)	5	$ 8	98	122
Research and development (including amounts to related parties)	4,812	$ 2,089	17,279	4,961
General and administrative (including amounts to related parties)	4,232	$ 1,121	11,912	3,851
Foreign currency transaction losses	15	$ 19	57	30
Other operating income	—	$ (15)	(26)	(47)
Total operating expenses	9,942	$ 3,366	30,725	9,802
Loss from operations	(5,501)	$ (2,215)	(23,137)	(3,682)
Other Income (Expense)				
Investment income	323	$ —	346	—
Interest expense	—	$ (118)	(20)	(321)
Total other income (expense)	323	$ (118)	326	(321)
Net loss	$ (5,178)	$ (2,333)	$ (22,811)	$ (4,003)
Other Comprehensive Loss				
Unrealized loss on available-for-sale securities	(693)	$ —	(719)	—
Total other comprehensive loss	(693)	$ —	(719)	—
Comprehensive loss	$ (5,871)	$ (2,333)	$ (23,530)	$ (4,003)
Reconciliation of net loss to net loss applicable to common stockholders				
Net loss	$ (5,178)	$ (2,333)	$ (22,811)	$ (4,003)
Accretion and dividends on convertible preferred stock	—	$ (223)	(1,747)	(815)
Net gain on extinguishment of convertible preferred stock	—	$ —	759	—
Net loss applicable to common stockholders	$ (5,178)	$ (2,556)	$ (23,799)	$ (4,818)
Basic and diluted net loss per common share	$ (0.20)	$ (0.97)	$ (2.59)	$ (1.82)
Weighted-average basic and diluted common shares	26,312	2,644	9,173	2,643

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