REGENXBIO Reports Fourth Quarter and Full-Year 2016 Financial Results and Recent Operational Highlights

March 7, 2017 9:07 PM EST

- *IND for RGX-314 Phase I trial for wet AMD is active and dosing initiated for RGX-501 Phase I/II trial for HoFH*
- *Interim trial updates for RGX-314 and RGX-501 expected by year-end 2017*
- *$159 million in cash, cash equivalents and marketable securities as of December 31, 2016*

ROCKVILLE, Md., March 07, 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 fourth quarter and full year ended December 31, 2016 and recent operational highlights.

"In 2016, REGENXBIO made significant progress in advancing our lead product candidate programs that has enabled us to achieve key milestones in the first quarter of 2017. The IND for RGX-314 for the treatment of wet AMD is now active and we recently initiated dosing for our Phase I/II clinical trial of RGX-501 for the treatment of HoFH," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We believe 2017 will continue to be a pivotal year for REGENXBIO, as we seek to improve lives through the curative potential of gene therapy by advancing our clinical trials and building a NAV gene therapy pipeline internally and with our NAV Technology Platform Licensees. We look forward to providing updates on our lead product candidate programs throughout the year, including interim trial updates for RGX-314 and RGX-501 by year-end 2017."

Recent Operational Highlights

- In February 2017, REGENXBIO announced 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. REGENXBIO is on track to begin enrollment in the RGX-314 Phase I clinical trial by mid-2017, and to provide an interim trial 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). REGENXBIO is on track to provide an interim trial 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 the first half of 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. Manufacturing of material to support the planned RGX-121 Phase I/II clinical trial is ongoing.

- In 2016, REGENXBIO invested in internal capabilities, expanded its contract manufacturing network and opened an advanced manufacturing and analytics lab.

- As of December 31, 2016, REGENXBIO's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by nine NAV Technology Platform Licensees. Since February 1, 2017, two NAV Technology Platform Licensees have provided updates on active clinical programs:

- AveXis, Inc. announced details on the planned pivotal trial of AVXS-101 in the E.U., which is expected to initiate in the second half of 2017. AVXS-101 uses the NAV AAV9 vector for the treatment of spinal muscular atrophy (SMA) Type 1.

- Audentes Therapeutics, Inc. announced that the IND is active for the Phase I/II clinical trial of AT342, and that preliminary data are expected to be available by the end of 2017. AT342 uses the NAV AAV8 vector for the treatment of Crigler-Najjar Syndrome.

Financial Results

Cash, cash equivalents and marketable securities were $159.0 million as of December 31, 2016, compared to $216.4 million as of December 31, 2015.

Revenues were $1.7 million and $4.6 million for the three months and year ended December 31, 2016, respectively, compared to $4.4 million and $7.6 million for the three months and year ended December 31, 2015, respectively.

Total operating expenses were $22.2 million and $69.9 million for the three months and year ended December 31, 2016, respectively, compared to $9.9 million and $30.7 million for the three months and year ended December 31, 2015, respectively.

Net loss was $19.6 million and $63.0 million, or $0.74 and $2.38 net loss per basic and diluted common share, for the three months and year ended December 31, 2016, respectively, compared to $5.2 million and $22.8 million, or $0.20 and $2.59 net loss per basic and diluted share, for the three months and year ended December 31, 2015, respectively.

Financial Guidance

REGENXBIO reiterated 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.

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 Platform 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 financial guidance and 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 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, 2016, to be filed in the first 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)

	December 31, 2016	December 31, 2015
Assets		

Current assets		
Cash and cash equivalents	$ 24,840	$ 54,116
Marketable securities	64,714	60,025
Accounts receivable	1,032	2,136
Prepaid expenses	1,775	1,020
Other current assets	1,010	851
Total current assets	93,371	118,148
Marketable securities	69,412	102,226
Property and equipment, net	9,324	538
Cost method investments	—	300
Restricted cash	225	—
Other assets	400	168
Total assets	$ 172,732	$ 221,380
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 1,543	$ 1,014
Accrued expenses and other current liabilities	8,126	3,198
Advance payments	—	127
Total current liabilities	9,669	4,339
Deferred rent, net of current portion	1,326	233
Total liabilities	10,995	4,572
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at December 31, 2016 and December 31, 2015	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at December 31, 2016 and December 31, 2015; 26,477 and 26,313 shares issued and outstanding at December 31, 2016 and December 31, 2015, respectively	3	3
Additional paid-in capital	276,354	269,144
Accumulated other comprehensive loss	(33)	(719)
Accumulated deficit	(114,587)	(51,620)
Total stockholders' equity	161,737	216,808
Total liabilities and stockholders' equity	$ 172,732	$ 221,380

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

	Three Months Ended December 31,		Years Ended December 31,	
	2016	2015	2016	2015
Revenues				
License revenue	$ 1,665	$ 4,390	$ 4,303	$ 5,025
License revenue from related party	—	—	—	2,000
Reagent sales	—	48	213	257
Grant revenue	31	3	73	306
Total revenues	1,696	4,441	4,589	7,588
Expenses				
Costs of revenues				
Licensing costs (including amounts to related parties)	330	878	861	1,405
Costs of reagent sales (including amounts to related parties)	—	5	98	98
Research and development (including amounts to related parties)	16,059	4,812	45,482	17,279
General and administrative (including amounts to related parties)	5,742	4,232	23,590	11,912

Other operating expenses (income)	34	15	(102)	31
Total operating expenses	22,165	9,942	69,929	30,725
Loss from operations	(20,469)	(5,501)	(65,340)	(23,137)
Other Income (Expense)				
Investment income	426	323	1,938	346
Interest expense	—	—	—	(20)
Total other income (expense)	426	323	1,938	326
Loss before income taxes	(20,043)	(5,178)	(63,402)	(22,811)
Income Tax Benefit	435	—	435	—
Net loss	$ (19,608)	$ (5,178)	$ (62,967)	$ (22,811)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of income tax expense (benefit)	(886)	(693)	686	(719)
Total other comprehensive income (loss)	(886)	(693)	686	(719)
Comprehensive loss	$ (20,494)	$ (5,871)	$ (62,281)	$ (23,530)
Basic and diluted net loss per common share	$ (0.74)	$ (0.20)	$ (2.38)	$ (2.59)
Weighted-average basic and diluted common shares	26,476	26,312	26,409	9,173

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