



REGENXBIO Reports First Quarter 2016 Financial Results and Recent Operational Highlights

May 05, 2016 08:01 AM EDT

- On track to initiate a Phase I/II clinical trial for RGX-501 for the treatment of homozygous familial hypercholesterolemia in the first half of 2016
- Progressing toward filing an IND with the U.S. Food and Drug Administration for RGX-111 for the treatment of Mucopolysaccharidosis Type I in the first half of 2016; expect to initiate a Phase I/II clinical trial in the third quarter of 2016
- First quarter 2016 cash burn of \$7.8 million, with cash, cash equivalents and marketable securities of \$208.6 million as of March 31, 2016; the Company continues to expect full year cash burn between \$60 million and \$70 million

ROCKVILLE, Md., May 05, 2016 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (REGENXBIO or the Company) (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 and recent operational highlights for the quarter ended March 31, 2016.

"During the first quarter of 2016, we made significant progress toward this year's goals of initiating two clinical studies and filing an Investigational New Drug application to support the initiation of a third study," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Currently, REGENXBIO and our partners at the University of Pennsylvania are recruiting participants in the Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia. In addition, we are completing steps that will enable us to file an Investigational New Drug application for RGX-111 for the treatment of Mucopolysaccharidosis Type I. REGENXBIO also continues to build our team and capabilities to support the activities that will best position us to advance our mission of developing and commercializing gene therapy products that transform the lives of people suffering from severe diseases."

Recent Operational Highlights

- REGENXBIO and trial sponsor the University of Pennsylvania (Penn) are recruiting participants in the Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH). The Company entered into agreements with Advanced BioScience Laboratories, Inc. (ABL), to support the transfer, process scale-up and production of RGX-501 for future use in clinical trials. The initial RGX-501 study drug to be used in the Phase I/II clinical trial was manufactured under a resource grant supported by the National Institutes of Health (NIH) and awarded to Penn.
- The Company is completing the initial production of RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I) with WuXi AppTec, Inc., and is preparing for regulatory filing submissions in the U.S. and Canada to support the planned Phase I/II clinical trial for RGX-111.
- The Company had meetings with regulatory authorities to discuss filing submissions for RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), and RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II). Preclinical data used in these meetings are being presented this week at the American Society of Gene & Cell Therapy 19th Annual Meeting. REGENXBIO plans to file an Investigational New Drug application (IND) for RGX-314 in the second half of 2016, and an IND for RGX-121 in the first half of 2017.
- REGENXBIO expanded the organization to 63 full-time employees as of May 2, 2016, including the following key executives dedicated to corporate initiatives and the progression of its lead programs:
 - Laura Coruzzi, Ph.D., J.D., Senior Vice President, Intellectual Property

- More than 30 years of experience in all aspects of life sciences patent law.
- Previous positions include Partner in the intellectual property practice at Jones Day and Partner in the biotechnology group at Pennie & Edmonds, LLP.
- Kimberly Sloan, Senior Vice President, Human Resources
 - More than 20 years of experience in domestic and global human resources.
 - Previous positions include Vice President, Human Resources at Biocon / MTF, and senior leadership roles at Princeton Financial Systems and Dun & Bradstreet.
- Rickey Reinhardt, M.D., Ph.D., Vice President, Clinical Research & Development
 - More than 20 years of experience in global clinical development.
 - Previous positions include Vice President of Translational Medicine and Clinical Development in the rare disease unit at GlaxoSmithKline, and Vice President of Global Molecule Development at Covance.

Financial Results

- Cash, cash equivalents and marketable securities as of March 31, 2016 were \$208.6 million, compared to \$216.4 million as of December 31, 2015.
- Revenues were \$0.4 million for the quarter ended March 31, 2016, compared to \$0.6 million for the quarter ended March 31, 2015.
- Total operating expenses were \$11.6 million for the quarter ended March 31, 2016, compared to \$4.6 million for the quarter ended March 31, 2015.
- Net loss was \$10.8 million, or \$0.41 net loss per basic and diluted share, for the quarter ended March 31, 2016, compared to \$4.0 million, or \$0.94 net loss per basic and diluted share, for the quarter ended March 31, 2015.

Guidance

- The Company 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 March 31, 2016, REGENXBIO's NAV Technology Platform was 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 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 Annual Report on Form 10-K for the year ended December 31, 2015, available on the SEC's website at www.sec.gov. Additional factors may be described in those sections of REGENXBIO's Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, to be filed with the SEC in the second 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, except per share data)

	<u>March 31,</u> <u>2016</u>	<u>December 31,</u> <u>2015</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 35,511	\$ 54,116
Marketable securities	65,544	60,025
Accounts receivable	2,036	2,136
Prepaid expenses	1,189	1,020
Other current assets	<u>1,300</u>	<u>851</u>
Total current assets	105,580	118,148
Marketable securities	107,553	102,226
Property and equipment, net	1,328	538
Cost method investments	300	300
Other assets	<u>223</u>	<u>168</u>
Total assets	<u>\$ 214,984</u>	<u>\$ 221,380</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 1,113	\$ 1,014
Accrued expenses and other current liabilities	4,633	3,198
Advance payments	<u>35</u>	<u>127</u>
Total current liabilities	5,781	4,339
Deferred rent, net of current portion	<u>602</u>	<u>233</u>
Total liabilities	6,383	4,572
Stockholders' equity	<u>208,601</u>	<u>216,808</u>
Total liabilities and stockholders' equity	<u>\$ 214,984</u>	<u>\$ 221,380</u>

**REGENXBIO INC.
STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**

(unaudited)

(in thousands, except per share data)

	<u>Three Months Ended March 31,</u>	
	<u>2016</u>	<u>2015</u>
Revenues		
License revenue	\$ 328	\$ 100
Reagent sales	59	104
Grant revenue	<u>6</u>	<u>440</u>
Total revenues	393	644
Expenses		
Costs of revenues		
Licensing costs (including amounts to related parties)	66	20
Costs of reagent sales (including amounts to related parties)	30	33
Research and development (including amounts to related parties)	6,183	2,791

General and administrative (including amounts to related parties)	5,479	1,716
Other operating expenses (income)	(114)	77
Total operating expenses	11,644	4,637
Loss from operations	(11,251)	(3,993)
Other Income (Expense)		
Investment income	483	2
Interest expense	—	(20)
Total other income (expense)	483	(18)
Net loss	\$ (10,768)	\$ (4,011)
Other Comprehensive Income		
Unrealized gain on available-for-sale securities	994	—
Total other comprehensive income	994	—
Comprehensive loss	\$ (9,774)	\$ (4,011)
Reconciliation of net loss to net loss applicable to common stockholders		
Net loss	\$ (10,768)	\$ (4,011)
Net decretion and dividends on convertible preferred stock	—	755
Net gain on extinguishment of convertible preferred stock	—	759
Net loss applicable to common stockholders	\$ (10,768)	\$ (2,497)
Basic and diluted net loss per common share	\$ (0.41)	\$ (0.94)
Weighted-average basic and diluted common shares	26,327	2,645

CONTACT: [

Investors [

Elizabeth Broder, 646-378-2945

ebroder@troutgroup.com

Media [

Laura Bagby, 312-448-8098

lbagby@6degreespr.com



REGENXBIO Inc.