



REGENXBIO Reports Third Quarter 2015 Results

November 05, 2015 09:02 AM EST

Closed Initial Public Offering Raising \$145.2 Million in Net Proceeds

ROCKVILLE, Md., Nov. 5, 2015 (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, today reported recent operational highlights and financial results for the quarter ended September 30, 2015.

"So far, 2015 has been a truly transformative year for REGENXBIO and we look forward to building upon this success in the future," said Kenneth T. Mills, President and CEO of REGENXBIO. "We have raised more than \$200 million in capital this year, including the completion of an initial public offering in September, which strengthened our balance sheet and continued to add leading healthcare investors as stockholders. We remain focused on advancing our lead investigational gene therapy candidates toward the clinic, and we plan to initiate a clinical trial for RGX-501 for the treatment of homozygous familial hypercholesterolemia and to file an Investigational New Drug application for RGX-111 for the treatment of mucopolysaccharidosis Type I in the first half of 2016."

Recent Operational Highlights:

- Completed an initial public offering of common stock at \$22.00 per share, raising \$145.2 million, net of underwriting discounts and commissions and offering expenses.
- Received orphan drug designation from the U.S. Food and Drug Administration for our investigational gene therapy product candidate RGX-111 for the treatment of mucopolysaccharidosis Type I (MPS I).
- Received endorsement from the U.S. National Institutes of Health Office of Biotechnology Activities' Recombinant DNA Advisory Committee, commonly referred to as the RAC, for our RGX-111 protocol.
- Further strengthened our management team with the addition of Curran Simpson, SVP of Technical Operations. Most recently, Mr. Simpson was the Head, North American Supply Chain and Interim Chief Operating Officer and Integration Lead with GlaxoSmithKline and Human Genome Sciences, a division of GlaxoSmithKline.
- Appointed David C. Stump, M.D., to our board of directors. Most recently, Dr. Stump was Executive Vice President, Research and Development at Human Genome Sciences.

Financial Results

Revenues were \$1.1 million for the quarter ended September 30, 2015, compared to \$0.5 million for the quarter ended September 30, 2014.

Net cash used in operating activities during the nine months ended September 30, 2015 was \$18.1 million. Cash, cash equivalents and marketable securities as of September 30, 2015 were \$224.4 million.

Total operating expenses were \$8.5 million for the quarter ended September 30, 2015, compared to \$2.2 million for the quarter ended September 30, 2014.

Net loss was \$7.3 million, or \$1.52 net loss per basic and diluted share, for the quarter ended September 30, 2015, compared to a net loss of \$1.8 million, or \$0.73 net loss per basic and diluted share, for the quarter ended September 30, 2014.

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, 2015, REGENXBIO's NAV Technology Platform is being applied in the development of 23 product

candidates for a variety of diseases, including five product candidates being developed internally by REGENXBIO and 18 product candidates being 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-501, RGX-111 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 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 our product candidates; REGENXBIO's ability to establish and maintain development partnerships; 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 prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on September 17, 2015 (the Prospectus) and 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 September 30, 2015, to be filed with the SEC in the fourth quarter of 2015. In addition to the risks described above and in the Prospectus 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)

	December 31,	September 30,
	2014	2015
Assets		
Current assets		
Cash and cash equivalents	\$ 1,121	\$ 205,324
Marketable securities	—	8,025
Accounts receivable		
Trade receivables	805	609
Related party receivables	750	—
Unbilled receivables	327	—
Prepaid expenses	28	1,400
Other current assets	—	127
Total current assets	3,031	215,485
Marketable securities	—	11,013
Property and equipment, net	—	385
Cost method investments	303	303
Deferred issuance costs	157	—
Other assets	—	128
Total assets	<u>\$ 3,491</u>	<u>\$ 227,314</u>
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities		
Accounts payable	\$ 334	\$ 3,105

Accrued expenses	1,115	1,727
Due to related party under services agreement	1,423	—
Related party promissory notes	2,403	—
Other related party payables	3,761	349
Advance payments	153	127
Total current liabilities	9,189	5,308
Deferred rent	—	198
Total liabilities	9,189	5,506
Convertible preferred stock	12,593	—
Total stockholders' equity (deficit)	(18,291)	221,808
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	\$ 3,491	\$ 227,314

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

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2014	2015	2014	2015
Revenues				
License revenue	\$ —	\$ 65	\$ 3,705	\$ 635
License revenue from related party	150	1,000	150	2,000
Reagent sales	23	61	317	200
Grant revenue	310	14	800	305
Total revenues	483	1,140	4,972	3,140
Expenses				
Costs of revenues				
Licensing costs to related parties	—	213	741	527
Costs of reagent sales (including amounts to related parties)	12	44	115	94
Research and development (including amounts to related parties)	1,085	5,664	2,869	12,471
General and administrative (including amounts to related parties)	1,070	2,567	2,732	7,671
Foreign currency transaction losses (gains)	25	4	12	41
Other operating income	(8)	(5)	(32)	(26)
Total operating expenses	2,184	8,487	6,437	20,778
Loss from operations	(1,701)	(7,347)	(1,465)	(17,638)
Other Income (Expense)				
Investment income	—	15	—	23
Interest expense	(92)	—	(202)	(20)
Total other income (expense)	(92)	15	(202)	3
Net loss	\$ (1,793)	\$ (7,332)	\$ (1,667)	\$ (17,635)
Other Comprehensive Loss				

Unrealized loss on available-for-sale securities	—	(26)	—	(26)
Total other comprehensive loss	—	(26)	—	(26)
Comprehensive loss	<u>\$ (1,793)</u>	<u>\$ (7,358)</u>	<u>\$ (1,667)</u>	<u>\$ (17,661)</u>

Reconciliation of net loss to net loss applicable to common stockholders

Net loss	\$ (1,793)	\$ (7,332)	\$ (1,667)	\$ (17,635)
Accretion and dividends on convertible preferred stock	(126)	—	(592)	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders	<u>\$ (1,919)</u>	<u>\$ (7,332)</u>	<u>\$ (2,259)</u>	<u>\$ (18,623)</u>
Basic and diluted net loss per common share	<u>\$ (0.73)</u>	<u>\$ (1.52)</u>	<u>\$ (0.85)</u>	<u>\$ (5.48)</u>
Weighted-average basic and diluted common shares	<u>2,643</u>	<u>4,809</u>	<u>2,643</u>	<u>3,397</u>

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