



REGENXBIO Reports Second Quarter 2017 Financial Results and Recent Operational Highlights

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- *Enrollment continues in RGX-314 Phase I clinical trial for wet AMD; interim update anticipated by year-end 2017*
- *Enrollment continues in RGX-501 Phase I/II clinical trial for HoFH; interim update anticipated by year-end 2017*
- *IND active for third lead product candidate – RGX-111 for MPS I*
- *\$209 million in cash, cash equivalents and marketable securities as of June 30, 2017*

ROCKVILLE, Md., Aug. 08, 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 second quarter ended June 30, 2017 and recent operational highlights.

"We continue to progress our lead product candidates, most notably with the continued enrollment in our Phase I clinical trial evaluating RGX-314 for the treatment of wet AMD and in our Phase I/II clinical trial evaluating RGX-501 for the treatment of HoFH. The active IND for RGX-111 for the treatment of MPS I – our third clinical-stage lead product candidate – is another pipeline achievement that demonstrates our strong internal capabilities and commitment to the development of a robust clinical pipeline of gene therapy product candidates in many diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We continue to advance our internal and partnered gene therapy product candidates, leveraging our proprietary NAV Technology Platform to demonstrate the curative potential of one-time administrations of gene therapy. We look forward to sharing more pipeline updates, including interim updates from the RGX-314 Phase I clinical trial for wet AMD and the RGX-501 Phase I/II clinical trial for HoFH, by year-end 2017."

Recent Operational Highlights

- REGENXBIO initiated dosing in the Phase I clinical trial evaluating RGX-314 for the treatment of wet age-related macular degeneration (wet AMD). Patient recruitment and enrollment is continuing and REGENXBIO expects to provide an interim update from the RGX-314 clinical trial by the end of 2017.
- Patient recruitment and enrollment is continuing in the Phase I/II clinical trial evaluating RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH). REGENXBIO expects to provide an interim update from the RGX-501 clinical trial by the end of 2017.
- REGENXBIO today announced that the Investigational New Drug (IND) application for the Phase I clinical trial of RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I) is active. Site activation in this planned multi-center, open-label, multiple-cohort, dose-escalation trial is underway to support recruitment and patient enrollment, with the first patient expected to be dosed in the first half of 2018.
- REGENXBIO plans to file an IND application for RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) in the second half of 2017, which will incorporate feedback received from the U.S. Food and Drug Administration (FDA) in its review of the IND application for RGX-111.
- REGENXBIO has completed production of investigational product for all four of its lead

product candidates in an amount which is expected to supply planned clinical trials through 2018 and has established a supply chain to ensure effective distribution to REGENXBIO's planned clinical trial sites worldwide.

- As of June 30, 2017, REGENXBIO's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by nine NAV Technology Licensees. Eight of these partnered product candidates have advanced to clinical stages of development.
 - In June 2017, AveXis, Inc. announced alignment with the FDA on its Good Manufacturing Practice (GMP) commercial manufacturing process for AVXS-101 for the treatment of spinal muscular atrophy (SMA) Type 1. AveXis announced that it plans to initiate the pivotal trial later in the third quarter of 2017. AVXS-101 uses the NAV AAV9 vector.
 - Also in June 2017, REGENXBIO and AveXis entered into an exclusive worldwide license agreement for AveXis to develop and commercialize gene therapy treatments using the NAV AAV9 vector to treat two rare neurological monogenic disorders: Rett syndrome (RTT) and amyotrophic lateral sclerosis (ALS).
 - In July 2017, Shire plc announced the submission of an IND application for SHP654, an investigational factor VIII gene therapy for the treatment of hemophilia A. SHP654 uses the NAV AAV8 vector.

Financial Results

Cash, cash equivalents and marketable securities were \$208.5 million as of June 30, 2017, compared to \$159.0 million as of December 31, 2016.

Revenues were \$6.6 million for the three months ended June 30, 2017, compared to \$2.4 million for the three months ended June 30, 2016.

Total operating expenses were \$21.6 million for the three months ended June 30, 2017, compared to \$17.3 million for the three months ended June 30, 2016.

Net loss was \$14.5 million, or \$0.47 net loss per basic and diluted common share, for the three months ended June 30, 2017, compared to \$14.4 million, or \$0.55 net loss per basic and diluted share, for the three months ended June 30, 2016.

Financial Guidance

REGENXBIO now expects full-year 2017 cash burn to be between \$75 million and \$80 million, which will support the continued development of its lead product candidate programs. Full-year 2017 cash burn guidance excludes the effect of REGENXBIO's previously announced underwritten public offering of common stock in March 2017 and the underwriters' exercise of their option to purchase additional shares in April 2017, which resulted in aggregate net proceeds to REGENXBIO of approximately \$81.5 million, after deducting underwriting discounts and commissions and offering expenses.

Conference Call Information

In connection with the earnings release, REGENXBIO will host a conference call today at 4:30 p.m. ET. To access the live call by phone, dial (855) 422-8964 (domestic) or (210) 229-8819 (international), and enter the passcode 53241624. To access a live or recorded webcast of the call, please visit the "Investors" section of the REGENXBIO website at www.regenxbio.com. The recorded webcast will be available for approximately 30 days following the call.

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 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 research, development and regulatory plans in connection with its NAV Technology Platform and gene therapy treatments, the research, development and regulatory plans of REGENXBIO's NAV Technology Licensees and REGENXBIO's cash burn. 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, 2017 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, which have been filed with the Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. Additional factors may be set forth in those sections of REGENXBIO's Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, which will be filed with the SEC in the third 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.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except per share data)

	<u>June 30, 2017</u>	<u>December 31, 2016</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 57,649	\$ 24,840
Marketable securities	104,434	64,714
Accounts receivable	50	1,032
Prepaid expenses	2,432	1,775
Other current assets	1,252	1,010
Total current assets	<u>165,817</u>	<u>93,371</u>
Marketable securities	46,417	69,412
Property and equipment, net	11,524	9,324
Restricted cash	225	225
Other assets	393	400
Total assets	<u>\$ 224,376</u>	<u>\$ 172,732</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 3,948	\$ 1,543
Accrued expenses and other current liabilities	7,514	8,126
Total current liabilities	<u>11,462</u>	<u>9,669</u>
Deferred rent, net of current portion	1,217	1,326
Total liabilities	<u>12,679</u>	<u>10,995</u>
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2017 and December 31, 2016	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at June 30, 2017 and December 31, 2016; 30,853 and 26,477 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	3	3
Additional paid-in capital	363,393	276,354
Accumulated other comprehensive loss	(646)	(33)
Accumulated deficit	<u>(151,053)</u>	<u>(114,587)</u>
Total stockholders' equity	<u>211,697</u>	<u>161,737</u>
Total liabilities and stockholders' equity	<u>\$ 224,376</u>	<u>\$ 172,732</u>

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenues				
License revenue	\$ 6,555	\$ 2,245	\$ 7,010	\$ 2,573
Reagent sales	—	107	—	166
Grant revenue	7	23	7	29
Total revenues	<u>6,562</u>	<u>2,375</u>	<u>7,017</u>	<u>2,768</u>
Expenses				
Costs of revenues				
Licensing costs	1,311	449	1,402	515
Costs of reagent sales	6	49	6	79
Research and development	13,917	10,680	30,536	16,863
General and administrative	6,355	6,169	12,977	11,648
Other operating expenses (income)	29	(20)	74	(134)
Total operating expenses	<u>21,618</u>	<u>17,327</u>	<u>44,995</u>	<u>28,971</u>
Loss from operations	(15,056)	(14,952)	(37,978)	(26,203)
Other Income				
Investment income	583	515	1,512	998
Total other income	<u>583</u>	<u>515</u>	<u>1,512</u>	<u>998</u>
Net loss	<u>\$ (14,473)</u>	<u>\$ (14,437)</u>	<u>\$ (36,466)</u>	<u>\$ (25,205)</u>
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications of \$480 for the six months ended June 30, 2017	(74)	246	(613)	1,240
Total other comprehensive income (loss)	<u>(74)</u>	<u>246</u>	<u>(613)</u>	<u>1,240</u>
Comprehensive loss	<u>\$ (14,547)</u>	<u>\$ (14,191)</u>	<u>\$ (37,079)</u>	<u>\$ (23,965)</u>
Basic and diluted net loss per common share	<u>\$ (0.47)</u>	<u>\$ (0.55)</u>	<u>\$ (1.27)</u>	<u>\$ (0.96)</u>
Weighted-average basic and diluted common shares	<u>30,662</u>	<u>26,362</u>	<u>28,678</u>	<u>26,344</u>

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