



REGENXBIO Reports Third Quarter 2019 Financial and Operating Results and Recent Operational Highlights

November 5, 2019 9:05 PM EST

ROCKVILLE, Md., Nov. 5, 2019 /PRNewswire/ --

- *Initiation of RGX-314 Phase IIb trial for wet AMD and IND filing for Phase II trial in diabetic retinopathy now expected in Q1 2020 as a result of partial clinical hold following FDA notification related to certain commercial, third-party surgical devices; matter is not related to RGX-314 gene therapy candidate*
- *Positive interim update from RGX-314 Phase I/IIa trial for wet AMD presented in October at AAO*
 - *Well-tolerated at all doses*
 - *Anti-VEGF treatment burden was decreased by over 80% in Cohort 5, with 75% of subjects anti-VEGF injection-free up to 6 months after RGX-314 administration*
 - *50% of subjects in Cohort 3 were anti-VEGF injection-free at 1.5 years after RGX-314 administration*
- *Completed dosing of Cohort 2 in the RGX-501 Phase I/II trial for HoFH and Cohort 1 in the RGX-121 Phase I/II trial for MPS II; interim updates expected at year end*
- *Revenue includes \$9.2 million in royalties from the Q3 2019 sales of Novartis' Zolgensma[®]*
- *\$417 million in cash, cash equivalents and marketable securities as of September 30, 2019; expect to end 2019 with cash, cash equivalents and marketable securities of at least \$365 million*
- *Webcast and conference call scheduled for today at 4:30 p.m. ET*

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 quarter ended September 30, 2019, and recent operational highlights.

"We continue to make significant advances in our pipeline and NAV Technology Platform, expanding the pipeline with programs to deliver therapeutic antibodies, beginning an exciting collaboration with Neurimmune, and initiating construction of a new manufacturing facility," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Our focus on patients drives us to build upon the clinical progress of gene therapy options for both large indications and rare diseases, and we are excited to lead the field in innovative approaches. We are also very encouraged by the early adoption of the first approved NAV Technology-based therapy, Novartis' Zolgensma[®], for the treatment of SMA."

"We anticipate a short delay in the initiation of our Phase IIb trial for RGX-314 in wet AMD and our investigative new drug, or IND, application filing for diabetic retinopathy, following notification from the FDA regarding certain third-party commercially-available surgical devices that were used to deliver RGX-314 in our Phase I/IIa trial," added Mr. Mills. "The notification is not related to the RGX-314 gene therapy itself, and we have not received reports of any device-related concerns or complications in the subjects already dosed in the Phase I/IIa trial. Assessments and monitoring of all enrolled subjects in the trial continue to be performed as usual. We are working with the FDA to address this matter, and as a result, we now plan to initiate our Phase IIb trial for RGX-314 in wet AMD and file our IND for diabetic retinopathy in Q1 2020."

"We are encouraged by the data from the Phase I/IIa clinical trial of RGX-314 in patients with wet AMD," said Dr. Stephen Pakola, Senior Vice President and Chief Medical Officer of REGENXBIO. "These data demonstrate significant long-term reduction of anti-VEGF treatment burden while maintaining or improving vision and retinal thickness after a single administration of our gene therapy. We look forward to initiating our Phase IIb trial of RGX-314 in wet AMD, and preparing to file an IND to evaluate RGX-314 in diabetic retinopathy, while continuing our collaboration with Clearside to evaluate the in-office suprachoroidal administration of RGX-314. We have also achieved important clinical development milestones in our other gene therapy programs, including completing dosing of Cohort 2 in our RGX-501 trial for HoFH and Cohort 1 in our RGX-121 trial for MPS II. Our internal pipeline of novel gene therapies positions us to develop meaningful potential treatments for a broad spectrum of patients with significant unmet needs."

Product Candidate Updates

Gene Therapy using NAV Vectors for AAV-Mediated Antibody Delivery:

- RGX-314 for the Treatment of Wet Age-related Macular Degeneration (wet AMD) and Diabetic Retinopathy (DR)
 - REGENXBIO has been informed by the U.S. Food and Drug Administration (FDA) that they are assessing aspects of the surgical delivery system, specifically the use of certain third-party commercially-available devices that are used to deliver RGX-314 in our Phase I/IIa trial for the treatment of wet AMD. As previously announced, this trial has completed dosing of all 42 patients with RGX-314. While discussions with the FDA are ongoing, however, the IND for the trial has been placed on a partial clinical hold, which is not related to the gene therapy product candidate itself. The Company is not aware of any delivery system concerns or complications from the Phase I/IIa trial, nor is it aware of any concerns or actions related to these third-party device suppliers or other companies using similar devices in AAV gene therapy surgical delivery systems. Regular assessments and safety monitoring of all subjects enrolled in the Phase I/IIa trial continue to be performed as normal.
 - The Company is conducting a review of the third-party device components and believes that there are readily available and suitable alternatives for these third-party devices, if needed. REGENXBIO will continue to work closely with the FDA regarding the potential replacement, testing or modification of third-party devices. As a result of these activities, REGENXBIO now expects to initiate the RGX-314 Phase IIb trial for wet AMD and file an IND for the Phase II trial in DR in the first quarter of 2020.
 - In October 2019, REGENXBIO presented interim results from all five dose cohorts of the RGX-314 Phase I/IIa dose escalation clinical trial in subjects with wet AMD at the American Academy of Ophthalmology Annual Meeting (AAO). As of October 9, 2019, RGX-314 continued to be well-tolerated across all cohorts, with no drug-related serious adverse events (SAEs) reported. Up to six months after administration of RGX-314, subjects in Cohort 5 demonstrated a reduction of over 80% from the mean annualized injection rate during the 12 months prior to administration of RGX-314, and 75% of subjects had not received anti-VEGF injections, with mean improvement in vision and retinal thickness. Durable effects on vision and retinal thickness had been demonstrated over 1.5 years in Cohort 3, and 50% of subjects remained free of anti-VEGF injections more than 1.5 years after RGX-314 administration.
 - REGENXBIO continues to evaluate in-office delivery of RGX-314 to the suprachoroidal space. Clinical plans for this route of administration are expected to be announced in 2020.
- Hereditary Angioedema (HAE)
 - REGENXBIO expects to provide an update in early 2020 on the preclinical studies of a new gene therapy product candidate for the treatment of HAE, as well as plans for clinical development.
- Neurodegenerative Diseases
 - REGENXBIO initiated design and development of vectorized antibody therapies in collaboration with Neurimmune AG, using Neurimmune's Reverse Translational Medicine™ platform along with the Company's NAV Technology Platform. Initially, the companies are focusing on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies).

Gene Therapy using NAV Vectors for Rare Genetic Diseases:

- RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)
 - REGENXBIO completed dosing of subjects in Cohort 2 in the Phase I/II clinical trial evaluating RGX-501 for the treatment of HoFH with corticosteroid prophylaxis and

expects to report interim data at the end of 2019.

- The primary endpoint of this trial is safety and tolerability, and secondary endpoints include changes in low-density lipoprotein (LDL-C) and other lipid outcome measures.
- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
 - REGENXBIO completed dosing of subjects in Cohort 1 in the Phase I/II clinical trial evaluating RGX-121 for the treatment of MPS II. The Company expects to report an interim data update from the Phase I/II clinical trial at the end of 2019.
 - The primary endpoint of this trial includes safety and tolerability, and secondary endpoints include change in biomarkers as measured in cerebrospinal fluid, serum and urine, and change in neurodevelopmental parameters.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
 - RGX-111 was recently administered to a subject with MPS I through an investigator-initiated study at CHOC Children's Hospital, following review and agreement by the FDA. As of November 4, 2019, RGX-111 has been well-tolerated.
 - Recruitment, screening and additional site activations are ongoing in the Phase I clinical trial evaluating RGX-111 for the treatment of MPS I.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) "Batten" Disease
 - REGENXBIO intends to submit an IND (or foreign equivalent) for the first-in-human clinical trial evaluating RGX-181 in the second half of 2020, following additional preclinical development and analyses to support clinical development.
 - Preclinical data presented at the European Society of Gene & Cell Therapy (ESGCT) 27th Annual Congress highlighted outcomes from a murine mouse model for CLN2 Batten disease.

Other Recent Operational Highlights

- In October 2019, REGENXBIO exercised the full option with Clearside Biomedical, Inc. for exclusive worldwide rights to Clearside's proprietary, in-office SCS Microinjector™ for the delivery of AAV vectors to the suprachoroidal space to treat a number of indications. REGENXBIO continues to evaluate RGX-314 using Clearside's SCS Microinjector for in-office, non-surgical delivery into the suprachoroidal space to treat wet AMD, DR and other conditions for which anti-VEGF treatment is currently the standard of care, while continuing to advance its RGX-314 subretinal delivery program currently in development.
- Construction of REGENXBIO's current good manufacturing practice (cGMP) production facility continues as planned. The new cGMP production facility is designed to allow for production of NAV Technology-based vectors at scales up to 2,000 liters using REGENXBIO's platform suspension cell culture process. The facility is expected to be operational in 2021.

NAV Technology Licensee Program Highlights

As of October 31, 2019, REGENXBIO's NAV Technology Platform was being applied in one marketed product, Novartis AG's Zolgensma, and more than 20 partnered product candidates in development by NAV Technology Licensees. Fifteen of these partnered product candidates are in active clinical development. REGENXBIO's NAV Technology Licensees are advancing product candidates in a broad range of therapeutic areas and disease indications. Recent updates from NAV Technology Licensees include:

Marketed Products

On October 22, 2019, Novartis AG reported Q3 2019 U.S. Zolgensma sales revenue of \$160 million, and REGENXBIO recognized royalty revenue of \$9.2 million in Q3 2019 as a result of these sales.

Partnered Product Candidates

- In August 2019, Audentes Therapeutics, Inc. stated that enrollment in the pivotal expansion cohort data of ASPIRO, the clinical trial evaluating AT132 in subjects with X-Linked Myotubular Myopathy (XLMTM), is expected to be complete in the fall of 2019. The Biologics License

Application (BLA) submission for AT132 is planned for mid-2020, and the Marketing Authorization Application (MAA) submission is planned for the second half of 2020. In October 2019, Audentes Therapeutics, Inc. announced new data from ASPIRO at the 24th International Annual Congress of the World Muscle Society. AT132 uses the NAV AAV8 vector.

- In September 2019, Ultragenyx Pharmaceutical, Inc. presented positive data from the second dose cohort of the ongoing Phase 1/2 study of DTX401 for the treatment of glycogen storage disease type Ia (GSDIa) at the Society for the Study of Inborn Errors of Metabolism (SSIEM) 2019 Annual Symposium. DTX401 uses the NAV AAV8 vector.
- In July 2019, REGENXBIO granted a non-exclusive worldwide license to the NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of Friedreich's ataxia to Pfizer, Inc.

Financial Results

Cash, cash equivalents and marketable securities were \$417.1 million as of September 30, 2019, compared to \$470.6 million as of December 31, 2018. The decrease in cash, cash equivalents and marketable securities during the nine months ended September 30, 2019, was primarily attributable to \$82.2 million of net cash used in operating activities during the period, partially offset by an unrealized gain of \$29.4 million related to our marketable equity securities of Prevail Therapeutics Inc.

Revenues were \$14.7 million for the three months ended September 30, 2019, compared to \$5.3 million for the three months ended September 30, 2018. The increase was primarily attributable \$9.2 million of royalty revenue recognized during the third quarter of 2019 related to net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019, and we are also eligible to receive a milestone payment of \$80.0 million from AveXis, Inc. upon the achievement of \$1 billion in cumulative net sales of Zolgensma.

Research and development expenses were \$35.7 million for the three months ended September 30, 2019, compared to \$18.5 million for the three months ended September 30, 2018. The increase was primarily attributable to personnel costs as a result of increased headcount, laboratory and facilities costs and external expenses associated with conducting clinical trials and manufacturing-related services.

General and administrative expenses were \$12.4 million for the three months ended September 30, 2019, compared to \$9.0 million for the three months ended September 30, 2018. The increase was primarily attributable to personnel costs as a result of increased headcount.

Net loss was \$34.6 million, or \$0.94 basic and diluted net loss per share, for the three months ended September 30, 2019, compared to net loss of \$19.2 million, or \$0.56 basic and diluted net loss per share, for the three months ended September 30, 2018.

Financial Guidance

Based on its current operating plan, REGENXBIO now expects its balance in cash, cash equivalents and marketable securities to be at least \$365 million as of December 31, 2019.

Conference Call

In connection with this announcement, REGENXBIO will host a conference call and webcast 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 1159968. 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 includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, research and development activities, preclinical studies, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to 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, 2018, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results

or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any 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>September 30, 2019</u>	<u>December 31, 2018</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 79,594	\$ 75,561
Marketable securities	252,125	244,200
Accounts receivable	19,175	8,587
Prepaid expenses	6,920	5,734
Other current assets	2,414	3,831
Total current assets	360,228	337,913
Marketable securities	85,408	150,819
Accounts receivable	24,345	23,012
Property and equipment, net	28,287	28,702
Operating lease right-of-use assets	5,905	—
Restricted cash	1,053	1,053
Other assets	4,011	2,315
Total assets	<u>\$ 509,237</u>	<u>\$ 543,814</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 9,608	\$ 4,412
Accrued expenses and other current liabilities	19,231	17,164
Deferred revenue	—	600
Operating lease liabilities	2,506	—
Total current liabilities	31,345	22,176
Deferred revenue	3,333	3,333
Operating lease liabilities	4,320	—
Deferred rent	—	1,098
Financing lease obligations	—	5,854
Other liabilities	1,844	2,505
Total liabilities	40,842	34,966
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2019 and December 31, 2018	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at September 30, 2019 and December 31, 2018; 36,840 and 36,120 shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	619,386	592,580
Accumulated other comprehensive income (loss)	363	(720)
Accumulated deficit	(151,358)	(83,016)
Total stockholders' equity	468,395	508,848
Total liabilities and stockholders' equity	<u>\$ 509,237</u>	<u>\$ 543,814</u>

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

	<u>Three Months Ended</u>		<u>Nine Months Ended</u>	
	<u>September 30,</u>		<u>September 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Revenues				
License and royalty revenue	\$ 14,700	\$ 5,306	\$ 23,465	\$ 177,728
Total revenues	14,700	5,306	23,465	177,728
Operating Expenses				
Cost of revenues	2,494	517	4,450	6,797
Research and development	35,692	18,508	90,378	59,544
General and administrative	12,402	9,008	37,365	25,706

Other operating expenses (income)	8	(2)	(54)	31
Total operating expenses	50,596	28,031	132,139	92,078
Income (loss) from operations	(35,896)	(22,725)	(108,674)	85,650
Other Income				
Interest income from licensing	716	109	2,091	8,362
Investment income	431	2,122	37,950	4,177
Total other income	1,147	2,231	40,041	12,539
Income (loss) before income taxes	(34,749)	(20,494)	(68,633)	98,189
Income Tax Benefit (Expense)	165	1,292	364	(2,558)
Net income (loss)	<u>\$ (34,584)</u>	<u>\$ (19,202)</u>	<u>\$ (68,269)</u>	<u>\$ 95,631</u>
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	(108)	(103)	1,043	(159)
Total other comprehensive income (loss)	(108)	(103)	1,043	(159)
Comprehensive income (loss)	<u>\$ (34,692)</u>	<u>\$ (19,305)</u>	<u>\$ (67,226)</u>	<u>\$ 95,472</u>
Net income (loss) per share:				
Basic	<u>\$ (0.94)</u>	<u>\$ (0.56)</u>	<u>\$ (1.86)</u>	<u>\$ 2.94</u>
Diluted	<u>\$ (0.94)</u>	<u>\$ (0.56)</u>	<u>\$ (1.86)</u>	<u>\$ 2.67</u>
Weighted-average common shares outstanding:				
Basic	36,813	33,988	36,618	32,576
Diluted	36,813	33,988	36,618	35,875

Zolgensma® is a registered trademark of AveXis. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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