REGENXBIO Reports Second Quarter 2020 Financial Results and Operational Highlights

August 6, 2020 8:05 PM EDT

-- RGX-314 subretinal delivery pivotal program for wet AMD to initiate by end of 2020; positive interim update from Cohorts 4 and 5 at one year in Phase I/IIa trial recently announced

-- Phase II trial for RGX-314 for wet AMD using suprachoroidal delivery expected to begin enrollment in Q3 2020

-- Phase II trial for RGX-314 for diabetic retinopathy on track to initiate in second half of 2020

-- Recently reported progress in RGX-111 and RGX-121 programs; additional program updates are expected later this year

-- Company announces new program, RGX-381, for the treatment of ocular manifestations of CLN2 disease

-- Company announces discontinuation of internal clinical development of RGX-501 for HoFH

-- $339 million in cash, cash equivalents and marketable securities as of June 30, 2020

-- Q2 earnings conference call Thursday, August 6 at 4:30 p.m. ET

ROCKVILLE, Md., Aug. 6, 2020 /PRNewswire/ -- 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 June 30, 2020, and recent operational highlights.

"Now halfway through 2020 and with the backdrop of the challenging COVID-19 pandemic, I am pleased that we have made significant progress in our gene therapy programs and continue to make important decisions with respect to our pipeline and plans. We expect to initiate the RGX-314 wet AMD pivotal program later this year based on the latest data from our RGX-314 Phase I/IIa trial, which has demonstrated durable treatment effect, with stable to improved visual acuity and retinal thickness. We also look forward to dosing patients in our RGX-314 trials using the suprachoroidal delivery approach for the treatment of wet AMD and diabetic retinopathy later this year," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Our team has also been hard at work moving our CNS-focused gene therapy programs forward. We have completed dosing of patients in the second cohort of our Phase I/II study of RGX-121 for the treatment of MPS II, which includes one patient dosed in Brazil at our first ex-U.S. gene therapy site. We look forward to providing additional updates for this program, as well as our RGX-111 program for the treatment of MPS I, later this year."

Mr. Mills continued: "We are committed to continuously evaluating our portfolio of gene therapy candidates, with innovation and patient needs in mind. Today, we have announced the expansion of our pipeline to include RGX-381 for the treatment of ocular manifestations of CLN2 disease, also known as Batten disease. This will complement our existing RGX-181 program for the treatment of CLN2 disease in the central nervous system. I look forward to providing additional details and updates for RGX-381 and RGX-181 in the coming months."

"Finally, we have made the difficult decision to discontinue the development of RGX-501 for the treatment of HoFH. This program was one of our first clinical-stage gene therapy programs, and we are grateful for the support of the HoFH patient community over the years. We will look for opportunities to support the continued advancement of this program through business development."

Recent Operational Highlights

Gene Therapy Using NAV Vectors for AAV-Mediated Antibody Delivery

- RGX-314 for the Treatment of Wet Age-related Macular Degeneration (wet AMD)
  - On August 4, 2020, REGENXBIO reported data from the RGX-314 Phase I/IIa trial using subretinal delivery. RGX-314 was generally well-tolerated as of July 13, 2020. For

Cohorts 4 and 5:
    - Durable treatment effect was observed with stable to improved visual acuity and retinal thickness at one year
    - Meaningful reductions in anti-VEGF treatment burden were demonstrated over one year
    - Dose-dependent intraocular RGX-314 protein expression levels were observed over one year
  - REGENXBIO expects to initiate a pivotal program for the subretinal delivery of RGX-314 for the treatment of wet AMD by the end of 2020.
  - REGENXBIO plans to dose patients in AAVIATE, the Phase II trial for the suprachoroidal delivery of RGX-314 using the SCS Microinjector™ for the treatment of wet AMD, in the third quarter of 2020.
    - Interim data is expected to be reported from the first cohort by the end of 2020.
- RGX-314 for the Treatment of Diabetic Retinopathy (DR)
  - REGENXBIO expects to initiate a Phase II trial in the second half of 2020 and interim data is expected to be reported in 2021.
- Research Program for the Treatment of Hereditary Angioedema (HAE)
  - Preclinical data from the HAE program was presented at the American Society of Gene and Cell Therapy (ASGCT) 23rd Annual Meeting in May 2020, demonstrating biological activity in a model of inflammatory edema and inhibition of kallikrein comparable to other anti-kallikrein treatments.
  - REGENXBIO expects to provide a program update in the second half of 2020.
- Research Program for the Treatment of Neurodegenerative Diseases
  - REGENXBIO continues to collaborate with Neurimmune AG to design and develop vectorized antibody therapies targeting both alpha synuclein and tau. REGENXBIO expects to provide a program update in the second half of 2020.

Gene Therapy Using NAV Vectors for Rare Genetic Diseases

- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
  - In July, REGENXBIO announced the completed dosing of three patients in Cohort 2 of its Phase I/II trial. As of June 24, 2020, RGX-121 was reported to be well-tolerated in patients across two dose levels, with no drug-related serious adverse events (SAEs).
  - The Company expects to provide a data and program update in the second half of 2020.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
  - In July, REGENXBIO reported data under a single-patient investigator-initiated Investigational New Drug application (IND) conducted at CHOC Children's, which demonstrated encouraging biomarker activity and continued progression of neurocognitive development. As of June 9, 2020, RGX-111 was reported to be well-tolerated in this patient, with no drug-related SAEs.
  - Recruitment and patient screening are ongoing in the Company's Phase I/II clinical trial evaluating RGX-111 for the treatment of MPS I.
  - REGENXBIO expects to provide a program update by the end of 2020.
- RGX-381 for the Treatment of Ocular Manifestations of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
  - RGX-381 is a new program targeting the ocular manifestations of CLN2 disease in patients and is designed to use the AAV9 vector to deliver the tripeptidyl peptidase 1 (TPP1) gene directly to the retina. REGENXBIO believes that one-time administration of RGX-381 could provide a durable source of TPP1 activity in the retina, thereby potentially preventing visual decline. There is currently no available treatment for ocular

manifestations of CLN2 disease.
    - REGENXBIO expects to submit an IND for a Phase I/II study of RGX-381 in patients with CLN2 disease in the second half of 2020 and plans to initiate enrollment in the first half of 2021.
    - The company has initiated two non-interventional natural history studies to assess ocular manifestations in CLN2 disease.
    - The Orphan Drug Designation and Rare Pediatric Disease Designation granted to the RGX-181 program also apply to the RGX-381 program.
- RGX-181 for the Treatment of CLN2 Disease
    - REGENXBIO expects to submit an IND for the intracisternal delivery of RGX-181 by the end of 2020, and plans to initiate enrollment in a Phase I/II trial in the first half of 2021.
- RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)
    - REGENXBIO has discontinued internal clinical development of RGX-501 for the treatment of HoFH in order to focus on furthering other gene therapy programs in its pipeline. The Company plans to evaluate strategic alternatives to support the continued advancement of this program, including through partnering.
- Research Program for the Treatment of Neuromuscular Disorders
    - REGENXBIO expects to announce plans for clinical development of a potential treatment for a neuromuscular disorder using NAV AAV8 in the second half of 2020.

Operational Updates

- Current Good Manufacturing Practice (cGMP) Manufacturing Facility
    - Construction of a new corporate, research and manufacturing headquarters in Rockville, Maryland continues, with plans to begin utilizing the new headquarters in the first half of 2021.The new cGMP production facility is expected to allow for production of NAV vectors at scales up to 2,000 liters using REGENXBIO's platform suspension cell culture process, which will complement REGENXBIO's current external manufacturing network and capabilities. The cGMP facility is expected to be operational starting in late 2021.

Marketed NAV Technology Product Highlights

REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma®. On July 21, 2020, Novartis AG reported second quarter 2020 U.S. Zolgensma sales revenue of $205 million, and REGENXBIO recognized royalty revenue of $12 million in the second quarter of 2020 as a result of these sales. Total net sales of Zolgensma since launch in May 2019 are more than $735 million. REGENXBIO is eligible to receive a milestone payment of $80.0 million from Novartis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $339.2 million as of June 30, 2020, compared to $400.0 million as of December 31, 2019. The decrease was primarily attributable to net cash flows used in operating activities of $57.1 million.

Revenues: Revenues were $16.6 million for the three months ended June 30, 2020, compared to $7.9 million for the three months ended June 30, 2019. The increase was primarily attributable to an $11.0 million increase in royalty revenue recognized on net sales of Zolgensma in the second quarter of 2020 as compared to the second quarter of 2019.

Research and Development Expenses: Research and development expenses were $38.1 million for the three months ended June 30, 2020, compared to $29.5 million for the three months ended June 30, 2019. The increase was primarily attributable to personnel-related costs as a result of increased headcount, expenses associated with conducting clinical trials for our lead product candidates, laboratory and facilities costs, and externally sourced services for preclinical, regulatory and manufacturing-related activities.

General and Administrative Expenses: General and administrative expenses were $15.6 million for the three months ended June 30, 2020, compared to $13.4 million for the three months ended June 30, 2019. The increase was primarily attributable to personnel-related costs as a result of increased headcount and professional fees for advisory and other services.

Net Loss: Net loss was $33.8 million, or $0.91 basic and diluted net loss per share, for the three months ended June 30, 2020, compared to net loss of $1.5 million, or $0.04 basic and diluted net loss per share, for the three months ended June 30, 2019.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $339.2 million to fund the completion of its internal manufacturing capabilities and clinical advancement of its product candidates into 2022.

Q2 Earnings 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 (800) 708-4539 (domestic) or (847) 619-6396 (international) and enter the passcode 49857700. 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, 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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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, 2019, 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)

	June 30, 2020	December 31, 2019
Assets		
Current assets		
Cash and cash equivalents	$ 94,222	$ 69,514
Marketable securities	174,964	226,696
Accounts receivable	42,876	38,148
Prepaid expenses	9,749	6,475
Other current assets	7,271	4,199
Total current assets	329,082	345,032
Marketable securities	70,054	103,785
Accounts receivable	3,618	4,155
Property and equipment, net	32,696	28,973
Operating lease right-of-use assets	8,635	10,078
Restricted cash	1,330	1,330
Other assets	4,323	4,555
Total assets	$ 449,738	$ 497,908
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 10,901	$ 6,409
Accrued expenses and other current liabilities	25,839	24,846
Deferred revenue	450	—
Operating lease liabilities	3,013	2,421
Total current liabilities	40,203	33,676
Deferred revenue	4,007	3,333
Operating lease liabilities	7,085	8,874
Other liabilities	582	1,828
Total liabilities	51,877	47,711
Stockholders' equity		

Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2020 and December 31, 2019	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at June 30, 2020 and December 31, 2019; 37,291 and 36,992 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	648,729	627,810
Accumulated other comprehensive income	750	205
Accumulated deficit	(251,622)	(177,822)
Total stockholders' equity	397,861	450,197
Total liabilities and stockholders' equity	$ 449,738	$ 497,908

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,	
	2020	2019	2020	2019
Revenues				
License and royalty revenue	$ 16,566	$ 7,881	$ 34,210	$ 8,765
Total revenues	16,566	7,881	34,210	8,765
Operating Expenses				
Cost of revenues	4,684	1,927	8,093	1,956
Research and development	38,111	29,483	75,146	54,686
General and administrative	15,554	13,405	30,387	24,963
Other operating expenses (income)	50	(62)	117	(62)
Total operating expenses	58,399	44,753	113,743	81,543
Loss from operations	(41,833)	(36,872)	(79,533)	(72,778)
Other Income				
Interest income from licensing	1,849	762	2,697	1,375
Investment income	5,722	34,524	2,536	37,519
Total other income	7,571	35,286	5,233	38,894
Loss before income taxes	(34,262)	(1,586)	(74,300)	(33,884)
Income Tax Benefit	500	129	500	199
Net loss	$ (33,762)	$ (1,457)	$ (73,800)	$ (33,685)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities, net	1,330	530	545	1,151
Total other comprehensive income	1,330	530	545	1,151
Comprehensive loss	$ (32,432)	$ (927)	$ (73,255)	$ (32,534)
Basic and diluted net loss per share	$ (0.91)	$ (0.04)	$ (1.98)	$ (0.92)
Weighted-average basic and diluted common shares outstanding	37,257	36,669	37,180	36,518

Contacts:

Tricia Truehart
Investor Relations and Corporate Communications
347-926-7709
ttruehart@regenxbio.com

Investors:
Eleanor Barisser, 212-600-1902
eleanor@argotpartners.com

Media:
David Rosen, 212-600-1902
david.rosen@argotpartners.com

View original content to download multimedia:http://www.prnewswire.com/news-releases/regenxbio-reports-second-quarter-2020-financial-results-and-operational-highlights-301107904.html

SOURCE REGENXBIO Inc.