REGENXBIO Reports Second Quarter 2019 Financial and Operating Results and Additional Positive Interim Phase I/IIa Trial Update for RGX-314 for the Treatment of Wet AMD

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- *Reports further positive interim update from RGX-314 Phase I/IIa trial for Wet AMD*
  - *Well-tolerated at all doses*
  - *Dose-dependent protein expression levels observed across all five dose cohorts*
  - *50% of subjects treated in Cohort 3 remain free of anti-VEGF injections at 18 months*
  - *Company plans to present interim data update from all five dose cohorts in October 2019*
  - *Remains on-track to initiate Phase IIb trial in late 2019*
- *Continues dosing in the RGX-501 Phase I/II trial for HoFH and the RGX-121 Phase I/II trial for MPS II*
- *Expanded pipeline using NAV® Vectors to deliver therapeutic antibodies for the treatment of hereditary angioedema and neurodegenerative diseases*
- *Launch of first FDA-approved gene therapy based on REGENXBIO's NAV Technology Platform, Novartis' Zolgensma® for the treatment of SMA*
- *$450 million in cash, cash equivalents and marketable securities as of June 30, 2019*
- *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 June 30, 2019, and recent operational highlights.

"We are encouraged by the continued positive interim data from the Phase I/IIa RGX-314 trial and the potential of NAV gene therapy as a one-time treatment for wet AMD, and look forward to sharing six-month results from all five cohorts in the trial in October 2019," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "In our clinical trial for wet AMD, we have seen dose-dependent increases in protein expression levels across cohorts, and 50% of Cohort 3 subjects continued to be free of anti-VEGF injections at 18 months following a single administration of our gene therapy. These findings are particularly encouraging, given the severity of the disease and the high anti-VEGF injection treatment burden of these enrolled patients prior to administration of RGX-314. With feedback from the FDA provided at our recent Type B meeting, we also look forward to our anticipated start of the Phase IIb trial in late 2019."

Mr. Mills added: "In addition to our excitement around RGX-314, the past quarter was highlighted by important milestones for REGENXBIO, including the FDA approval and launch of Zolgensma® —the first FDA-approved gene therapy based on REGENXBIO's NAV Technology Platform. We recently announced the expansion of our pipeline using NAV Vectors to deliver therapeutic antibodies, beginning with novel treatments for hereditary angioedema and chronic neurodegenerative diseases; this approach has enormous potential for patients who lack treatments or who are currently underserved by existing therapies. We currently have an internal pipeline of novel gene therapy candidates across large, chronic diseases and rare, monogenic diseases, which positions us to potentially develop meaningful treatments for a broad spectrum of patients with significant unmet needs."

Lead Product Candidate Updates

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

- RGX-314 for the Treatment of Wet AMD
  - As of July 31, 2019, 42 subjects across five dose cohorts have been treated in the Phase I/IIa trial of RGX-314. RGX-314 continues to be well-tolerated across all five dose cohorts, with no drug related serious adverse events (SAEs) reported.
  - Dose-dependent increases in RGX-314 protein expression levels, as measured from aqueous samples by electrochemiluminescence immunoassay (ECL) at approximately one month after administration of RGX-314, have been observed across all doses.
  - 50% of subjects (3/6) in Cohort 3 continue to remain injection-free at 18 months.

    - REGENXBIO expects to present long-term clinical data from Cohort 3 out to 18 months and interim data from Cohort 4 and Cohort 5 in October 2019.
    - Following the Type B meeting with the U.S. Food and Drug Administration (FDA) held in July 2019, REGENXBIO remains on track to initiate a Phase IIb trial for wet AMD in late 2019.
- RGX-314 for the Treatment of Diabetic Retinopathy (DR)
    - REGENXBIO is on track to file a new Investigational New Drug (IND) application for a Phase II trial evaluating RGX-314 in subjects with DR in the second half of 2019.
- Hereditary Angioedema (HAE)
    - REGENXBIO continues work on the HAE program and expects to provide an update in early 2020 on the preclinical studies, as well as plans for entering clinical trials.
- Neurodegenerative Disease
    - REGENXBIO, in collaboration with Neurimmune AG, has initiated work to jointly develop novel therapies using AAV vectors to deliver human antibodies targeting abnormal tau and will provide program updates as the collaboration advances.

Gene Therapy using NAV Vectors for Rare Genetic Diseases:

- RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia (HoFH)
    - Dosing resumed and recruitment continues in the Phase I/II clinical trial evaluating RGX-501 for the treatment of HoFH.
    - REGENXBIO expects to report interim data from Cohort 2 with corticosteroid prophylaxis from the Phase I/II clinical trial in the second half of 2019.
- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
    - Recruitment, screening, and dosing continue in the first of two dose cohorts in the Phase I/II clinical trial evaluating RGX-121 for the treatment of MPS II.
    - REGENXBIO expects to present an interim data update from the Phase I/II clinical trial in the second half of 2019.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
    - REGENXBIO expects to file an IND or foreign equivalent for the first-in-human clinical trial evaluating RGX-181 for the treatment of CLN2 in the second half of 2019.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
    - Recruitment, screening and additional site activations are ongoing in the Phase I clinical trial evaluating RGX-111 for the treatment of MPS I.

Other Recent Operational Highlights

- In May 2019, REGENXBIO announced its plans to construct a current good manufacturing practice (cGMP) production facility in Rockville, Maryland. The new cGMP production facility will be integrated into REGENXBIO's previously announced new 139,000 square foot headquarters, for which construction is currently underway, and will 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 will be designed to meet global regulatory requirements and is expected to be operational in 2021.

NAV Technology Licensee Program Highlights

As of July 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. Over 200 subjects have been treated in clinical trials sponsored by NAV Technology Licensees. 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

- In May 2019, Novartis announced the FDA approval of Zolgensma as a one-time infusion for pediatric patients with spinal muscular atrophy (SMA) who are less than two years of age. Novartis has stated that it anticipates regulatory approvals of Zolgensma in Japan and Europe in the second half of 2019. REGENXBIO earned a $3.5 million milestone payment upon Zolgensma's approval in the United States and is eligible to receive an additional $80 million milestone payment upon the achievement of $1 billion in cumulative net sales of Zolgensma. REGENXBIO will receive tiered royalties on sales of Zolgensma up to a low double-digit percentage. Zolgensma uses the NAV AAV9 vector.

Partnered Product Candidates

- In July 2019, REGENXBIO announced that it granted a non-exclusive worldwide license for its NAV AAV9 vector to Pfizer Inc. for the development and commercialization of gene therapy for the treatment of Friedreich's ataxia, the most common hereditary ataxia. In return, Pfizer will provide REGENXBIO with an upfront payment, and REGENXBIO has the potential to receive ongoing fees, development and commercial milestone payments and royalties on net sales of products incorporating the NAV AAV9 vector.
- In July 2019, Abeona Therapeutics, Inc. announced positive data from its ongoing Phase I/II clinical trial evaluating ABO-102 for the treatment of Sanfilippo syndrome type A (MPS IIIA). ABO-102 uses the NAV AAV9 vector.
- In June 2019, Rocket Pharmaceuticals, Inc. announced it dosed the first subject in its Phase I clinical trial of RP-A501 for the treatment of Danon disease. RP-A501 uses the NAV AAV9 vector.

Financial Results

Cash, cash equivalents and marketable securities were $449.7 million as of June 30, 2019, compared to $470.6 million as of December 31, 2018. Cash, cash equivalents and marketable securities as of June 30, 2019 include $32.1 million of marketable equity securities of Prevail Therapeutics Inc.REGENXBIO is a co-founder of Prevail and currently owns 2.43 million shares of Prevail common stock. Prior to Prevail's initial public offering (IPO) in June 2019, REGENXBIO's equity interests in Prevail had a carrying value of $0.4 million and were included in other assets on the consolidated balance sheet. Upon the completion of Prevail's IPO in June 2019, the securities were reclassified to marketable securities, and subsequently remeasured at fair value. The decrease in cash, cash equivalents and marketable securities during the six months ended June 30, 2019, was primarily attributable to $53.0 million of net cash used in operating activities during the period, partially offset by the unrealized gain of $31.7 million related to the marketable equity securities of Prevail.

Revenues were $7.9 million for the three months ended June 30, 2019, compared to $40.0 million for the three months ended June 30, 2018. The decrease was primarily attributable to $40.0 million of non-recurring license revenue recognized during the three months ended June 30, 2018 under our amended license agreement with AveXis Inc. for the development and commercialization of treatments for SMA. The decrease was partially offset by revenue recognized during the three months ended June 30, 2019 resulting from license options exercised and development milestones achieved by licensees during the period, as well as royalties on net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019, and we expect royalties to increase in future periods. We are also eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1 billion in cumulative net sales of Zolgensma.

Research and development expenses were $29.5 million for the three months ended June 30, 2019, compared to $21.5 million for the three months ended June 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 $13.4 million for the three months ended June 30, 2019, compared to $8.3 million for the three months ended June 30, 2018. The increase was primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services.

Net loss was $1.5 million, or $0.04 basic and diluted net loss per share, for the three months ended June 30, 2019, compared to net income of $10.6 million, or $0.33 basic and $0.30 diluted net income per share, for the three months ended June 30, 2018. Net loss for the three months ended June 30, 2019, includes the unrealized gain of $31.7 million on REGENXBIO's marketable equity securities of Prevail recognized during the period.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities to be at least $350 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 6839996. 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)

	June 30, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	$ 55,142	$ 75,561
Marketable securities	286,354	244,200
Accounts receivable	9,679	8,587
Prepaid expenses	6,036	5,734
Other current assets	2,281	3,831
Total current assets	359,492	337,913
Marketable securities	108,194	150,819
Accounts receivable	23,955	23,012
Property and equipment, net	27,330	28,702
Operating lease right-of-use assets	5,904	—
Restricted cash	1,053	1,053
Other assets	3,211	2,315
Total assets	$ 529,139	$ 543,814
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 5,851	$ 4,412
Accrued expenses and other current liabilities	16,534	17,164
Deferred revenue	—	600
Operating lease liabilities	2,276	—
Total current liabilities	24,661	22,176
Deferred revenue	3,333	3,333
Operating lease liabilities	4,654	—
Deferred rent	—	1,098
Financing lease obligations	—	5,854
Other liabilities	1,899	2,505
Total liabilities	34,547	34,966
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2019 and December 31, 2018	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at June 30, 2019 and December 31, 2018; 36,752 and 36,120 shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	610,891	592,580

Accumulated other comprehensive income (loss)	471	(720)
Accumulated deficit	(116,774)	(83,016)
Total stockholders' equity	494,592	508,848
Total liabilities and stockholders' equity	$ 529,139	$ 543,814

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenues				
License and royalty revenue	$ 7,881	$ 40,031	$ 8,765	$ 172,422
Total revenues	7,881	40,031	8,765	172,422
Operating Expenses				
Cost of revenues	1,927	3,872	1,956	6,280
Research and development	29,483	21,486	54,686	41,036
General and administrative	13,405	8,318	24,963	16,698
Other operating expenses (income)	(62)	5	(62)	33
Total operating expenses	44,753	33,681	81,543	64,047
Income (loss) from operations	(36,872)	6,350	(72,778)	108,375
Other Income				
Interest income from licensing	762	6,898	1,375	8,253
Investment income	34,524	1,196	37,519	2,055
Total other income	35,286	8,094	38,894	10,308
Income (loss) before income taxes	(1,586)	14,444	(33,884)	118,683
Income Tax Benefit (Expense)	129	(3,850)	199	(3,850)
Net income (loss)	$ (1,457)	$ 10,594	$ (33,685)	$ 114,833
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	530	132	1,151	(56)
Total other comprehensive income (loss)	530	132	1,151	(56)
Comprehensive income (loss)	$ (927)	$ 10,726	$ (32,534)	$ 114,777
Net income (loss) per share:				
Basic	$ (0.04)	$ 0.33	$ (0.92)	$ 3.60
Diluted	$ (0.04)	$ 0.30	$ (0.92)	$ 3.29
Weighted-average common shares outstanding:				
Basic	36,669	32,082	36,518	31,858
Diluted	36,669	35,272	36,518	34,884

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