REGENXBIO Reports Second Quarter 2023 Financial Results and Recent Operational Highlights

August 02, 2023 04:05 PM EDT

- *Continued progress on '5x'25' strategy to advance five AAV Therapeutics from REGENXBIO's internal pipeline and licensed programs into pivotal-stage or commercial products by 2025*
- *ABBV-RGX-314 program for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie*
  - *Interim results from Phase II AAVIATE® and ALTITUDE® trials of dose level 3 cohorts with short-course prophylactic steroid eye drops resulted in zero cases of intraocular inflammation*
  - *Additional interim efficacy data from the Phase II AAVIATE and ALTITUDE trials expected at upcoming medical conferences in second half 2023 and first half 2024*
  - *Interim data from Phase II Bridging Study evaluating subretinal delivery of ABBV-RGX-314 produced by the NAVXpress ™ platform process demonstrated the anticipated clinical profile in the low dose cohort: well-tolerated and with the majority of subjects (11/15, 73%) injection-free through six months*
- *Initial safety results from Cohort 1 of the Phase I/II AFFINITY DUCHENNE™ trial of RGX-202 support well-tolerated profile; initial microdystrophin protein expression levels from Cohort 1 to be presented at World Muscle Society meeting in October*
- *Completed enrollment of the Phase III part of CAMPSIITE™ clinical trial of RGX-121 for treatment of MPS II; remains on track to file BLA in 2024 using the accelerated approval pathway*
- *$415 million in cash, cash equivalents and marketable securities as of June 30, 2023; operational runway into 2025*
- *Conference call Wednesday, August 2, 2023, at 4:30 PM (EDT)*

ROCKVILLE, Md., Aug. 2, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the second quarter ended June 30, 2023, and recent operational highlights.

"We continue to stay on track advancing our '5x'25' vision to have five gene therapies either on the market or in late-stage development by 2025," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are executing against a transformational year for REGENXBIO, with multiple clinical milestones and updates anticipated in 2023. The encouraging safety and tolerability data for the high-dose cohorts of our in-office wet AMD and DR programs further validate the potential of the suprachoroidal space for AAV delivery to treat chronic retinal diseases. We successfully reached our goal of enrolling patients in CAMPSIITE clinical trial of RGX-121 for treatment of MPS II and reported initial safety results from the first cohort of the AFFINITY DUCHENNE study and expect to present initial efficacy data later this year. Overall, we're making excellent progress advancing our '5x'25' strategy to bring leading, ground-breaking AAV Therapeutics to patients in need."

PROGRAM HIGHLIGHTS AND MILESTONES

ABBV-RGX-314: ABBV-RGX-314 is an investigational one-time AAV therapeutic being developed in collaboration with AbbVie for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other additional chronic retinal conditions. ABBV-RGX-314 uses the NAV® AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF).

ABBV-RGX-314 is currently being evaluated in nine ongoing clinical trials, including two pivotal trials, a Phase II bridging study, a Long-term Follow-up study, and a Fellow Eye treatment study in patients with wet AMD, all utilizing subretinal delivery, as well as two Phase II clinical trials in patients with wet AMD and DR, and two corresponding Long-term Follow-up studies, all utilizing in-office suprachoroidal delivery.

- ABBV-RGX-314 Subretinal Delivery for the Treatment of Wet AMD
  - Enrollment is ongoing in ATMOSPHERE® and ASCENT™ pivotal trials and the Fellow

Eye treatment study for the treatment of patients with wet AMD using subretinal delivery. Material from REGENXBIO's NAVXpress™ platform process has been incorporated in these pivotal trials and is expected to be produced at REGENXBIO's Manufacturing Innovation Center (RMIC) for future commercialization of ABBV-RGX-314. These trials are expected to support global regulatory submissions with the U.S. Food and Drug Agency (FDA) and the European Medicines Agency (EMA) in late 2025 through the first half of 2026.
    - A Phase II pharmacodynamic study designed to evaluate the same dose levels being used in the two pivotal trials is now fully enrolled. In July 2023, updated interim data was presented at the American Society of Retina Specialists annual meeting, demonstrating that ABBV-RGX-314 manufactured using REGENXBIO's NAVXpress platform process was well tolerated at both dose levels, initial data in the low dose cohort through 6 months exhibited expected protein levels along with stable to improved best corrected visual acuity and central retinal thickness, as well as meaningful reductions in anti-VEGF burden, with most subjects (11/15, 73%) remaining injection-free.

- ABBV-RGX-314 Suprachoroidal Delivery for Treatment of Wet AMD and DR
    - In July 2023, REGENXBIO presented interim data from the Phase II AAVIATE® & ALTITUDE® trials demonstrating that ABBV-RGX-314 suprachoroidal delivery administered to patients in cohorts at dose level 3 ($1.0x10^{12}$ genome copies per eye) with short-course (seven-week) prophylactic topical steroid eye drops (N=39) resulted in zero cases of intraocular inflammation. Time of post-administration follow up ranged from six weeks to six months.
    - REGENXBIO expects to report additional interim data from the ALTITUDE trial, including full twelve-month results from Cohorts 1-3, at the American Academy of Ophthalmology meeting (November 3-6, 2023) and from the AAVIATE trial, including full six-month results from Cohorts 5 and 6, at the Hawaiian Eye and Retina meeting (January 13-19, 2024).

RGX-202: RGX-202 is an investigational one-time AAV therapeutic for Duchenne Muscular Dystrophy (Duchenne), using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

- In July 2023, REGENXBIO reported that two Duchenne patients have received doses of RGX-202. As of July 6, 2023, RGX-202 was reported to be well-tolerated in the patients dosed, aged 4 and 10 years, with no drug-related serious adverse events. Time of post-administration follow up was 45 days and more than three months. The Phase I/II AFFINITY DUCHENNE™ trial continues to recruit patients (aged 4 to 11 years)REGENXBIO is using commercial-scale cGMP material from the RMIC in the clinical trial.
- REGENXBIO expects to report additional interim data of the AFFINITY DUCHENNE trial, including longer-term safety and microdystrophin protein expression levels in muscle at three months, at the World Muscle Society Congress (October 3-7, 2023).

RGX-121: RGX-121 is an investigational one-time AAV therapeutic for the treatment of mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase enzyme.

- Today REGENXBIO announced it has completed enrollment of 10 MPS II patients in the Phase I/II/III CAMPSIITE™ trial to support a Biologics License Application filing in 2024 using the accelerated approval pathway.
- In May 2023, REGENXBIO announced that the FDA granted regenerative medicine advanced therapy (RMAT) designation for RGX-121. RMAT designation is designed to expedite the drug

development and review processes for promising new treatments, including gene therapies, and recognizes that the preliminary clinical evidence from RGX-121 indicates its potential to address unmet medical needs for MPS II.

RGX-111: RGX-111 is an investigational one-time AAV therapeutic for the treatment of severe Mucopolysaccharidosis Type I (MPS I), also known as Hurler syndrome, using the NAV AAV9 vector to deliver the *α-l-iduronidase* gene.

- A Phase I/II trial of RGX-111 for the treatment of MPS I is fully enrolled with follow-up ongoing.
- REGENXBIO continues to expect to complete analytical characterization of recently manufactured commercial-scale cGMP material and share additional updates on program plans by the end of 2023.

RGX-181: RGX-181 is an investigational one-time AAV therapeutic for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease, using the NAV AAV9 vector to deliver the *tripeptidyl peptidase 1 (TPP1*) gene directly to the central nervous system.

- Physician investigators in Brazil continue with follow up for the first child with CLN2 disease dosed with RGX-181 under a single-patient investigator-initiated study.
- REGENXBIO expects investigators to report initial interim data from the single-patient investigator-initiated study, including 6-month results, at the Society for the Study of Inborn Errors of Metabolism Annual Symposium meeting being held August 29 to September 1, 2023.

RGX-381: RGX-381 is an investigational one-time AAV Therapeutic for the treatment of the ocular manifestations of CLN2 disease, using the NAV AAV9 vector to deliver the *TPP1* gene directly to the retina.

- Today, REGENXBIO announced dosing of the first patient in the Phase I/II trial of RGX-381.
- Initial data from this trial is expected to be shared in 2024.

RESEARCH & OPERATIONAL UPDATES

New Exon-Skipping Therapy for Duchenne Muscular Dystrophy

- In July 2023, REGENXBIO announced the development of a potential one-time gene therapy for Duchenne, which is based on a novel exon-skipping construct targeting exon 53 of the DMD gene. Company scientists have demonstrated that AAV-mediated exon skipping by the expression of antisense ribonucleic acid has the potential to restore high and sustained levels of near full-length dystrophin in Duchenne patients with relevant mutations in the DMD gene.
- REGENXBIO is initiating Investigational New Drug (IND) application-enabling studies and expects to submit an IND to the FDA in the first half of 2025.
- The RMIC in Maryland is fully operational, producing cGMP bulk substance lots to support programs using the NAVXpress platform process. REGENXBIO is one of only a few gene therapy companies worldwide with a cGMP facility capable of production at scales up to 2,000 liters. The company continues to use the facility to produce commercial-scale cGMP material for its entire clinical pipeline and performance qualification lots to support planned regulatory filings for ABBV-RGX-314 and RGX-121.

NAV TECHNOLOGY PLATFORM LICENSEE PROGRAM HIGHLIGHTS

As of June 30, 2023, REGENXBIO's NAV Technology Platform was being applied in one marketed product and multiple clinical-stage partnered programs, with the potential to impact a broad range of therapeutic areas and disease indications.

- Zolgensma®, a one-time AAV Therapeutic for the treatment of spinal muscular atrophy, is a marketed product utilizing REGENXBIO's NAV AAV9 vector. In July 2023, Novartis AG reported second quarter 2023 global sales of Zolgensma of $311 million.
- In May 2023, Rocket Pharmaceuticals announced that the EMA has granted Priority Medicines designation to RP-A501 for the treatment of Danon Disease. RP-A501 is being

developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.

- In May 2023, Ultragenyx Pharmaceutical Inc. announced the completion of dosing in the Phase III study of DTX401 for the treatment of Glycogen Storage Disease Type Ia and expects data in the first half of 2024. DTX401 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV8 vector.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $415.4 million as of June 30, 2023, compared to $565.2 million as of December 31, 2022. The decrease was primarily driven by cash used to fund operating activities during the first half of 2023.

Revenues: Revenues were $20.0 million for the three months ended June 30, 2023, compared to $32.6 million for the three months ended June 30, 2022. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased from $28.4 million in the second quarter of 2022 to $19.0 million in the second quarter of 2023.

Research and Development Expenses: Research and development expenses were $59.9 million for the three months ended June 30, 2023, compared to $61.0 million for the three months ended June 30, 2022. The decrease was primarily attributable to clinical trial and manufacturing expenses for ABBV-RGX-314 resulting from an increase in development cost reimbursement from AbbVie under our eye care collaboration, and was partially offset by an increase in clinical trial expenses for our other lead product candidates.

General and Administrative Expenses: General and administrative expenses were $23.7 million for the three months ended June 30, 2023, compared to $20.8 million for the three months ended June 30, 2022. The increase was primarily attributable to expenses for professional services and other corporate overhead costs.

Net Loss: Net loss was $72.1 million, or $1.66 basic and diluted net loss per share, for the three months ended June 30, 2023, compared to a net loss of $68.2 million, or $1.58 basic and diluted net loss per share, for the three months ended June 30, 2022.

FINANCIAL GUIDANCE

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $415.4 million as of June 30, 2023, to fund its operations into 2025. This cash runway guidance is based on the Company's current operational plans and excludes the impact of any payments that may be received from AbbVie upon the achievement of development or commercial milestones under our ABBV-RGX-314 collaboration.

CONFERENCE CALL

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 4:30 p.m. ET. A live audio webcast will be available at **HERE**. Interested parties may also pre-register for the earnings conference call **HERE**. Once registration is completed, participants will be provided a dial-in number with a personalized conference code to access the call. Those who plan on participating are advised to dial in 15 minutes prior to the start time.

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, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a '5x'25' strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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," "assume," "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, 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, 2022, 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. Except as required by law, 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)

	June 30, 2023	December 31, 2022
Assets		
Current assets		
Cash and cash equivalents	$ 68,600	$ 96,952
Marketable securities	251,482	267,690
Accounts receivable	21,380	28,082
Prepaid expenses	15,112	13,900
Other current assets	22,235	9,352
Total current assets	378,809	415,976
Marketable securities	95,302	200,560
Accounts receivable, net	1,143	1,504
Property and equipment, net	138,680	141,685
Operating lease right-of-use assets	62,436	65,116
Restricted cash	2,255	2,030
Other assets	3,833	6,397
Total assets	$ 682,458	$ 833,268
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 11,639	$ 27,213
Accrued expenses and other current liabilities	50,517	46,794
Deferred revenue	448	1,829
Operating lease liabilities	6,326	5,997
Liability related to sale of future royalties	48,963	48,601
Total current liabilities	117,893	130,434
Operating lease liabilities	85,254	88,802
Liability related to sale of future royalties	67,377	89,005
Other liabilities	6,079	8,832
Total liabilities	276,603	317,073
Stockholders' equity		
Preferred stock; no shares issued and outstanding at June 30, 2023 and December 31, 2022	—	—
Common stock; 43,621 and 43,299 shares issued and outstanding at June 30, 2023 and December 31, 2022, respectively	4	4
Additional paid-in capital	996,239	973,145
Accumulated other comprehensive loss	(10,098)	(15,401)
Accumulated deficit	(580,290)	(441,553)
Total stockholders' equity	405,855	516,195
Total liabilities and stockholders' equity	$ 682,458	$ 833,268

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,	
	2023	**2022**	**2023**	**2022**
Revenues				
License and royalty revenue	$ 19,977	$ 32,649	$ 39,115	$ 54,867
Total revenues	19,977	32,649	39,115	54,867
Operating Expenses				
Cost of revenues	9,475	12,951	13,587	28,668
Research and development	59,886	61,008	118,402	116,635
General and administrative	23,698	20,832	46,332	43,150
Other operating expenses	26	391	59	474
Total operating expenses	93,085	95,182	178,380	188,927

Loss from operations	(73,108)	(62,533)	(139,265)	(134,060)
Other Income (Expense)				
Interest income from licensing	40	153	110	247
Investment income	2,127	1,061	4,293	1,860
Interest expense	(1,120)	(6,860)	(3,875)	(12,990)
Total other income (expense)	1,047	(5,646)	528	(10,883)
Loss before income taxes	(72,061)	(68,179)	(138,737)	(144,943)
Income Tax Benefit	—	—	—	41
Net loss	$ (72,061)	$ (68,179)	$ (138,737)	$ (144,902)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	1,524	(2,813)	5,303	(12,194)
Total other comprehensive income (loss)	1,524	(2,813)	5,303	(12,194)
Comprehensive loss	$ (70,537)	$ (70,992)	$ (133,434)	$ (157,096)
Net loss per share, basic and diluted	$ (1.66)	$ (1.58)	$ (3.19)	$ (3.37)
Weighted-average common shares outstanding, basic and diluted	43,531	43,111	43,491	43,028

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