Ocugen Provides Business Update with First Quarter 2024 Financial Results

May 14, 2024

Conference Call and Webcast Today at 8:30 a.m. ET

- *All three first-in-class modifier gene therapy product candidates currently in the clinic with OCU400 Phase 3 in progress*
- *OCU400 on track to meet 2026 Biologics License Application (BLA) and Market Authorization Application (MAA) approval targets*

MALVERN, Pa., May 14, 2024 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, and vaccines, today reported first quarter 2024 financial results along with a general business update.

“We’ve experienced several important clinical and regulatory milestones since the beginning of 2024 that we believe are leading the way to a new treatment paradigm for patients with blindness diseases,” said Dr. Shankar Musunuri, Chairman, Chief Executive Officer, and Co-Founder of Ocugen. “It’s very encouraging to have FDA clearance and EMA support for the Phase 3 clinical trial of our lead modifier gene therapy candidate that offers a potential one-time treatment for life.”

With FDA clearance to begin the Phase 3 liMeliGhT clinical trial, OCU400 becomes the first gene therapy to progress to late-stage trials with a broad retinitis pigmentosa (RP) indication. Until now, there has been only one marketed product to treat one of the 100 gene mutations associated with RP. The gene-agnostic mechanism of action for OCU400 provides hope for a much larger RP patient population. In the U.S. and Europe combined, RP affects nearly 300,000 people.

Ocugen expects to begin dosing patients in the Phase 3 liMeliGhT clinical trial in the second quarter of 2024. The Phase 3 trial will have a sample size of 150 participants—one arm of 75 participants with the *RHO* gene mutation and the other arm with 75 participants that are gene-agnostic. Luminance Dependent Navigation Assessment (LDNA) is the primary endpoint for the study and focuses on the proportion of responders, in treated and untreated groups, achieving an improvement of at least 2 Lux levels from baseline in the study eyes.

Leveraging a dual-track strategy, the Company plans to expand the Phase 3 OCU400 clinical trial in the second half of 2024 to include patients with Leber congenital amaurosis (LCA), contingent on favorable results from the Phase 1/2 study.

Modifier gene therapy has the potential to treat inherited retinal diseases as well as multifactorial blindness diseases affecting millions of patients. Leveraging the nuclear receptor gene RAR-related orphan receptor A (RORA), OCU410 is designed to regulate all four pathways involved with dry age-related macular degeneration (dAMD)—including lipid metabolism, inflammation, oxidative stress, and membrane attack complex (complement). Ocugen is developing OCU410 as a one-time gene therapy for the treatment of geographic atrophy (GA), an advanced stage of dAMD, affecting 2-3 million people in the U.S. and Europe combined. OCU410ST is being developed as a one-time gene therapy for the treatment of Stargardt disease, affecting approximately 100,000 people in the U.S. and Europe combined.

In April, dosing was completed in the second cohort (medium dose) of the Phase 1/2 ArMaDa clinical trial for OCU410. Dosing in the first cohort (low dose) of the Phase 1/2 GARDian trial for OCU410ST was completed earlier in the first quarter and in April 2024, the Data Safety and Monitoring Board approved the continuation to cohort 2 (medium dose).

“Our efforts in the first quarter of the year evidence the importance of our gene therapy programs and the need to operate the business to ensure their success,” said Dr. Musunuri. “We are opportunistic about Ocugen’s cell therapy and vaccine platforms, knowing that these technologies have great therapeutic and financial potential and are pursuing partnerships to support our entire pipeline.”

Ophthalmic Gene Therapies —First-in-class

- **OCU400** —Received FDA clearance of IND amendment to initiate OCU400 Phase 3 liMeliGhT clinical trial in RP. EMA provided acceptability of the U.S.-based trial for submission of Marketing Authorization Application (MAA). Currently, the multi-center Phase 3 clinical trial is in progress.
- **OCU410 –** Currently in Phase 1/2 stage of clinical development with active patient enrollment. Dosing is complete in the second cohort (medium dose) in the dose-escalation phase of the study. Once the third cohort (high dose) is complete, the Company will move into the Phase 2 clinical trial—the expansion phase—in the third quarter of 2024
- **OCU410ST** – Currently in Phase 1/2 stage of clinical development with active patient enrollment. Dosing is complete for cohort 1 (low dose). Initiated enrollment in cohort 2 (medium dose) in the dose-escalation phase of the trial.

Regenerative Cell Therapies—First-in-class

- **NeoCart®** – Completed renovating an existing facility into a current Good Manufacturing Practice ("GMP") facility in accordance with the FDA's regulations. Intend to initiate the Phase 3 trial, contingent on adequate availability of funding.

Vaccines Portfolio—First-in-class

- **Mucosal Vaccine Platform** – NIAID is collaborating with Ocugen on clinical development of OCU500. Planning to submit IND by mid-2024 to initiate Phase 1 clinical trial.

Biologics

- **OCU200** —Continue to work with FDA to address comments to lift the clinical hold.

First Quarter 2024 Financial Results

- The Company's cash and cash equivalents totaled $26.4 million as of March 31, 2024, compared to $39.5 million as of December 31, 2023. The Company had 257.3 million shares of common stock outstanding as of March 31, 2024.
- Total operating expenses for the three months ended March 31, 2024 were $13.2 million and included research and development expenses of $6.8 million and general and administrative expenses of $6.4 million. This compares to total operating expenses for the three months ended March 31, 2023 of $18.5 million that included research and development expenses of $10.2 million and general and administrative expenses of $8.3 million.
- Ocugen reported a $0.05 net loss per common share for the three months ended March 31, 2024 compared to a $0.08 net loss per common share for the three months ended March 31, 2023.

Conference Call and Webcast Details

Ocugen has scheduled a conference call and webcast for 8:30 a.m. ET today to discuss the financial results and recent business highlights. Ocugen's senior management team will host the call, which will be open to all listeners. There will also be a question-and-answer session following the prepared remarks.

Attendees are invited to participate on the call or webcast using the following details:

Dial-in Numbers: (800) 715-9871 for U.S. callers and (646) 307-1963 for international callers
Conference ID: 8699924
Webcast: Available on the events section of the Ocugen investor site

A replay of the call and archived webcast will be available for approximately 45 days following the event on the Ocugen investor site.

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies and vaccines that improve health and offer hope for patients across the globe. We are making an impact on patient's lives through courageous innovation—forging new scientific paths that harness our unique intellectual and human capital. Our breakthrough modifier gene therapy platform has the potential to treat multiple retinal diseases with a single product, and we are advancing research in infectious diseases to support public health and orthopedic diseases to address unmet medical needs. Discover more at www.ocugen.com and follow us on X and LinkedIn.

Cautionary Note on Forward-Looking Statements
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, including, but not limited to, strategy, business plans and objectives for Ocugen's clinical programs, plans and timelines for the preclinical and clinical development of Ocugen's product candidates, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, the ability to initiate new clinical programs; Ocugen's financial condition, statements regarding qualitative assessments of available data, potential benefits, expectations for ongoing clinical trials, anticipated regulatory filings and anticipated development timelines, which are subject to risks and uncertainties. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such statements are subject to numerous important factors, risks, and uncertainties that may cause actual events or results to differ materially from our current expectations, including, but not limited to, the risks that preliminary, interim and top-line clinical trial results may not be indicative of, and may differ from, final clinical data; that unfavorable new clinical trial data may emerge in ongoing clinical trials or through further analyses of existing clinical trial data; that earlier non-clinical and clinical data and testing of may not be predictive of the results or success of later clinical trials; and that that clinical trial data are subject to differing interpretations and assessments, including by regulatory authorities. These and other risks and uncertainties are more fully described in our annual and periodic filings with the Securities and Exchange Commission (SEC), including the risk factors described in the section entitled "Risk Factors" in the quarterly and annual reports that we file with the SEC. Any forward-looking statements that we make in this press release speak only as of the date of this press release. Except as required by law, we assume no obligation to update forward-looking statements contained in this press release whether as a result of new information, future events, or otherwise, after the date of this press release.

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OCUGEN, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands)
(Unaudited)

	March 31, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 26,375	$ 39,462
Prepaid expenses and other current assets	3,623	3,509
Total current assets	29,998	42,971
Property and equipment, net	17,654	17,290
Other assets	4,142	4,286
Total assets	$ 51,794	$ 64,547
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 1,731	$ 3,172
Accrued expenses and other current liabilities	12,434	13,343
Operating lease obligations	589	574
Current portion of long term debt	1,296	—
Total current liabilities	16,050	17,089
Non-current liabilities		
Operating lease obligations, less current portion	3,414	3,567
Long term debt, net	1,533	2,800
Other non-current liabilities	536	527
Total liabilities	21,533	23,983
Stockholders' equity		
Convertible preferred stock	1	1
Common stock	2,575	2,567
Treasury stock	(48)	(48)
Additional paid-in capital	325,799	324,191
Accumulated other comprehensive income	25	20
Accumulated deficit	(298,091)	(286,167)
Total stockholders' equity	30,261	40,564
Total liabilities and stockholders' equity	$ 51,794	$ 64,547

OCUGEN, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)
(Unaudited)

	Three months ended March 31,	
	2024	**2023**
Collaborative arrangement revenue	$ 1,014	$ 443
Total revenue	1,014	443
Operating expenses		
Research and development	6,826	10,172
General and administrative	6,404	8,306
Total operating expenses	13,230	18,478
Loss from operations	(12,216)	(18,035)
Other income (expense), net	292	709
Net loss	$ (11,924)	$ (17,326)
Shares used in calculating net loss per common share — basic and diluted	257,232,636	225,523,627
Net loss per share of common stock — basic and diluted	$ (0.05)	$ (0.08)