Ocugen Provides Business Update with Second Quarter 2024 Financial Results

August 8, 2024

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

- *Actively dosing patients in OCU400 Phase 3 liMeliGhT clinical trial*
- *OCU410 preliminary safety and efficacy data expected later this year*
- *Expanded access program approved for OCU400*
- *$32.6 million net cash from underwritten public offering of common stock*

MALVERN, Pa., Aug. 08, 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, biologics, and vaccines, today reported second quarter 2024 financial results along with a business update.

“The first half of 2024 has been marked with significant accomplishments for our modifier gene therapy platform—including dosing patients in the OCU400 Phase 3 clinical trial for retinitis pigmentosa (RP) and progressing into Phase 2 of the OCU410 ArMaDa clinical trial for the treatment of geographic atrophy (GA),” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “These meaningful milestones bring us closer to providing a potential one-time therapy for life for patients living with RP (300,000 in the U.S. and Europe) and GA (2-3 million in the U.S. and Europe) who desperately need effective treatment options. Thanks to our Ocugen team for their tireless efforts to keep these and all our clinical trials on track.”

The OCU400 Phase 3 trial has a sample size of 150 participants: one arm has 75 participants with *RHO* gene mutations, and the other arm has 75 participants with mutations in any of several other genes associated with RP. The Luminance Dependent Navigation Assessment (LDNA) is the primary endpoint for the study. In this assessment, a participant navigates an obstacle course that constitutes a more sensitive and specific measurement of visual function than the mobility measurement used in previous Phase 3 clinical trials. The Phase 3 liMeliGhT trial will focus on the proportion of responders, in treated and untreated groups, who achieve an improvement of at least 2 Lux (light) levels from baseline in the study eyes. More than 60% of the intent-to-treat patients from the Phase 1/2 clinical trial, including patients with the *RHO* mutation, meet the responder criteria established for Phase 3. The Phase 3 mobility test responder rate for the only FDA-approved product to treat one mutation in RP was 52%. The Phase 3 trial is powered greater than 95% assuming a 50% responder rate.

Recently, the FDA approved the OCU400 expanded access program (EAP) for the treatment of adult patients, aged 18 and older, with RP. This is the first ever gene therapy candidate to treat patients with RP, regardless of mutation, approved for an EAP and the EAP further supports the gene-agnostic mechanism of action for this novel modifier gene therapy.

Novel modifier gene therapy has the potential to address multiple inherited retinal diseases as well as multifactorial causes of blindness that affect millions of patients, like dry age-related macular degeneration (dAMD). OCU410 and OCU410ST aim to treat geographic atrophy secondary to dAMD and Stargardt disease, respectively. These modifier gene therapies leverage a nuclear hormone receptor gene called *RORA* (RAR-related orphan receptor A) as a potential one-time therapy for life with a single sub-retinal injection.

OCU410 is specifically designed to address multiple pathways implicated in the pathogenesis of dAMD and offers a distinct advantage over current treatment options that target only one pathway—the complement system—and require frequent intravitreal injections (about 6-12 doses per year), accompanied by various safety concerns, such as roughly 12% of patients progressing to wet AMD. OCU410 has the potential to regulate all four pathways related to disease progression—lipid metabolism, inflammation, oxidative stress, and the complement system—with a one-time sub-retina injection.

OCU410ST has received an Orphan Drug Designation from the FDA for the treatment of Stargardt disease, which has no approved treatment and affects approximately 100,000 people in the U.S. and Europe combined. The third cohort of the clinical trial is currently receiving the high dose. OCU410ST has the potential to be the first one-time gene therapy for Stargardt disease.

Ocugen continues to pursue strategic partnerships that will drive long-term strategy, and most importantly, will help patients access these novel modifier gene therapies globally. During the 2024 BIO International Convention, Ocugen engaged with potential partners and pharmaceutical executives to explore opportunities for the Company’s dynamic pipeline.

“Ocugen’s inclusion in the Russell Index in June further bolsters the value of our pipeline and recognizes the Company’s robust growth strategy,” said Dr. Musunuri. “This ranking supports our efforts to enable long-term shareholder value, garner significant visibility for Ocugen within the investment community, and broaden our shareholder base. I look forward to the second half of 2024 as we continue to solidify Ocugen’s position as a biotechnology leader.”

Subsequent to June 30, 2024, the Company closed a public offering of common stock with net proceeds of $32.6 million—extending its expected cash runway into the third quarter of 2025. The offering was led by a large premier mutual fund, along with participation from leading life sciences investors.

Ophthalmic Gene Therapies—First-in-Class

OCU400 – Ocugen is actively dosing subjects in the OCU400 Phase 3 liMeliGhT trial for the treatment of RP. With dosing of the Phase 3 trial underway, OCU400 remains on track for the 2026 BLA and MAA approval targets.

OCU410 – In July 2024, Ocugen announced the completion of dosing in the third cohort of the OCU410 Phase 1/2 ArMaDa clinical trial for the treatment of GA. To date, nine patients with GA have been dosed in the Phase 1/2 clinical trial (with low, medium, and high doses). Phase 2 of the clinical trial has been initiated and will assess the safety and efficacy of OCU410 in a larger group of patients who will be randomized into either of two treatment groups (medium or high dose) or a control group.

OCU410ST – Currently dosing the high dose of OCU410ST in the dose-escalation phase of the study.

Regenerative Cell Therapies—First-in-class

NeoCart® – Ocugen intends to initiate the Phase 3 trial contingent on the availability of adequate funding.

Vaccines Portfolio—First-in-class

Inhaled Mucosal Vaccine Platform – NIAID plans to submit an IND to initiate the OCU500 (COVID-19) Phase 1 clinical trial this year. Ocugen is continuing discussions with relevant government agencies as well as strategic partners regarding funding for the development of the OCU510 and OCU520 platforms.

Ophthalmic Biologic Product

OCU200 – Ocugen continues to work with the FDA to lift the clinical hold.

Second Quarter 2024 Financial Results

- Received $32.6 million net cash from underwritten public offering of common stock that closed on August 2, 2024.
- The Company's cash, cash equivalents, and restricted cash totaled $16.0 million as of June 30, 2024, compared to $39.5 million as of December 31, 2023. The Company had 257.4 million shares of common stock outstanding as of June 30, 2024.
- Total operating expenses for the three months ended June 30, 2024 were $16.6 million and included research and development expenses of $8.9 million and general and administrative expenses of $7.7 million. This compares to total operating expenses for the three months ended June 30, 2023 of $24.0 million that included research and development expenses of $14.5 million and general and administrative expenses of $9.5 million.
- Ocugen reported a $0.04 net loss per common share for the three months ended June 30, 2024 compared to a $0.10 net loss per common share for the three months ended June 30, 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 also will be a question-and-answer session following the prepared remarks.

Attendees are invited to participate on the call or webcast:

Dial-in Numbers: (800) 715-9871 for U.S. callers and (646) 307-1963 for international callers
Conference ID: 7453742
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, biologics, and vaccines that improve health and offer hope for patients across the globe. We are making an impact on patients' 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 and expected cash runway into the third quarter of 2025, 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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(Tables to follow)

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

	June 30, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 15,697	$ 39,462
Prepaid expenses and other current assets	2,920	3,509
Total current assets	18,617	42,971
Property and equipment, net	17,474	17,290
Restricted cash	302	—
Other assets	4,149	4,286
Total assets	$ 40,542	$ 64,547
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 3,391	$ 3,172
Accrued expenses and other current liabilities	12,814	13,343
Operating lease obligations	461	574
Current portion of long term debt	1,306	—
Total current liabilities	17,972	17,089
Non-current liabilities		
Operating lease obligations, less current portion	3,546	3,567
Long term debt, net	1,552	2,800
Other non-current liabilities	545	527
Total non-current liabilities	5,643	6,894
Total liabilities	23,615	23,983
Stockholders' equity		
Convertible preferred stock	—	1
Common stock	2,576	2,567
Treasury stock	(48)	(48)
Additional paid-in capital	327,742	324,191
Accumulated other comprehensive income	28	20
Accumulated deficit	(313,371)	(286,167)
Total stockholders' equity	16,927	40,564
Total liabilities and stockholders' equity	$ 40,542	$ 64,547

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

	Three months ended June 30,		Six months ended June 30,	
	2024	2023	2024	2023
Collaborative arrangement revenue	$ 1,141	$ 485	$ 2,155	$ 928
Total revenue	1,141	485	2,155	928

Operating expenses				
Research and development	8,902	14,574	15,728	24,746
General and administrative	7,688	9,451	14,092	17,757
Total operating expenses	16,590	24,025	29,820	42,503
Loss from operations	(15,449)	(23,540)	(27,665)	(41,575)
Other income (expense), net	169	475	461	1,184
Net loss	$ (15,280)	$ (23,065)	$ (27,204)	$ (40,391)
Net loss — basic and diluted	(15,280)	(23,065)	(27,204)	(40,391)
Redeemed Series B convertible preferred stock	4,988	—	4,988	—
Net loss available to common shareholders— basic and diluted	(10,292)	(23,065)	(22,216)	(40,391)
Shares used in calculating net loss per common share — basic and diluted	257,353,857	238,311,498	257,293,247	231,952,888
Net loss per share available to common shareholders — basic and diluted	$ (0.04)	$ (0.10)	$ (0.09)	$ (0.17)