Ocugen Provides Business Update with Second Quarter 2025 Financial Results

August 1, 2025

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

- *Initiated dosing in OCU410ST Phase 2/3 GARDian3 pivotal confirmatory clinical trial*
- *Actively dosing patients in OCU400 Phase 3 liMeliGhT clinical trial and on track for 2026 BLA filing*
- *OrthoCellix reverse merger intended to unlock the value of NeoCart/regenerative cell therapies and enable the Company to focus capital on modifier gene therapy platform*
- *Signed binding term sheet for exclusive Korean rights to OCU400 with upfront fees and near-term development milestone payments totaling up to $11 million*

MALVERN, Pa., Aug. 01, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today reported second quarter 2025 financial results along with a business update.

“While our modifier gene therapy clinical trials advance—now with two in late-stage—we are securing strategic partnerships and evolving the business to support three successful Biologics License Application (BLA) filings over the next three years,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “We have also made important appointments to our Board of Directors, Retina Scientific Advisory Board, and Leadership Team to provide the Company with scientific and strategic know-how to bring us closer to delivering paradigm-changing gene therapies to millions of people with blindness diseases.”

In June, the Company announced a proposed reverse merger with OrthoCellix, a wholly-owned subsidiary, and Carisma Therapeutics, Inc. to create a Nasdaq-listed, late clinical-stage regenerative cell therapy company with a first-in-class technology platform, focused on orthopedic diseases. The combined company will focus on the development of OrthoCellix’s NeoCart ® technology for the treatment of articular knee cartilage defects. Previously, NeoCart® received Regenerative Medicine Advanced Therapy (RMAT) designation and concurrence from the U.S. Food and Drug Administration (FDA) on a single, confirmatory Phase 3 clinical trial to enable submission of a BLA.

Aligned with Ocugen’s business development strategy to pursue regional partnerships for OCU400, the Company signed a binding term sheet to negotiate and enter into a licensing agreement with a well-established leader in the pharmaceutical and healthcare sector in Korea for exclusive Korean rights to OCU400. Pursuant to the term sheet, under the license agreement, in addition to the upfront and milestone fees, the Company will be entitled to sales milestones of $1 million for every $15 million of net sales in Korea in addition to a royalty of 25% on net sales of OCU400 generated by Ocugen’s partner. Ocugen will manufacture commercial supply of OCU400 under terms of a supply agreement. A regional approach preserves Ocugen’s rights to larger geographies to maximize total patient reach while also generating return for shareholders.

Following the FDA’s agreement to proceed with a Phase 2/3 GARDian3 pivotal confirmatory trial for OCU410ST for Stargardt disease, the agency granted Rare Pediatric Disease Designation (RPDD) to OCU410ST in May. This designation underscores the urgent need to address Stargardt disease, which remains a significant unmet medical need. Stargardt disease is an inherited retinal disorder that typically presents in childhood and affects approximately 100,000 people in the U.S. and Europe combined, and approximately 1 million globally. Currently, there is no FDA-approved treatment available for Stargardt disease.

The OCU410ST Phase 2/3 GARDian3 clinical trial is progressing well with the first patient dosed in July after FDA clearance in June. The GARDian3 clinical trial builds upon encouraging results and positive data from the Phase 1 GARDian trial, which demonstrated 48% slower lesion growth at 12-month follow-up in evaluable treated eyes compared to untreated eyes. Additionally, evaluable treated eyes showed a statistically significant (p=0.031) and clinically meaningful improvement of nearly 2-line/9-letter gain in best corrected visual acuity (BCVA) at 12-month follow-up when compared to untreated eyes.

Positive preliminary efficacy and safety data from the OCU410 Phase 1 ArMaDa clinical trial at 12 months demonstrated no drug-related serious adverse events (SAEs), 23% slower geographic atrophy (GA) lesion growth in treated eyes versus fellow eyes after a single injection, and 2-line/10-letter gain in visual acuity in treated eyes when compared to untreated fellow eyes. Preliminary results from ongoing Phase 2 clinical trial (N=31), 6-month interim analysis, demonstrated a 27% slower lesion growth and preservation of retinal tissue. These data support the potential for OCU410 to provide a one-time treatment for life for the 2-3 million people in the U.S. & EU combined who suffer from GA.

Patients are actively being recruited in the United States and Canada for the OCU400 Phase 3 liMeliGhT clinical trial, which remains on track for BLA and MAA submissions in 2026. This is the only broad retinitis pigmentosa (RP) gene-agnostic trial to address multiple genetic mutations with a single therapeutic approach. In addition, the European Medicines Agency has granted eligibility to submit the OCU400 Marketing Authorization Application (MAA) through the centralized procedure, based on the current study design and statistical analysis plan.

Regarding the Company’s inhaled vaccines portfolio, the National Institute of Allergy and Infectious Diseases (NIAID) intends to initiate the Phase 1 clinical trial for OCU500 in the third quarter of 2025.

In addition to the notable leadership appointments, Ocugen welcomed the National Security Commission on Emerging Biotechnology (NSCEB) and U.S. Rep. Chrissy Houlahan to its manufacturing facility as part of the NSCEB's Biotech Across America events, highlighting biotech innovation in Pennsylvania. Rep. Houlahan subsequently announced the bipartisan BIOTech Caucus to build greater awareness and understanding of biotechnology among lawmakers and support transformative advances in healthcare. Dr. Musunuri supports the formation of this very important bipartisan BIOTech Caucus that includes senior congressional leaders such as Rep. Pete Sessions in addition to local leaders, which will prioritize biotechnology at the national level to ensure U.S. leadership globally.

"The meaningful progress Ocugen is making across its novel modifier gene therapy platform, along with strategic leadership changes and significant external alliances are evidence of a strong first half of 2025," said Dr. Musunuri. "We look forward to providing critical program updates and data in the coming months."

Modifier Gene Therapy Platform—a Novel First-in-Class Platform

- **OCU400 for RP –** On track to complete enrollment in support of BLA/MAA filings in 2026. Data and Safety Monitoring Board (DSMB) convened and found no SAEs related to OCU400 and recommended to continue study dosing as planned.
- **OCU410ST for Stargardt Disease –** FDA granted RPDD for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, retinitis pigmentosa 19, and cone-rod dystrophy 3. FDA cleared the Investigational New Drug (IND) amendment to initiate a Phase 2/3 pivotal confirmatory trial of OCU410ST and dosing has been initiated.
- **OCU410 for GA –** Phase 1 data at 12 months demonstrates reduced lesion growth, preservation of retinal tissue, and—most importantly—a positive effect on the functional visual measure of low luminance visual acuity (LLVA). Interim Phase 2 data at 6 months demonstrated very encouraging results consistent with Phase 1 data.

Ophthalmic Biologic Product

- **OCU200** – DSMB approved continuation of dosing in the third cohort and the Company intends to complete the Phase 1 clinical trial in the second half of 2025.

Second Quarter 2025 Financial Results

- The Company's cash, cash equivalents, and restricted cash totaled $27.3 million as of June 30, 2025, compared to $58.8 million as of December 31, 2024, providing cash runway into the first quarter of 2026. The Company had 292.2 million shares of common stock outstanding as of June 30, 2025.
- Total operating expenses for the three months ended June 30, 2025 were $15.2 million and included research and development expenses of $8.4 million and general and administrative expenses of $6.8 million. This compares to total operating expenses for the three months ended June 30, 2024 of $16.6 million that included research and development expenses of $8.9 million and general and administrative expenses of $7.7 million.
- Ocugen reported a $0.05 net loss per common share for the three months ended June 30, 2025, compared to a $0.06 net loss per common share for the three months ended June 30, 2024.

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 executive leadership 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: 9627149
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 pioneering biotechnology leader in gene therapies for blindness diseases. Our breakthrough modifier gene therapy platform has the potential to address significant unmet medical need for large patient populations through our gene-agnostic approach. Unlike traditional gene therapies and gene editing, Ocugen's modifier gene therapies address the entire disease—complex diseases that are potentially caused by imbalances in multiple gene networks. Currently we have programs in development for inherited retinal diseases and blindness diseases affecting millions across the globe, including retinitis pigmentosa, Stargardt disease, and geographic atrophy—late stage dry age-related macular degeneration. 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 first quarter of 2026, statements regarding qualitative assessments of available data, potential benefits, expectations for ongoing

clinical trials, anticipated regulatory filings and anticipated development timelines, and Ocugen's negotiations regarding the license agreement with a Korean partner and Ocugen's potential merger transaction regarding the OrthoCellix business, 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; that a definitive agreement for the license with a Korean partner will be delayed or not executed at all, or that, if executed, it may not be on terms anticipated; that the OrthoCellix merger transaction may not close or, if closed, may not result in the benefits anticipated. 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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CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share amounts)
(Unaudited)

	June 30, 2025	December 31, 2024
Assets		
Current assets		
Cash	$ 27,013	$ 58,514
Prepaid expenses and other current assets	5,870	3,168
Total current assets	32,883	61,682
Property and equipment, net	15,445	16,554
Restricted cash	312	307
Other assets	4,954	3,899
Total assets	$ 53,594	$ 82,442
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 4,237	$ 4,243
Accrued expenses and other current liabilities	12,899	15,500
Operating lease obligations	853	519
Current portion of long term debt	—	1,326
Total current liabilities	17,989	21,588
Non-current liabilities		
Operating lease obligations, less current portion	3,945	3,313
Long term debt, net	28,025	27,345
Other non-current liabilities	583	564
Total non-current liabilities	32,553	31,222
Total liabilities	50,542	52,810
Stockholders' equity		
Common Stock	2,924	2,915
Treasury stock	(48)	(48)
Additional paid-in capital	370,474	366,938
Accumulated other comprehensive income	12	48
Accumulated deficit	(370,31)	(340,221)
Total stockholders' equity	3,052	29,632
Total liabilities and stockholders' equity	$ 53,594	$ 82,442

OCUGEN, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)

(Unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2025	**2024**	**2025**	**2024**
Collaborative arrangement revenue	$ 1,373	$ 1,141	$ 2,854	$ 2,155
Total revenue	1,373	1,141	2,854	2,155
Operating expenses				
Research and development	8,402	8,902	17,932	15,728
General and administrative	6,766	7,688	13,218	14,092
Total operating expenses	15,168	16,590	31,150	29,820
Loss from operations	(13,795)	(15,449)	(28,296)	(27,665)
Interest (expense) income, net	(1,058)	173	(1,972)	475
Other (expense) income, net	114	(4)	179	(14)
Net loss	$ (14,739)	$ (15,280)	$ (30,089)	$ (27,204)
Other comprehensive income (loss)				
Foreign currency translation adjustment	(28)	3	(36)	8
Comprehensive loss	$ (14,767)	$ (15,277)	$ (30,125)	$ (27,196)
Net loss attributable to common shareholders — basic and diluted	(14,739)	(15,259)	(30,089)	(27,157)
Weighted shares used in calculating net loss per common share — basic and diluted	292,067,192	257,353,857	292,032,072	257,293,247
Net loss per share attributable to common shareholders — basic and diluted	$ (0.05)	$ (0.06)	$ (0.10)	$ (0.11)
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	—	(21)	—	(47)
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	—	54,745	—	54,745
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	—	(0.38)	—	(0.86)