



Ocugen Provides Business Update with Fourth Quarter and Full Year 2024 Financial Results

March 5, 2025

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

- Reached an alignment with FDA on Phase 2/3 pivotal confirmatory clinical trial for OCU410ST for Stargardt disease potentially expediting clinical development by two to three years
- Completed dosing in OCU410 Phase 2 ArMaDa clinical trial (N=51) for geographic atrophy (GA). Data and Safety Monitoring Board (DSMB) recently reviewed interim safety data from Phase 2. All subjects from multiple dose levels in Phase 2 to date demonstrated a very favorable safety and tolerability profile.
- Positive 2-year long-term data across multiple mutations of retinitis pigmentosa (RP) from the Phase 1/2 clinical trial of OCU400 demonstrated a durable and statistically significant ($p=0.005$) improvement in visual function (LLVA) in all evaluable treated subjects at two years when compared to untreated eyes
- OCU400 remains on track to meet 1H 2025 recruitment completion and potential BLA/MAA filings by mid-2026

MALVERN, Pa., March 05, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today reported fourth quarter and full year 2024 financial results along with a general business update.

"Ongoing data from all three clinical programs, along with patient testimonials, continue to support the benefit of one-time treatment from our revolutionary modifier gene therapy platform—as we are seeing consistent stabilization or improvement in treated eyes while maintaining a favorable safety and tolerability profile," said Dr. Shankar Musunuri, Chairman, CEO and Co-founder of Ocugen. "Throughout 2024, the Company continually advanced its clinical programs in line with enrollment and dosing timelines, and we will continue to drive the product pipeline forward in 2025."

Recently, the Company announced it will proceed with a Phase 2/3 pivotal confirmatory clinical trial of OCU410ST for Stargardt disease after alignment with the FDA. This accelerated regulatory pathway for OCU410ST is driven by the incredible unmet medical need that exists for 100,000 Stargardt patients in the U.S. and Europe who have no treatment option available. Accelerating the clinical timeline of OCU410ST is expected to save significant costs in addressing disease burden even sooner than anticipated.

The OCU410ST Phase 2/3 clinical trial will randomize 51 subjects, 34 of whom will receive a single, subretinal, 200- μ L injection of OCU410ST at a concentration of 1.5×10^{11} vector genomes (vg)/mL in the eye with worse visual acuity, and 17 of whom will serve as untreated controls. One-year data will be utilized for the BLA filing planned for 2027. 6-month data from Phase 1 of the OCU410ST GARDian clinical trial demonstrated considerably slower lesion growth (52%) from baseline in treated eyes versus untreated fellow eyes and clinically meaningful 2-line (10-letter) improvement in visual function (BCVA), which is statistically significant ($p=0.02$) in treated eyes.

This week, the DSMB for the OCU410 ArMaDa clinical trial for GA convened and reviewed the safety and tolerability profile of an additional 15 subjects from the Phase 2 portion of the study. No serious adverse events related to OCU410 have been reported to date in all 60 subjects, including Phase 1. Unlike currently available treatments for GA, there were no cases of ischemic optic neuropathy, vasculitis, intraocular inflammation, endophthalmitis or choroidal neovascularization, and no adverse events of special interest.

Preliminary 9-month efficacy data of OCU410 in GA patients showed considerably slower lesion growth (44%) from baseline and clinically meaningful 2-line (10-letter) improvement in visual function (LLVA) in treated eyes compared to untreated eyes in the Phase 1 portion of the trial. Furthermore, a single subretinal OCU410 treatment preserves more retinal tissue around GA lesions of treated eyes at 9 months compared to published data on currently available GA therapies. GA, an advanced form of dry age-related macular degeneration, affects 2-3 million people in the U.S. and Europe combined.

Two-year data from the Phase 1/2 OCU400 clinical trial for RP demonstrated a durable and statistically significant ($p=0.005$) improvement in visual function (LLVA) in all evaluable treated subjects when compared to untreated eyes. 100% (10/10) of treated evaluable subjects showed improvement or preservation in visual function compared to untreated eyes, and OCU400 was observed to have a favorable safety and tolerability profile in all subjects across multiple mutations.

In parallel with clinical milestones, considerable regulatory accomplishments were achieved, including orphan medicinal product designation (OMPD) from the European Medicines Agency (EMA) for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, retinitis pigmentosa 19 (RP19), and cone-rod dystrophy 3 (CORD), and positive opinions from the EMA's Committee for Advanced Therapies (CAT) for OCU400, OCU410, and OCU410ST Advanced Therapy Medicinal Product (ATMP) classification.

Ocugen's biologic candidate moved into the clinic and patients are currently being dosed in the OCU200 Phase 1 clinical trial for diabetic macular edema (DME). OCU200 has the potential to change the treatment landscape for DME, diabetic retinopathy, and wet age-related macular degeneration with its unique mechanism of action, binding the active component—tumstatin—to integrin receptors that play a crucial role in disease pathogenesis and holds the promise to benefit all DME patients, including the 30-40% of patients who do not respond to current anti-VEGF therapies.

"I am extremely proud of our team at Ocugen, who are working tirelessly to enroll patients across all four clinical programs in close collaboration with the study centers and investigators," said Dr. Huma Qamar, Chief Medical Officer at Ocugen. "It is very gratifying to think about the potential unmet medical need that can be addressed through modifier therapy for patients who currently have no options and are facing the prospect of losing their vision altogether."

Finally, the Investigational New Drug (IND) application for OCU500—the Company's inhaled mucosal vaccine for COVID-19 was cleared by the FDA. The National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health, is expected to sponsor and conduct the Phase 1 trial to assess the safety, tolerability, and immunogenicity of OCU500 administered via two different routes, inhalation into the lungs and intranasally as a spray.

To extend the cash runway into the first quarter of 2026, and support Ocugen's clinical trials, the Company secured \$65 million in equity/debt financings in the second half of 2024. Further supporting Ocugen's efforts to enable long-term shareholder value, garner significant visibility within the investment community, and broaden the shareholder base, the Company was included in the Russell Index in May 2024.

"Meaningful progress in 2024 and recent success with FDA brought us closer to our goal of three potential BLAs in the years—2026, 2027, 2028, encompassing RP, Stargardt and GA treatments, respectively," said Dr. Musunuri. "While this is an ambitious goal, I am confident that we have the strategic and scientific expertise, and an unrelenting commitment to patients, to deliver on our commitment."

Business Updates

Novel Modifier Gene Therapy Platform—Potentially Targeting Three BLA Filings in the Next Three Years

- **OCU400** –The European Commission (EC) provided a positive opinion from the EMA's CAT for OCU400 ATMP classification. Actively enrolling patients in the U.S. and Canada in the Phase 3 liMeliGhT clinical trial for the treatment of RP and are on track to complete enrollment in the first half of 2025 and file BLA and MAA submissions in mid-2026.
- **OCU410ST** –The EMA granted OMPD for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, retinitis pigmentosa 19 (RP19), and cone-rod dystrophy 3 (CORD3). The FDA approved a Phase 2/3 pivotal confirmatory clinical trial for BLA filing. Plan to initiate the Phase 2/3 study mid-2025 and targeting BLA submission by 2027. The EC provided a positive opinion from the EMA's CAT for OCU410ST ATMP classification.
- **OCU410** – Completed dosing in Phase 2 of the OCU410 Phase 1/2 ArMaDa clinical trial ahead of schedule. Intend to complete 1-year follow up in the first quarter of 2026 and targeting a BLA filing in 2028. The EC provided a positive opinion from the EMA's CAT for OCU410 ATMP classification.

Financial Results

- Fourth quarter — Research and development expenses for the three months ended December 31, 2024, were \$8.3 million compared to \$7.8 million for the three months ended December 31, 2023. General and administrative expenses for the three months ended December 31, 2024, were \$6.3 million compared to \$5.2 million for the three months ended December 31, 2023. Ocugen reported a \$0.05 net loss per common share for the three months ended December 31, 2024, compared to a \$0.04 net loss per common share for the three months ended December 31, 2023.
- Full year — Research and development expenses for the year ended December 31, 2024, were \$32.1 million compared to \$39.6 million for the year ended December 31, 2023. General and administrative expenses for the year ended December 31, 2024, were \$26.7 million compared to \$32.0 million for the year ended December 31, 2023. Ocugen reported a \$0.20 net loss per common share for the year ended December 31, 2024, compared to a \$0.26 net loss per common share for the year ended December 31, 2023.
- Ocugen's cash and restricted cash, totaled \$58.8 million as of December 31, 2024, compared to \$39.5 million as of December 31, 2023. The Company estimates that its current cash, cash equivalents, and investments will enable it to fund its operations into the first quarter of 2026. The Company had 291.4 million shares of common stock outstanding as of December 31, 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 leadership 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: 5045393

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 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 and efficacy thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, expected cash runway into the first quarter of 2026, the ability to initiate new clinical programs, statements regarding qualitative assessments of available data, potential benefits, expectations for ongoing and future 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)

	As of December 31,	
	2024	2023
Assets		
Current assets		
Cash	\$ 58,514	\$ 39,462
Prepaid expenses and other current assets	3,168	3,509
Total current assets	61,682	42,971
Property and equipment, net	16,554	17,290
Restricted cash	307	—
Other assets	3,899	4,286
Total assets	\$ 82,442	\$ 64,547
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	\$ 4,243	\$ 3,172
Accrued expenses and other current liabilities	15,500	13,343
Operating lease obligations	519	574
Current portion of long term debt	1,326	—
Total current liabilities	21,588	17,089
Non-current liabilities		
Operating lease obligations, less current portion	3,313	3,567
Long term debt, net	27,345	2,800
Other non-current liabilities	564	527
Total non-current liabilities	31,222	6,894
Total liabilities	52,810	23,983
Stockholders' equity		
Convertible preferred stock	—	1
Common stock	2,915	2,567
Treasury stock	(48)	(48)
Additional paid-in capital	366,938	324,191
Accumulated other comprehensive income	48	20
Accumulated deficit	(340,221)	(286,167)
Total stockholders' equity	29,632	40,564

Total liabilities and stockholders' equity

\$ 82,442 \$ 64,547

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

	<u>Three months ended December 31,</u>		<u>Year ended December 31,</u>	
	<u>2024</u>	<u>2023</u>	<u>2024</u>	<u>2023</u>
Collaborative arrangement revenue	\$ 764	\$ 1,409	\$ 4,055	\$ 6,036
Total revenue	764	1,409	4,055	6,036
Operating expenses				
Research and development	8,290	7,779	32,126	39,573
General and administrative	6,314	5,155	26,686	31,994
Total operating expenses	14,604	12,934	58,812	71,567
Loss from operations	(13,840)	(11,525)	(54,757)	(65,531)
Other income (expense), net	(40)	555	703	2,453
Net loss	<u>\$ (13,880)</u>	<u>\$ (10,970)</u>	<u>\$ (54,054)</u>	<u>\$ (63,078)</u>
Net loss attributable to common shareholders— basic and diluted	(13,880)	(10,970)	(54,010)	(63,078)
Weighted shares used in calculating net loss per common share — basic and diluted	<u>290,924,531</u>	<u>256,506,387</u>	<u>270,995,121</u>	<u>244,327,057</u>
Net loss per share attributable to common shareholders — basic and diluted	<u>\$ (0.05)</u>	<u>\$ (0.04)</u>	<u>\$ (0.20)</u>	<u>\$ (0.26)</u>
Net loss attributable to Series B Convertible Preferred shareholders — basic and diluted	—		(44)	
Weighted shares used in calculating net loss per Series B Convertible Preferred Stock — basic and diluted	—	—	<u>54,745</u>	
Net loss per share attributable to Series B Convertible Preferred shareholders — basic and diluted	<u>\$ —</u>	<u>\$ —</u>	<u>\$ (0.80)</u>	<u>\$ —</u>