Ocugen

Novel cell therapies for retinal diseases

Pharma and biotech

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Ocugen's modifier gene therapy (MGT) platform is designed to regulate gene expression to restore visual function across both genetically diverse inherited retinal diseases (IRDs) and retinal conditions with wider prevalence such as geographic atrophy (GA) associated with dry age-related macular degeneration (AMD). Lead candidate OCU400 is in Phase III studies for retinitis pigmentosa (RP), with a potential FDA biologics license application (BLA) in 2026 and commercialisation in 2027.

NHR platform targets multiple genes in RP

RP, the most common IRD, affects c 300,000 patients in the US and Europe, and often causes blindness. A key limitation of single gene replacement therapies (like Luxturna, approved for an RPE65 mutation) is they only target one gene at a time, limiting their scope given the wide variety of genetic defects that contribute to a given IRD (more than 60 are associated with RP). The MGT platform is designed to be 'gene-agnostic', by involving the targeted delivery of nuclear hormone receptors (NHRs) to modify the expression of multiple genes and gene networks.

Lead RP candidate OCU400 in Phase III studies

OCU400 delivers a functional copy of the retina-specific NHR gene, *NR2E3*, via an AAV5 vector. *NR2E3* regulates several retinal functional pathways, and OCU400 aims to restore retinal cell function in RP patients with diverse mutations. Ocugen is recruiting patients for its Phase III liMeliGhT trial (targeted n=150), with enrollment completion expected in H225. This follows positive two-year data from its Phase I/II RP trial, showing durable, statistically significant low-light visual acuity gains.

Pipeline includes programs in GA and SD

OCU410, a one-time therapy for GA secondary to dry-AMD, is designed to target multiple disease progression pathways. Interim results from an ongoing Phase II trial (n=31) showed 27% slower lesion growth at six months. In July 2025, Ocugen started the Phase II/III GARDian3 trial (planned n=51) for OCU410ST, targeting all Stargardt disease (SD) variants (ABCA4-associated retinopathies). This study builds on positive Phase I data, where OCU410ST showed 48% slower lesion growth and visual acuity gains at 12 months compared to untreated eyes.

Additional capital likely needed by H226

As of 30 June 2025, Ocugen held $27.0m in gross cash and announced a $20m equity issue in August. Its H125 operating cash burn was $30.1m, driven by $17.9m in R&D and $13.2m in G&A costs. Ocugen recently spun out its cell therapy unit, enabling greater focus on retinal programs.

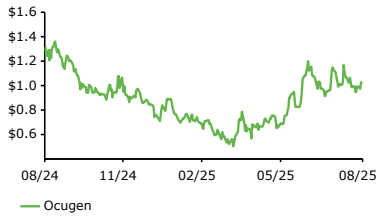
Consensus estimates

Year end	Revenue ($m)	PBT ($m)	EPS ($)	DPS ($)	EV/sales (x)	P/E (x)	Yield (%)
12/24	4.1	(54.1)	(0.20)	0.00	70.9	N/A	N/A
12/25e	2.5	(63.9)	(0.25)	0.00	115.1	N/A	N/A

Source: LSEG Data & Analytics. Note: EPS figures are as reported (basic).

Price	$1.03
Market cap	$287m

Share price performance



Share details

Code	OCGN
Listing	NASDAQ
Shares in issue (excl. 20m shares from August 2025 equity issue)	292.3m
Net cash/(debt) at 30 June 2025	$(1.0)m

Business description

Ocugen is a clinical-stage biotechnology company developing novel gene therapies to treat blinding retinal diseases. Its subsidiary is advancing cell therapies for orthopaedic indications.

Bull points

- Deep pipeline targeting IRDs and more prevalent retinal diseases (GA and diabetic macular edema).
- OCU400 has received orphan drug designations from the FDA and the EMA.
- Binding term sheet signed in Q225 for Korean rights to OCU400 (RP), which would include a 25% royalty rate and c $150m in sales milestones.

Bear points

- Continued need for funding for clinical programs.
- Binary clinical development and regulatory risks, which can be amplified in AAV vector-delivered gene therapies, given higher immunogenicity risks.
- Complex manufacturing requirements associated with AAV vector-derived genetic therapies.

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