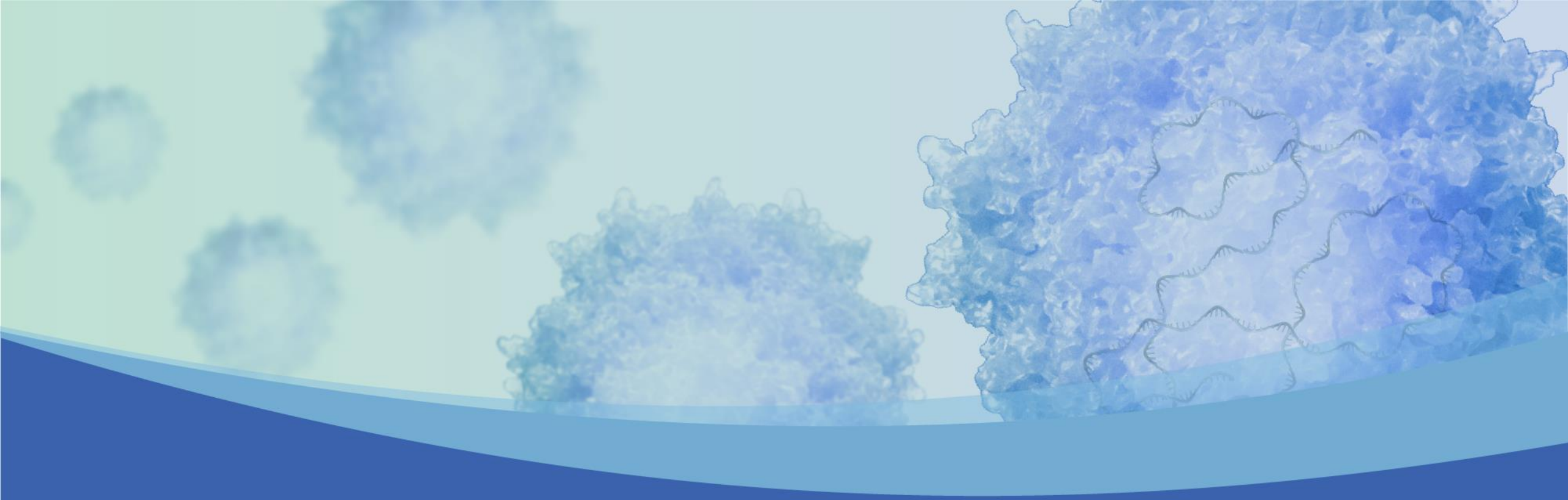


ocugen™

Courageous Innovation

# Dedicated to Bringing Game-Changing Gene Therapies to Market and Working Even Harder to Provide Access to Patients Globally

April 2026

# Forward Looking Statement

This presentation 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 potential safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, the ability to initiate new clinical programs, 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 presentation speak only as of the date of this presentation.

Except as required by law, we assume no obligation to update forward-looking statements contained in this presentation whether as a result of new information, future events, or otherwise, after the date of this presentation.

# Leader in Ophthalmology Gene Therapy

Pioneering biotechnology company leading the way to address major blindness diseases with novel modifier gene therapy

# Targeting Three Biologics License Applications (BLAs) in Three Years

| Program | Phase I | Phase II | Phase III | Target BLA/ MAA* Submission |
|---|---|---|---|---|
| **OCU400** Retinitis Pigmentosa 300,000 (U.S. + EU) | Gene-agnostic targeting >100 genes, broad indication<br>**Designations:** RMAT[1], ODD[2], OMPD[3] & ATMP[4] | Phase 3 Enrollment Completed<br>Phase 3 in Progress (Largest Orphan Gene Therapy Clinical Trial) | | 2026<br>Rolling Submission |
| **OCU410ST** Stargardt Disease 100,000 (U.S. + EU) | ABCA4-associated retinopathies caused by 1,200+ mutations<br>**Designation:** ODD, RPDD[5] & OMPD | Phase 2/3 Enrollment Completed<br>Phase 2/3 Pivotal Confirmatory Clinical Trial in Progress | | 2027 |
| **OCU410** Geographic Atrophy 2-3 million (U.S. + EU) | Advanced dry age-related macular degeneration (dAMD)<br>**Designation:** ATMP | Plan to Initiate Phase 3 in 2026 | | 2028 |

* Market Authorization Application will follow BLA submission

[1] Regenerative Medicine Advanced Therapy (RMAT); [2] Orphan Drug Designation (ODD); [3] Orphan Medicinal Product Designation (OMPD); [4] Advance Therapy Medicinal Products (ATMP); [5] Rare Paediatric Disease Designation (RPDD);

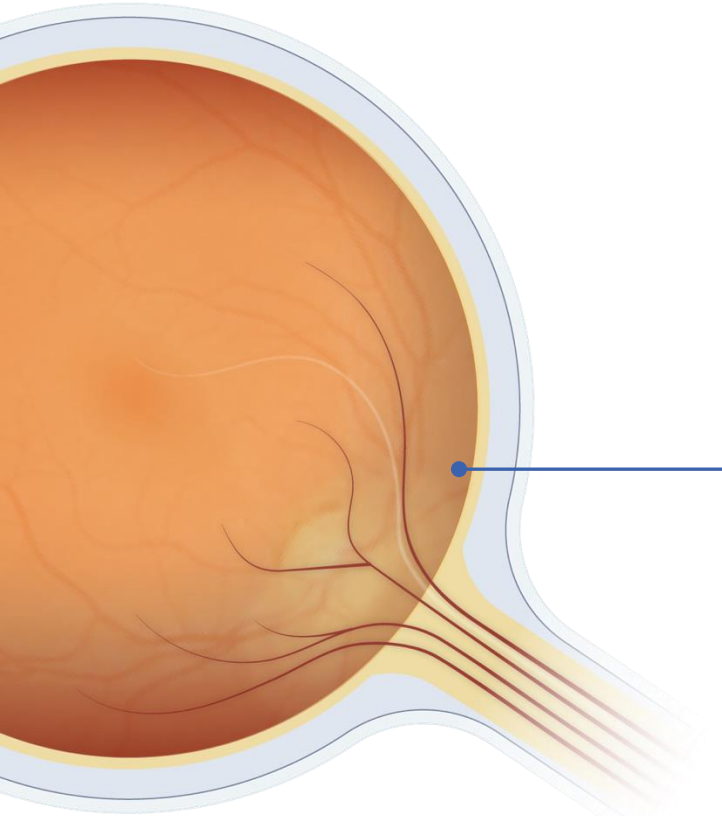
# Modifier Gene Therapy Platform

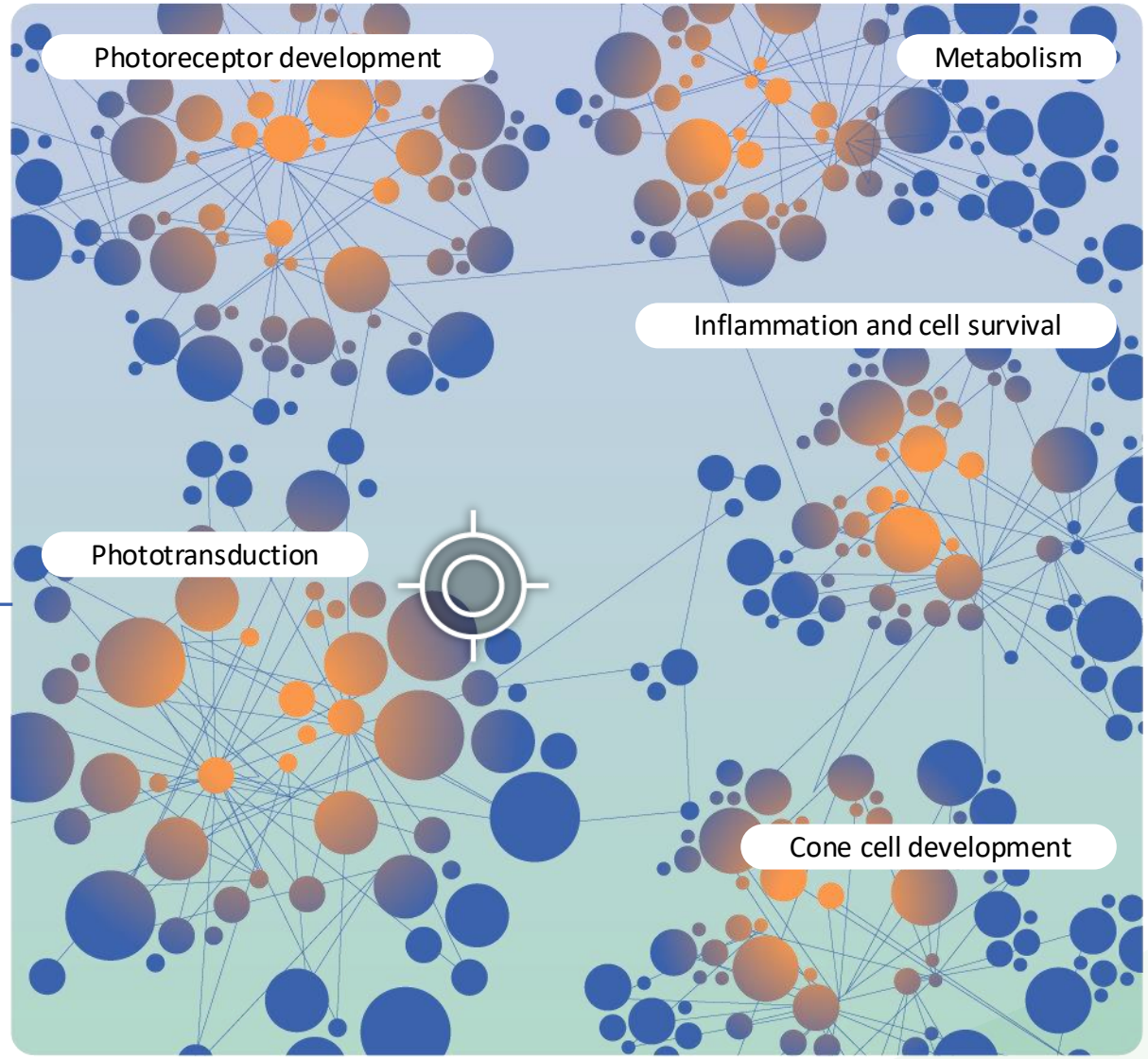
Breakthrough technology designed to address many rare diseases as well as complex diseases that affect millions

Problem

# Traditional therapy has limited therapeutic potential

Traditional therapy can only target one single gene at a time, limiting therapeutic potential.






**65%**

of all human proteins are expressed in the retina

**785**

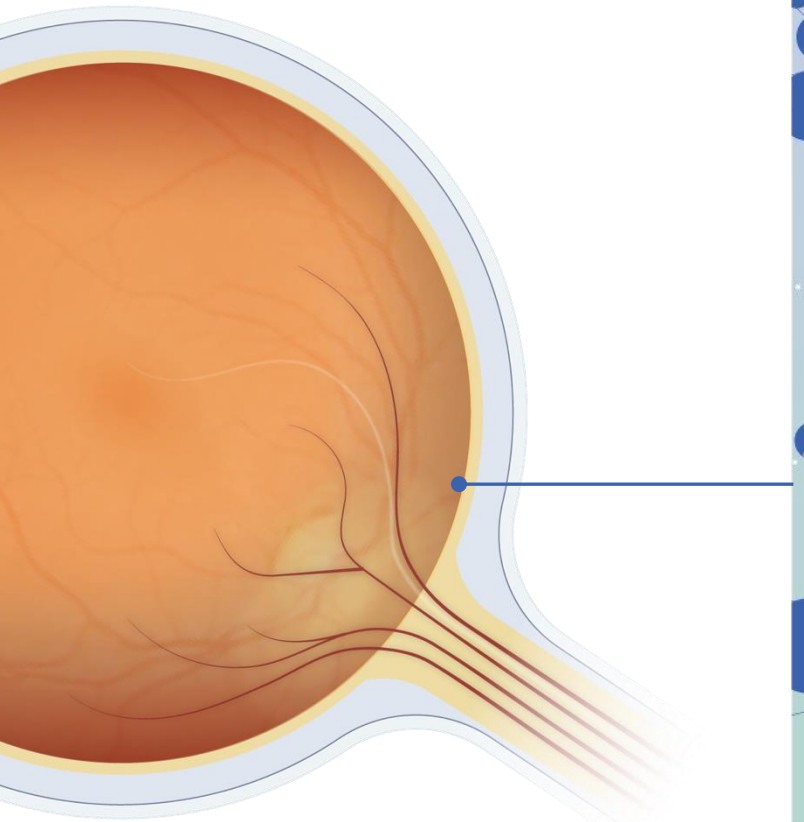
genes are highly specialized to it, interacting through complex pathways

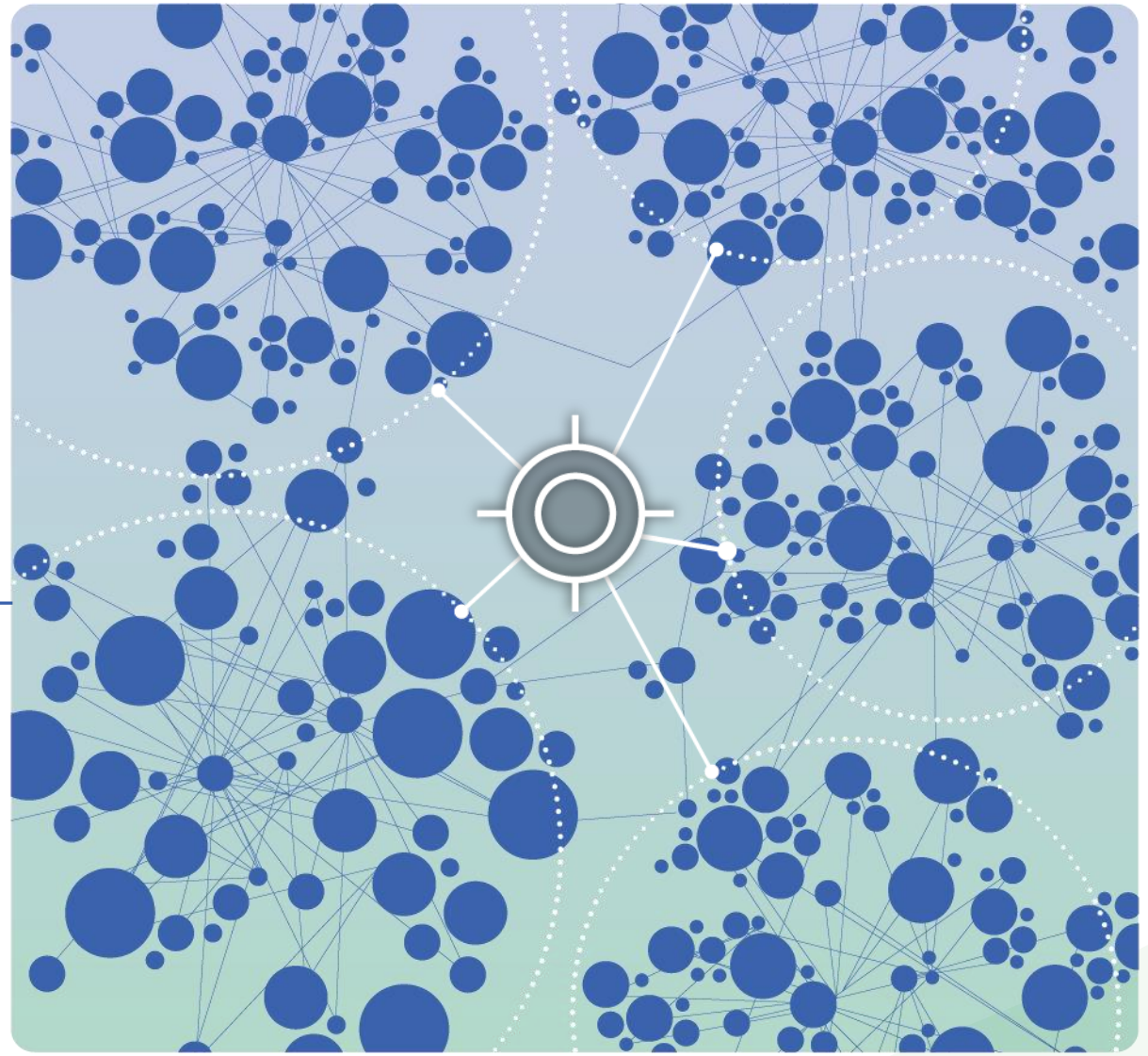
**250+**

mutations affecting the retina have already been identified

# The Solution is a Gene-Agnostic Approach

Our breakthrough technology is designed to address rare diseases and complex diseases





## Targeting master regulators

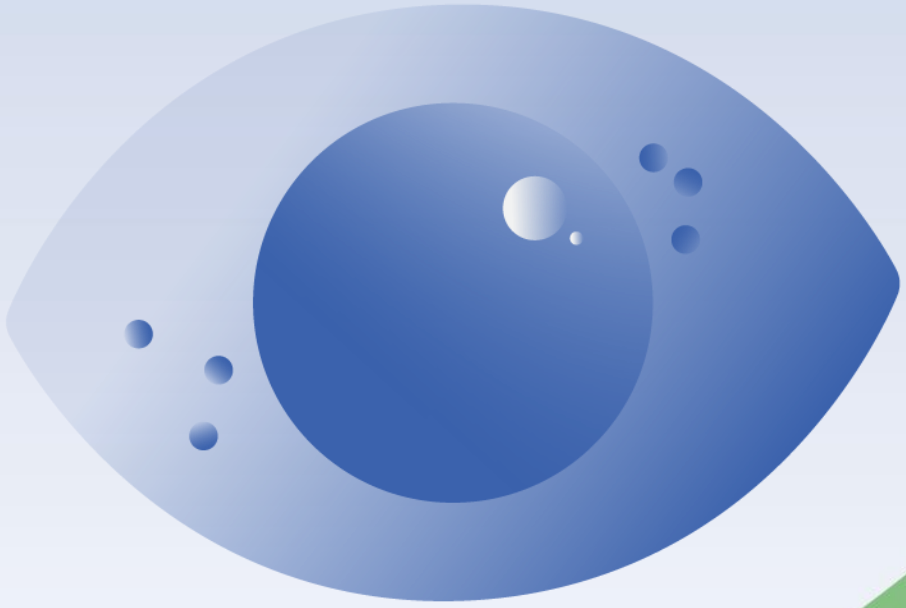
Master regulators control entire gene networks. By targeting them, Ocugen's gene therapy platform addresses the root cause of IRDs and multifactorial diseases (e.g. dAMD).

- Gene-Agnostic
- Multifactorial
- Durable Effect
- Broad Impact

# OCU400

## Retinitis Pigmentosa (RP)

Broad indication, gene-agnostic, targets 100+ genes



# First-in-Class Gene Therapy for Retinitis Pigmentosa

## Retinitis Pigmentosa

Retinitis Pigmentosa (RP) is a group of rare, inherited retinal diseases caused by mutations in over 100 genes, leading to progressive vision loss and, in many cases, blindness.

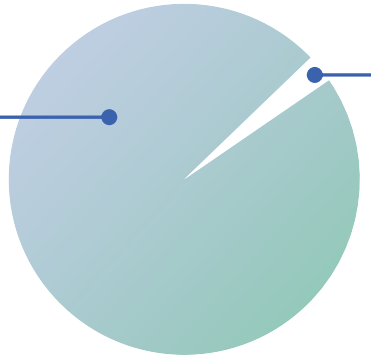
**1.6 million**
globally suffer from RP

**1**
approved treatment available

## Market Potential

U.S. + EU

**298,000**
Patients going untreated

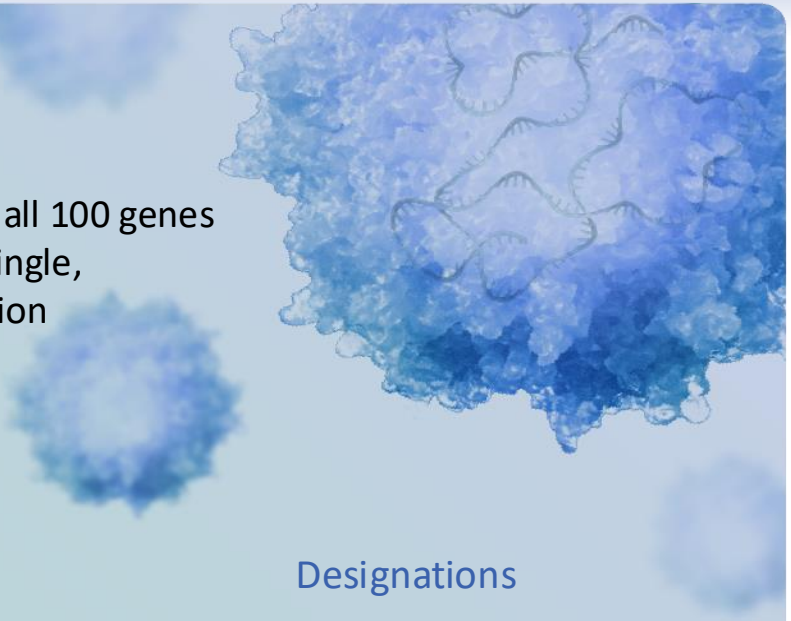


**2,000**
Luxturna® only addresses one gene (RPE65)

$52M peak annual sales

## OCU400

One product for all 100 genes delivered via a single, subretinal injection



### Regulatory Milestones

(Anticipated)

**2026**
Phase 3 trial underway — largest orphan gene therapy trial for RP

- Enrollment Completed
- Manufacturing process validation

Rolling Submission U.S. (BLA)
Followed by EU (MAA)

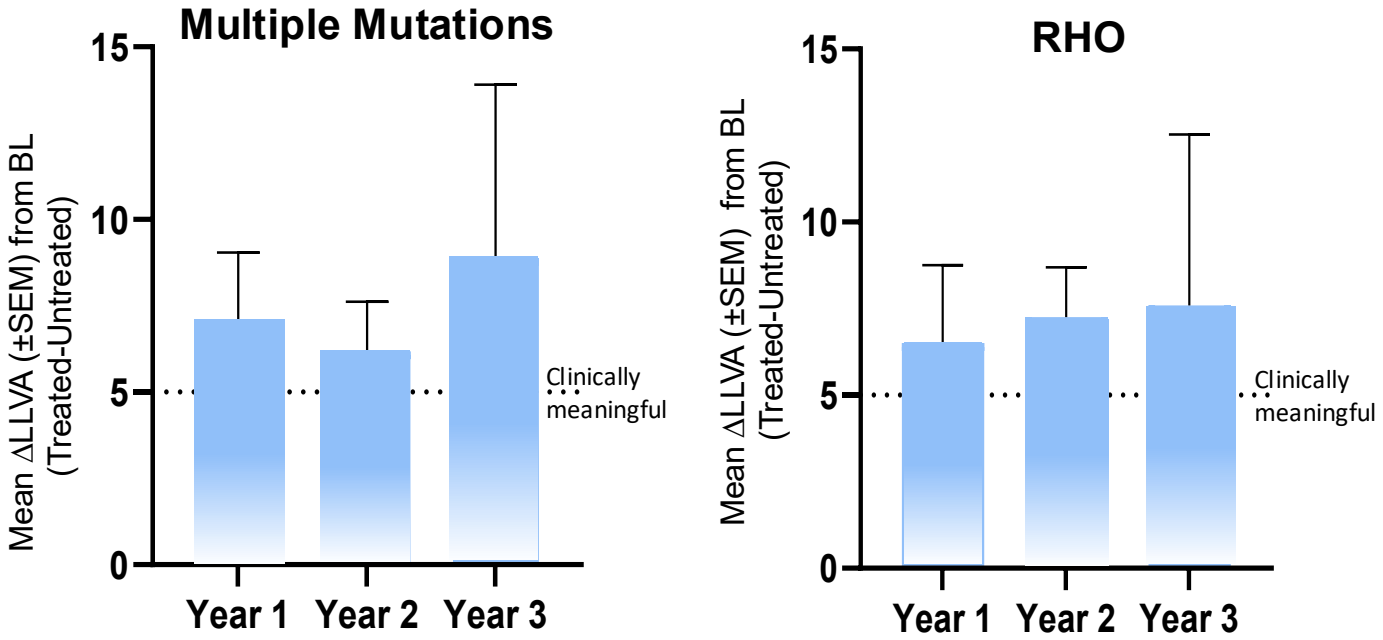
### Designations

- FDA (RMAT + ODD)
- EMA (ATMP+ OMPD)

Regenerative Medicine Advanced Therapy (RMAT); Orphan Drug Designation (ODD); Orphan Medicinal Product Designation (OMPD); EAP= Expanded Access Program

# Long-Term Durability, Safety and Tolerability Data at 3 Years

## Mean Change in LLVA (ETDRS Letters) from Baseline




Results from Phase 1/2 Study

Improvement in visual function in treated eyes when compared to untreated eyes, demonstrates gene-agnostic Mechanism of Action

**0**

Severe Adverse Events Reported related to OCU400

**88 %**

treated evaluable subjects demonstrated improvement or preservation in visual function compared to untreated eyes at 3 Years

OCU400 demonstrated a durable improvement in visual function (LLVA) in all evaluable treated subjects at 3 Yr when compared to untreated eyes

Evaluable, consented subjects for multiple mutations at Year 1 (N=11), Year 2 (N=11), Year 3 (N=8)
LogMar equivalent of ETDRS letters are represented for Year 3

Improvement or Preservation in evaluable Treated Eyes
Preservation = -/+4 letters from Baseline, Improvement: ≥5 Letters from Baseline

# Phase 3 liMeliGhT Trial—Largest RP Data Set

## Phase 3 Study Design

**MTD**
Determined in Phase 1/2

140 RP patients
2:1 ratio

2 **Treatment Group**
$2.5\times10^{10}$ vg per eye 250 µL

1 **Control Group**
No Treatment

## Endpoints

### Target Population

Early- to late-stage disease in broad RP population including pediatrics (3+ years)

**Primary**

12-month change in function vision assessed by LDNA*

Improvement in Lux Level in LDNA from baseline to 12 months

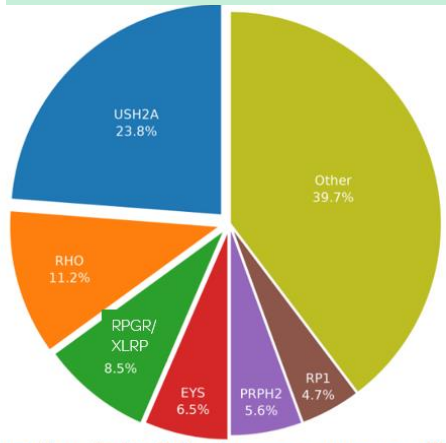
**Secondary**

Visual function improvement in treated eye vs. control eye

Assessed by **Low Luminance Visual Acuity (LLVA)**

Exploratory:
Patient Global Impression of Change (PGIC)

### Top Genes Associated with RP




Patients with mutations in the following genes were enrolled in OCU400 Trial

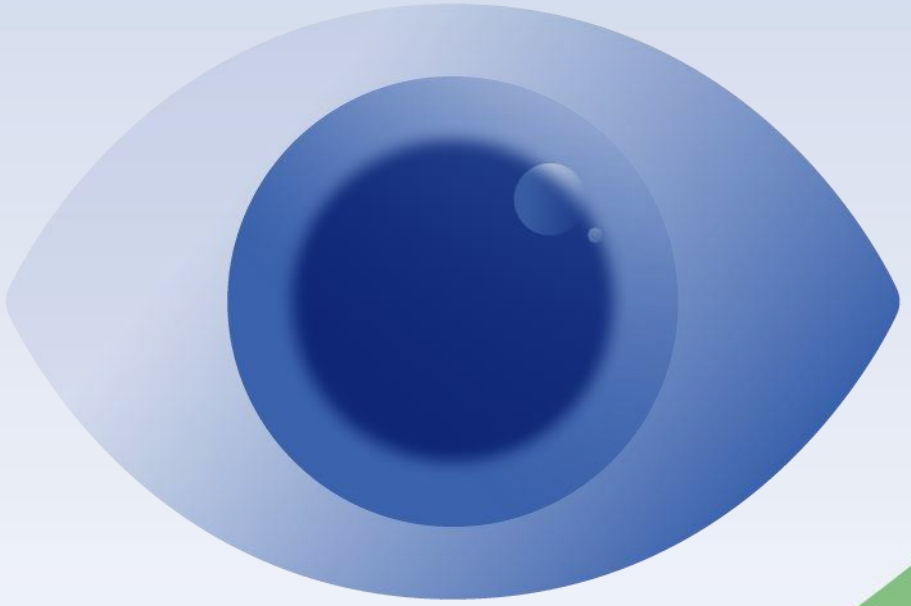
| | | | | | |
|---|---|---|---|---|---|
| RHO | XLRP (RPGR) | USH2A | EYS | RP1 | PRPF31 |
| PDE6A | NR2E3 | ABCA4 | RP2 (XLRP) | FAM161A | ADGRV1 |
| PDE6B | SAG | TULP1 | TRNT1 | CWC27 | IMPG2 |
| PRPF3 | BBS2 | GNB3 | CRB1 | GPR179 | PROM1 |
| IMPDH1 | RBP3 | ESPN | MKS1 | RP1L1 | SNRNP200 |
| PRPH2 | NPHP3 | CERKL | C2ORF71 | ARL2BP | INPP5E |

*LDNA= Luminance Dependent Navigation Assessment is a mobility test administered on a maze under different lux levels

# OCU410ST

## Stargardt Disease

ABCA4 -associated retinopathies >1,200 mutations



OCU410ST

# First-in-Class Gene Therapy for Stargardt Disease

## Stargardt Disease

A rare IRD associated with 1,200+ mutations of the *ABCA4* gene

**1 million**
globally suffer from *ABCA4*-associated retinopathies

**0**
approved treatments available

## Market Potential

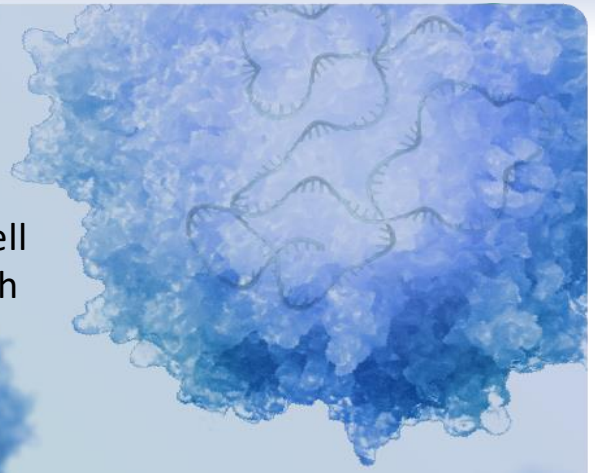
U.S. + EU

**100,000**
Potential Patients

**100%**
of Patients going untreated

## OCU410ST

for upregulation of networks of key genes improving the cell environment and survival with a single, subretinal injection



### Regulatory Milestones

(Completed/anticipated)

**2025**
Initiated pivotal Phase 2/3

**2026**
- Enrollment Completed

Interim analysis

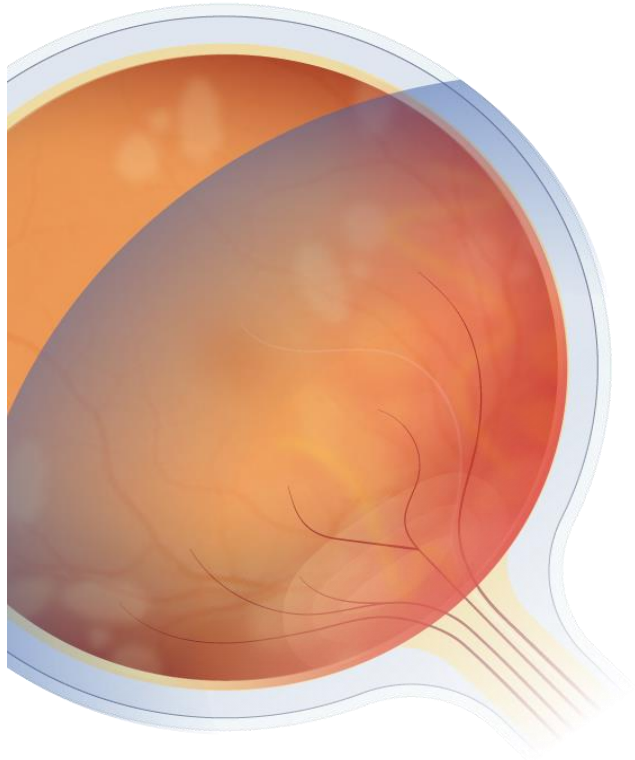
**2027**
Topline Data, BLA submission

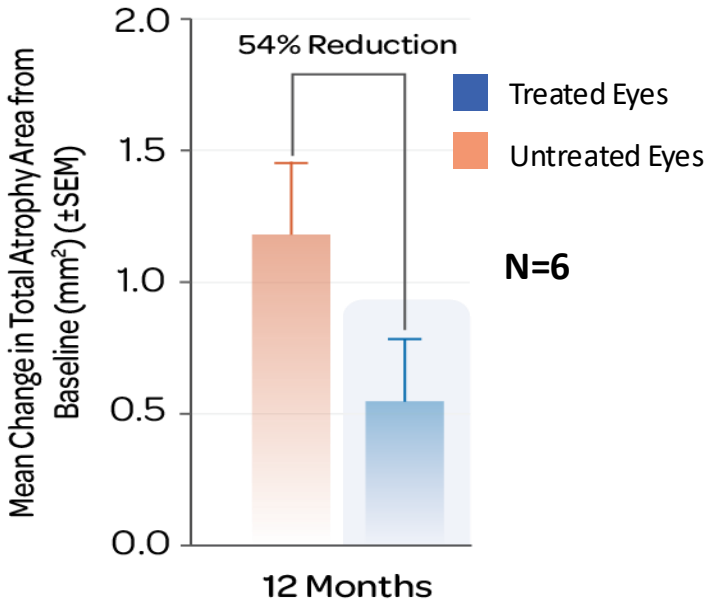
### Designations

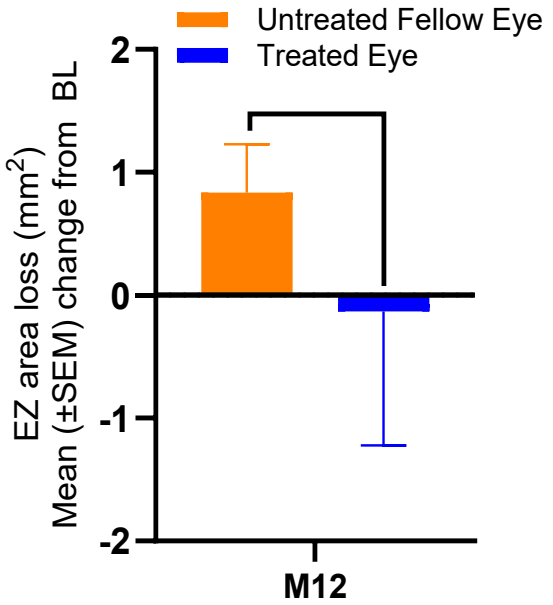
- FDA (RPDD + ODD)
- EMA (ATMP+ OMPD)

Advanced Therapeutic Medicinal Product (ATMP); Orphan Drug Designation (ODD); Orphan Medicinal Product Designation (OMPD)

# Phase 1 GARDian1 Trial Demonstrated Clinically Meaningful Benefit






**Lesion Size Reduction**

**54%**

treated vs. Control

**EZ Preservation**

**116%**

Treated vs. Control

Improvement or Preservation in evaluable Treated Eyes

Preservation = -/+4 letters from Baseline, Improvement: ≥5 Letters from Baseline

**No Serious Adverse Events Reported**

*Khanani *et al*., Nature Eye, January 10, 2026 (https://doi.org/10.1038/s41433-025-04202-5 )

# GARDian3- Phase 2/3 Pivotal Confirmatory Trial

## Trial Design

**MTD**
Established in Phase 1

Randomized 2:1 (N=51)

**17**
Control Group
No Treatment

**34**
Treatment Group
$3\times10^{10}$ vg per eye in 200 µL

**DSMB**
4-week Data Reviews

| First | Second | All |
|---|---|---|
| 17 | 17 | Subjects |

**DMC**
Interim-Data Analysis

## Endpoints

### Target Population

Early- to late-stage disease population
Including pediatrics (3+ years)

**Primary**

12-month change in atrophic lesion size from baseline vs. control

Measured in $mm^2$ by fundus autofluorescence (FAF)

**Secondary**

Functional improvement in vision vs. control eye

Assessed by LLVA and BCVA

EZ analysis (exploratory)

### Adaptive design with sample size re-estimation [3Q2026]

Interim Analysis: 24 subjects complete 8 months post-OCU410ST (16 treated and 8 controls)

# OCU410

## Geographic Atrophy

Advanced dry age-related macular degeneration (dAMD)

# First-in-Class Gene Therapy for GA Patients

## Geographic Atrophy

Geographic Atrophy (GA) is an advanced form of dry AMD. GA causes irreversible degeneration of retina cells in the macula, leading to loss of central vision.

**~8 million**

globally suffer from advanced dAMD

**2**

approved treatments available that address only 1 of the 4 pathways involved in disease progression

### Market Size

U.S. + EU

**2-3M**

Patients

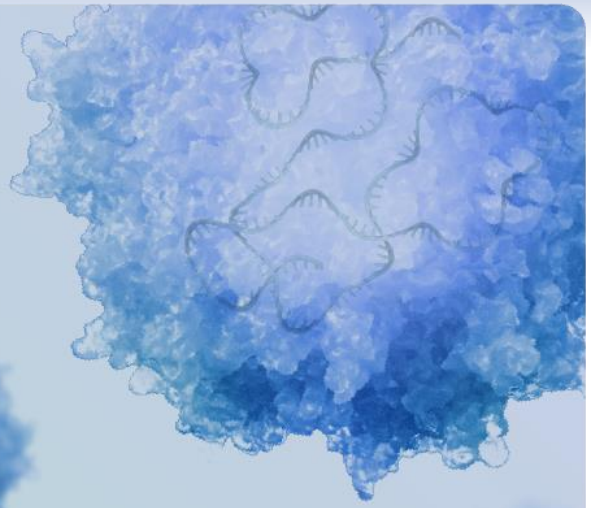
### Approved Products in US

SYFOVRE® and IZERVAY®

**>$1B combined annual sales**

## OCU410

Designed to address all four pathways associated with GA without 6-12 injections per year and related side effects



### Regulatory Milestones

(Anticipated)

- **2026** Phase 2 Topline data released
- **2026** Initiate Phase 3
- **2027** Complete enrollment
- **2028** Topline data, BLA submission

### Designations

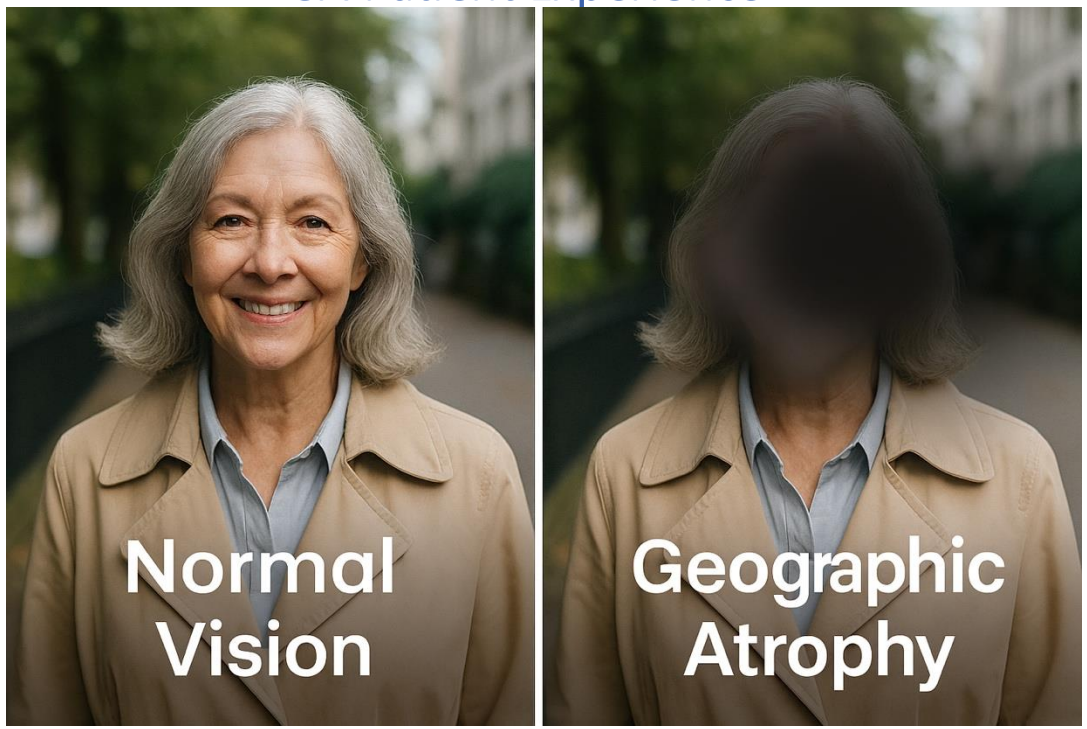
- EMA (ATMP)

### Recent Milestone

Positive 12-month Phase 2 data

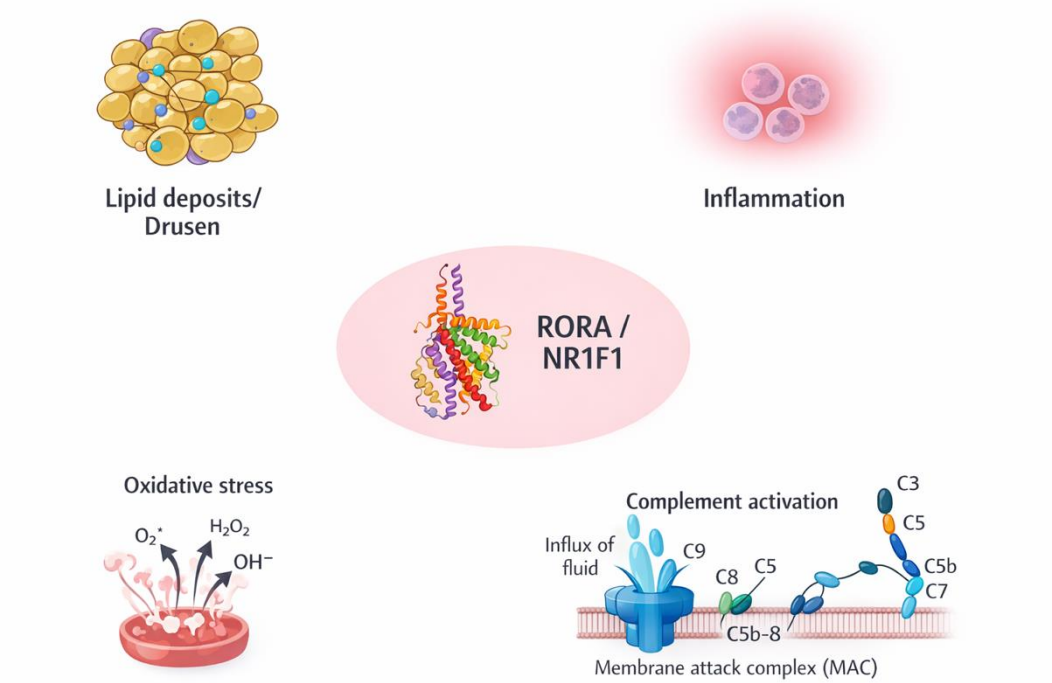
# OCU410 Aims to Disrupt GA Treatment Driven by a Novel MOA

## GA Patient Experience




Driving global change at the patient level
(2-3M patients in U.S. and EU )

## RORA 4-way MOA[1]




Addresses all disease pathways – marketed therapies only address the complement system

[1]Akula et al. *Gene Ther* 2024; MOA= Mechanism of Action: anti-drusen activity (improves retinal function), anti-inflammatory (suppresses inflammation in HMC3 cells), anti-oxidative (improves ARPE19 cell survival), anti-complement (increases Cd59 protein)

# Phase 2 ArMaDa Trial: To Assess Safety and Efficacy of OCU410 in GA

Target Population: **Geographic atrophy secondary to dry AMD**

## Randomization

1:1:1

| 17 | 17 | 17 |
|---|---|---|
| Medium Dose | High Dose | Control Group |
| 1x $10^{10}$ vg per eye, 200 µL | 3x $10^{10}$ vg per eye, 200 µL | No Treatment |

## Endpoints

Primary: Change in GA lesion size measured in $mm^2$ by FAF at Month 12

Exploratory: EZ preservation (correlates to visual function)

## Key Protocol Inclusion Criteria:

- Subjects 50 years and older
- BCVA of ≥21 ETDRS Letters
- Total GA area ≥2.0 and ≤ 20.5 mm2 (1 to 8 disk areas)
- GA within foveal and nonfoveal region
- CNV in fellow eye is not exclusionary
- Subjects who had a history of pegcetacoplan or avacincaptad pegol use were enrolled with 3M washout period

Phase 2 (ArMaDa Trial): NCT06018558

# OCU410 Demonstrates Favorable Safety and Tolerability Profile

| Adverse Events (AE), Serious AEs, Adverse Event of Special Interest (AESI) | Control (N=13) | OCU410 Med Dose (N=16) | OCU410 High Dose (N=16) |
|---|---|---|---|
| Endophthalmitis and Retinal Detachments | 0 | 0 | 0 |
| Retinal Vasculitis and/or Retinal Vascular Occlusion | 0 | 0 | 0 |
| Choroidal Neovascularization (CNV) | 1* | 2* | 1* |
| Intraocular Inflammation | 0 | 0 | 1# |
| Ischemic Optic Neuropathy | 0 | 0 | 0 |
| Treatment Emergent Serious Adverse Events | 0 | 0 | 0 |
| Treatment Emergent Adverse Events Considered Severe | 0 | 0 | 0 |

No OCU410-related SAEs and AESIs reported to date

# Intraocular Inflammation deemed related to study procedure – resolved
*CNV reported as AEs were not related to OCU410 based on DSMB review

# OCU410 Demonstrates Statistically Significant Reduction in Lesion Size at 12 Months

## OCU410 shows ~2X effect size compared to approved therapies

### Potentially addresses current unmet need in treating GA:

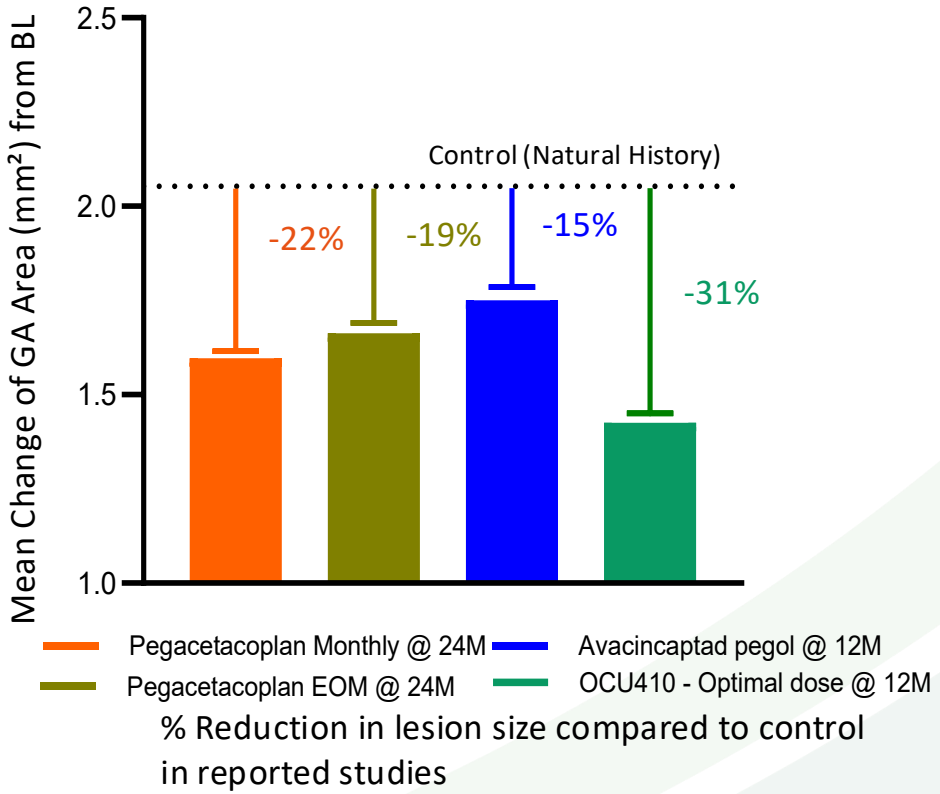
- Potential one-and-done treatment for life versus 6-12 injections per year
- May overcome up to 40% drop-out reported in the current standard of care

### Topline, 12-month efficacy results in Phase 1 and Phase 2:

- Promising efficacy in Phase 1 and Phase 2
- Apparent structural preservation on GA lesion
- EZ preservation may support visual function

### Topline data suggests favorable safety and tolerability profile:

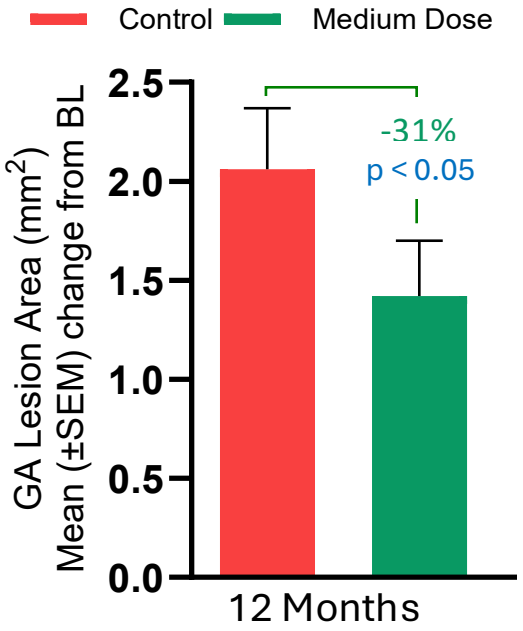
- No SAEs and AESIs deemed related to OCU410



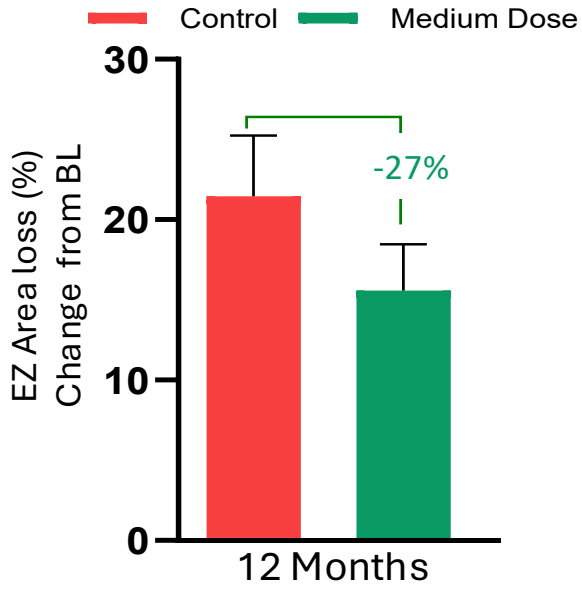

References: Apellis OAKS/DERBY (Heier et al, Lancet, Product Insert), Iveric GATHER 2 (Liao 2023, Khanani 2024, Lancet/Ophthalmology, Product Insert), Natural History Meta-analysis (Fleckenstein 2018, Ophthalmology). Change from Baseline for OCU410 was against ArMaDa control subjects; Dropout rates for approved therapies reported after 10 injections (PIPER|SANDLER Industry Note, June 2025);
OCU410 Optimal Dose = Medium Dose

# Phase 2 Data Driving Optimal Dose for Phase 3

*Change in GA Lesion Area (FAF; N=28)*




*Ellipsoid Zone Loss (SD-OCT; N=25)*
*(Correlates to Visual Function)*




- **No disease progression in~20% of treated subjects**
- **75% of treated subjects showed >30% reduction in lesion growth**

Lesion Size Reduction

31%

treated vs control

EZ Preservation

27%

treated vs control

References for Natural History: Mones and Biarnes, 2018, TVST, N=117;
For Primary Endpoint analysis evaluable subjects include controls (N=12) and medium dose (N=16); for EZ loss analysis, Controls (N=12) and medium dose (N=13)
GA Lesion ≥2.5 $mm^2$ and ≤17.5$mm^2$ (Lesion criteria in prior pivotal trials supporting approval); FAF= Fundus Autofluorescence; SD-OCT= Spectral Domain Optical Coherence Tomography;
Primary analysis conducted by MMRM and p-value <0.05
Phase 3 considerations: Dose - 1x$10^{10}$ vg per eye; Primary Endpoint – Lesion Size Reduction; Secondary Endpoint – EZ Preservation

# Phase 3 – Assess Efficacy, Safety and Tolerability of OCU410 in GA

## Proposed Trial Design

**MTD**
Established in Phase 1

**Optimal Dose**
established in Phase 2

Randomized 2:1 (N=300)

**100**
Control Group
No Treatment

**200**
Treatment Group
$1\times10^{10}$ vg per eye in 200 µL

**DMC**
Interim-Data Analysis

## Proposed Endpoints

### Target Population

Adults (55+years) with geographic atrophy secondary to dry AMD (55+ years), early to late-stage disease

**Primary**

12-month change in GA lesion size from baseline vs. control

Measured in square root mm by fundus autofluorescence (FAF)

**Secondary**

12-month change in EZ Loss Area in study eyes vs. control (SD-OCT)

Correlates to visual function

### Adaptive design with sample size re-estimation

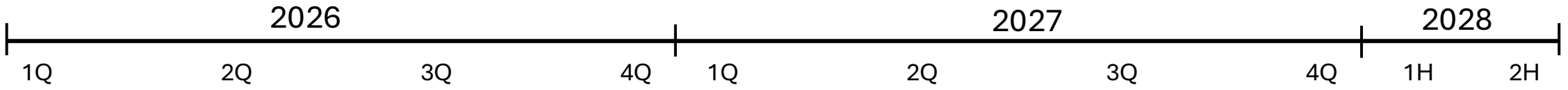
Interim Analysis: 150 subjects complete 12 months post-OCU410 administration (100 treated and 50 control)

# Ocugen Hopes to Deliver on Its Promise to Transform the Treatment Landscape for Patients with GA

## OCU410 potentially creates a new standard of care

- **First-in-class** RORA MOA designed to support central retina and photoreceptor integrity
- **Promising Phase 2 results** indicate 31% reduction in lesion size and 27% slower EZ loss
- **Potential to eliminate treatment burden** and patient fatigue to reduce treatment attrition
- **Optimized Phase 3 trial design** and targeted GA lesion size for vision preservation
- **Upcoming Global Phase 3**, n=~300, adaptive design, >95% power, 3Q 2026 (Target)

# Anticipated Milestones – Three BLAs by 2028




## OCU400
Retinitis Pigmentosa

- Initiate Rolling BLA Submission
- Phase 3 topline data
- Complete BLA Submission
- Approval/ Launch

## OCU410ST
Stargardt Disease

- 100% Enrollment Completion
- Interim Outcome Analysis[1]
- Phase 3 top line data
- BLA Submission
- Approval/ Launch

## OCU410
Geographic Atrophy

- Phase 2 Study Results
- Initiate Phase 3
- Phase 3 Enrollment completion
- BLA Submission

[1]Re-estimation to minimize clinical risk (Outcome - Impact / no-impact to BLA timeline)

# Advancing cures for blindness

IR@ocugen.com