



Courageous Innovation

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

February 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 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 time lines, 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



# We're Here to Make an Impact Through Courageous Innovation

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

## Values



Respect



Integrity



Teamwork



Accountability



GMP Facility



Headquarters, Malvern, Pennsylvania



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



\* Market Authorization Application will follow BLA submission

<sup>1</sup> Regenerative Medicine Advanced Therapy (RMAT); <sup>2</sup> Orphan Drug Designation (ODD); <sup>3</sup> Orphan Medicinal Product Designation (OMPD); <sup>4</sup> Advance Therapy Medicinal Products (ATMP); <sup>5</sup> 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

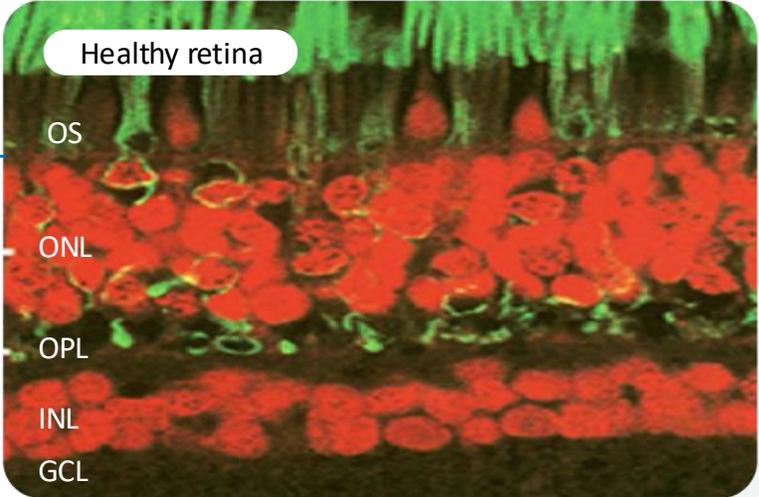
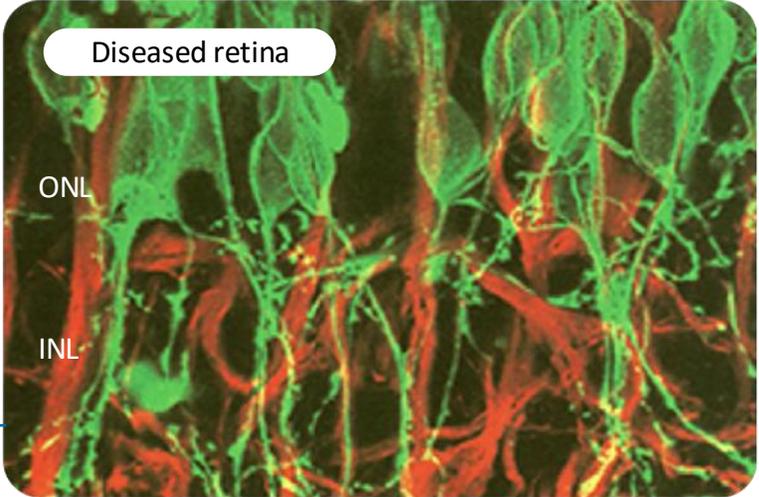
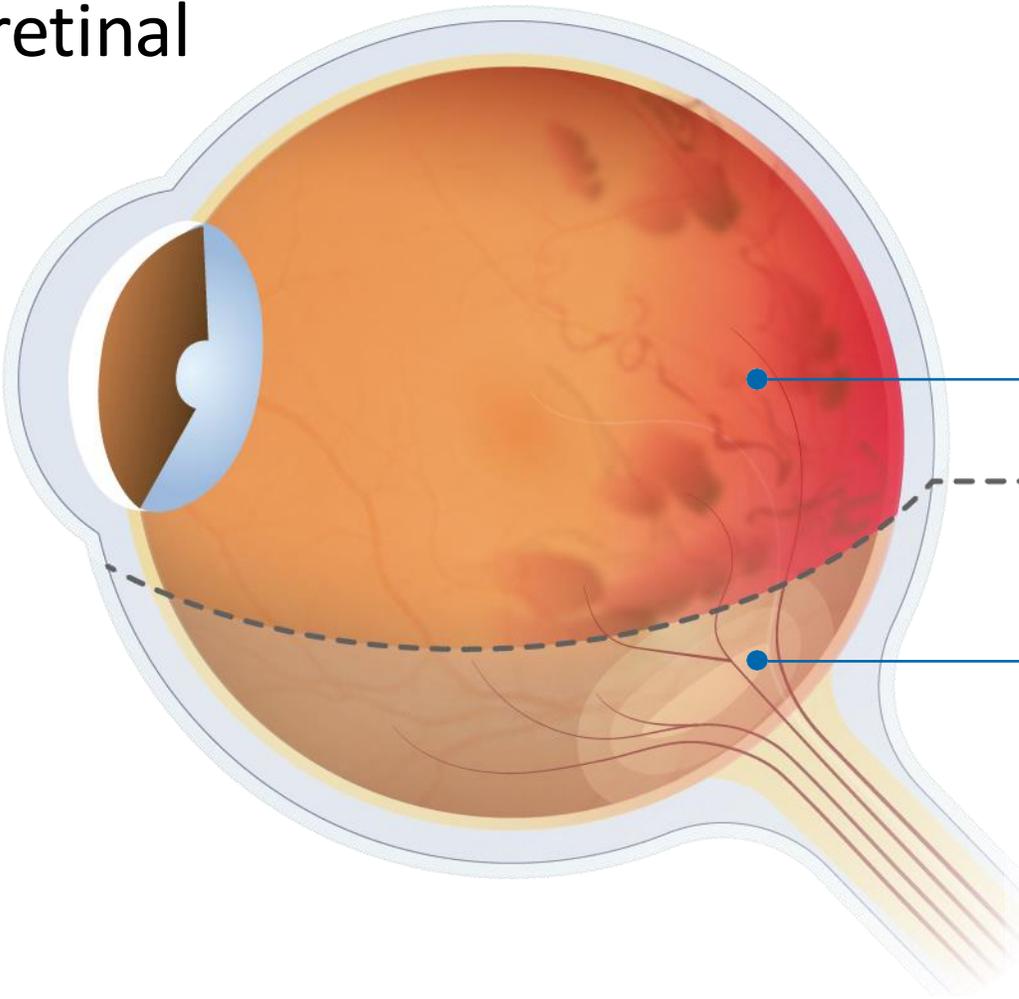
Problem

No therapy available for many retinal diseases, specially inherited retinal diseases such as RP, Stargardt and others

Most retinal therapies treat symptoms or target single genes, leaving millions with progressive vision loss and no effective, lasting treatment options.

3 million+

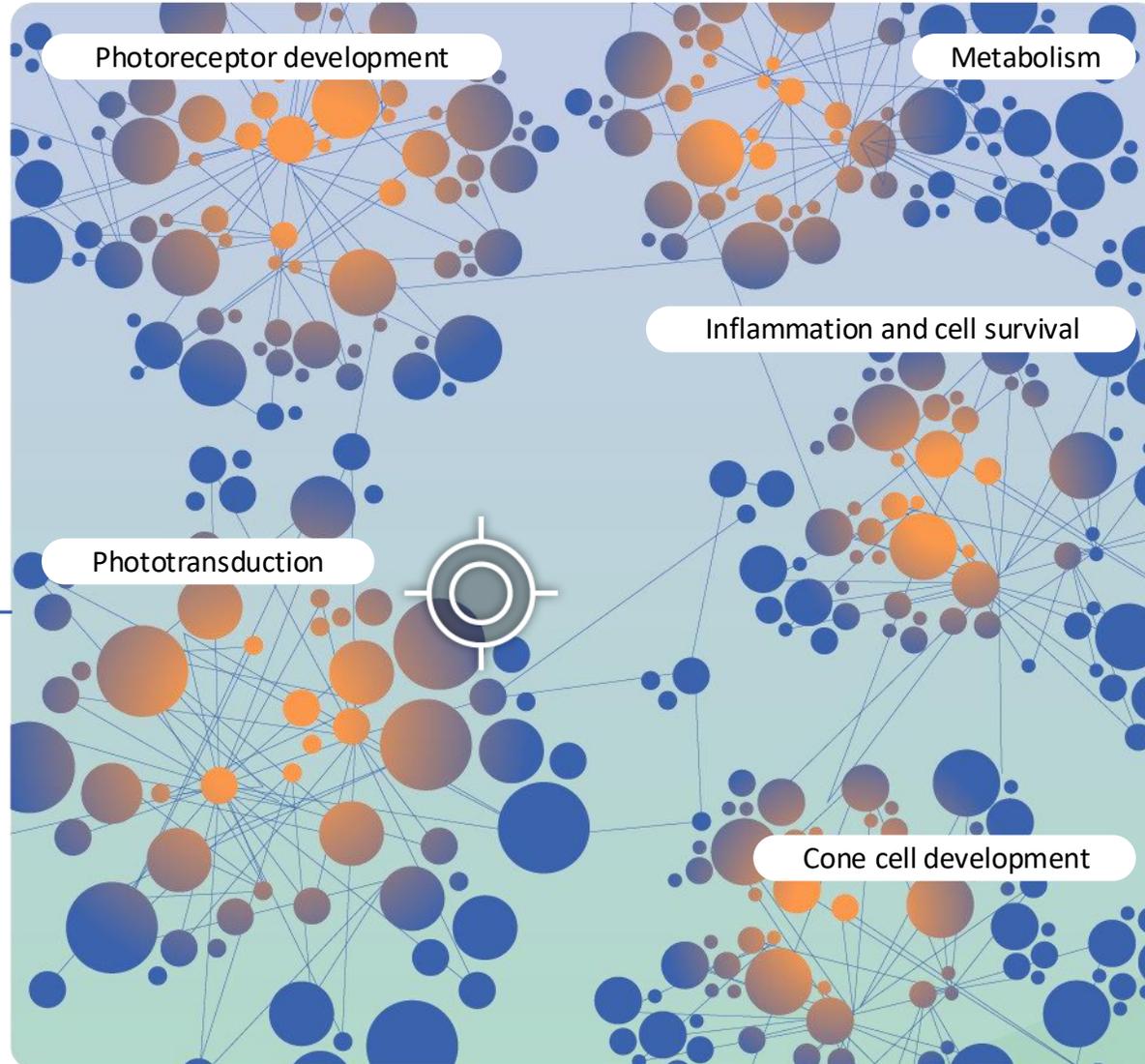
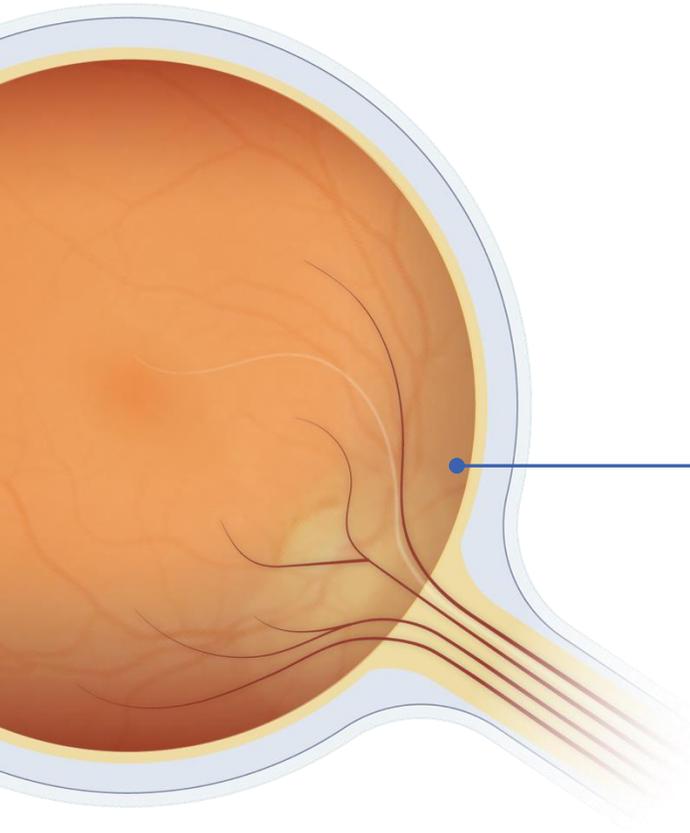
people living with retinal disease globally



<sup>1</sup> Jones BW, Marc RE, Pfeiffer RL. Retinal Degeneration, Remodeling and Plasticity. 2016 Oct 28. In: Kolb H, Fernandez E, Nelson R, editors. Webvision: The Organization of the Retina and Visual System [Internet]. Salt Lake City (UT): University of Utah Health Sciences Center; 1995-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK482309/>  
<sup>2</sup> <https://www.nature.com/articles/s41434-024-00440-6>

# 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

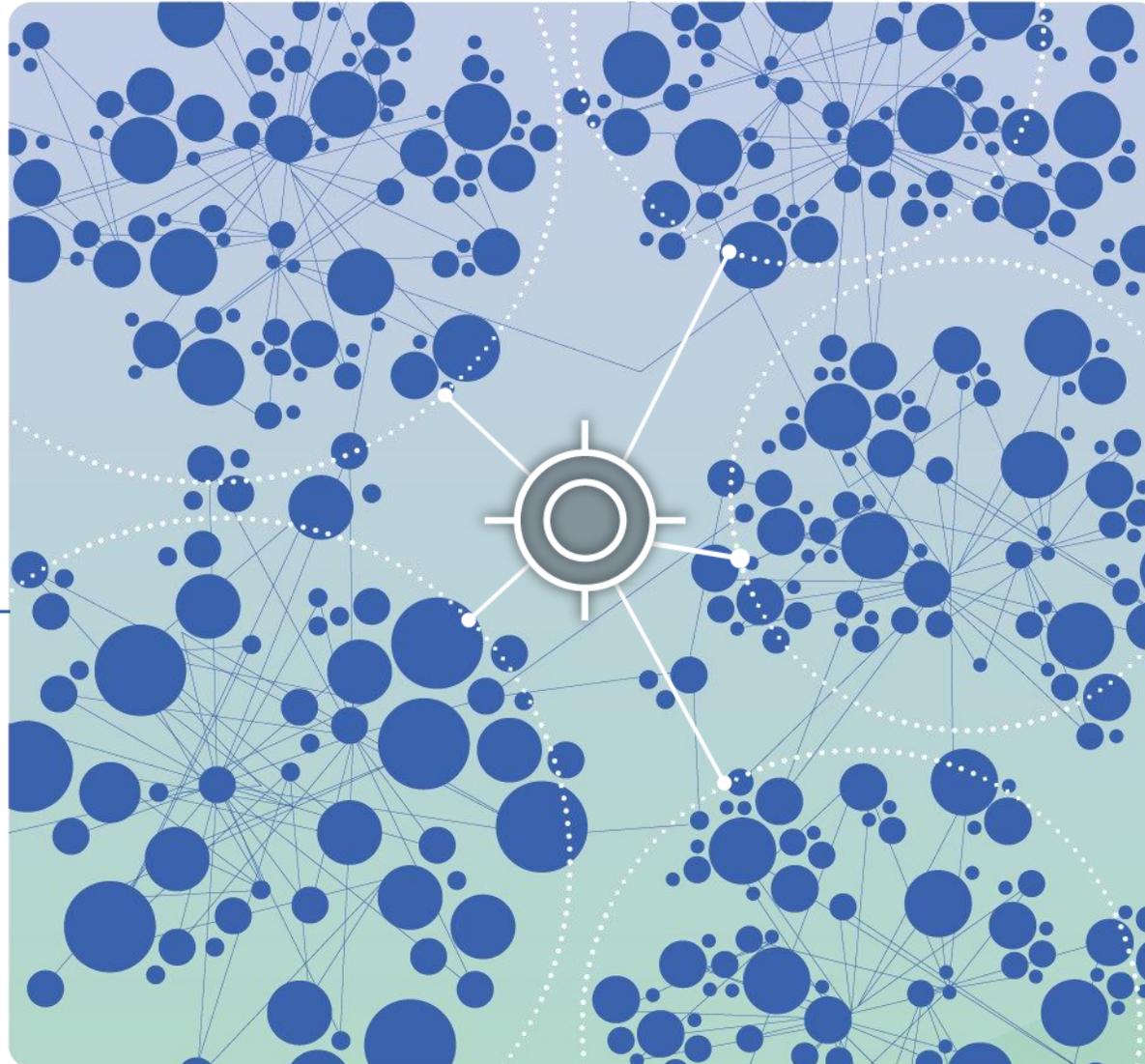
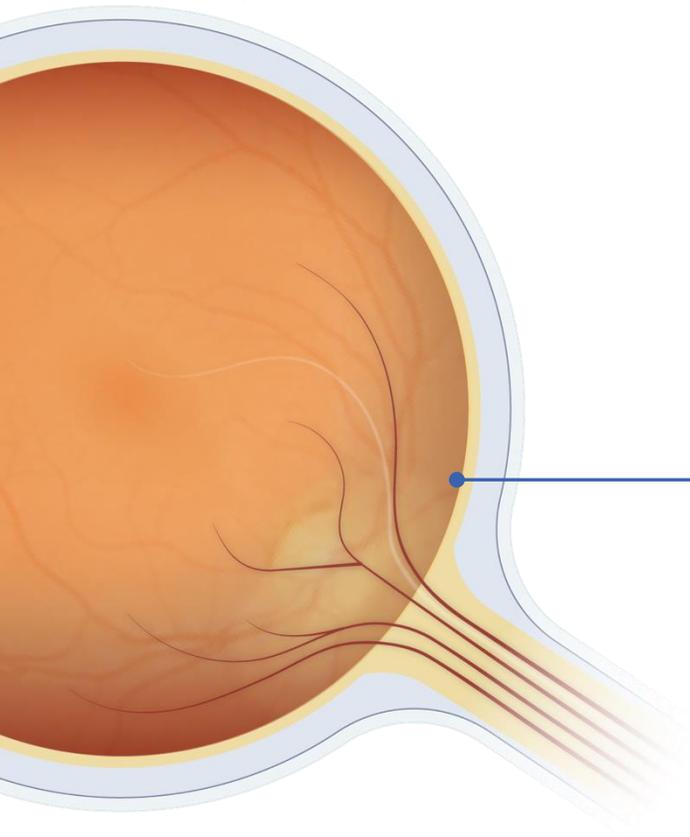
genes are highly specialized to it, interacting through complex pathways

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

# Modifier Gene Therapy Platform

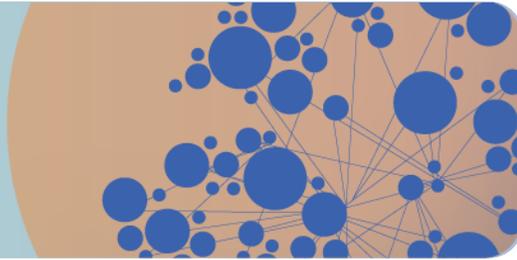
1. AAV5  
Carries Modifier  
Gene (M)\*



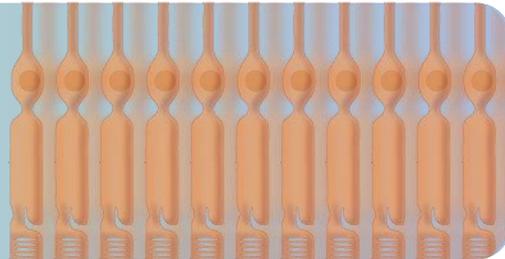
2. Modifier Gene (M)  
Regulation of target gene  
Expression



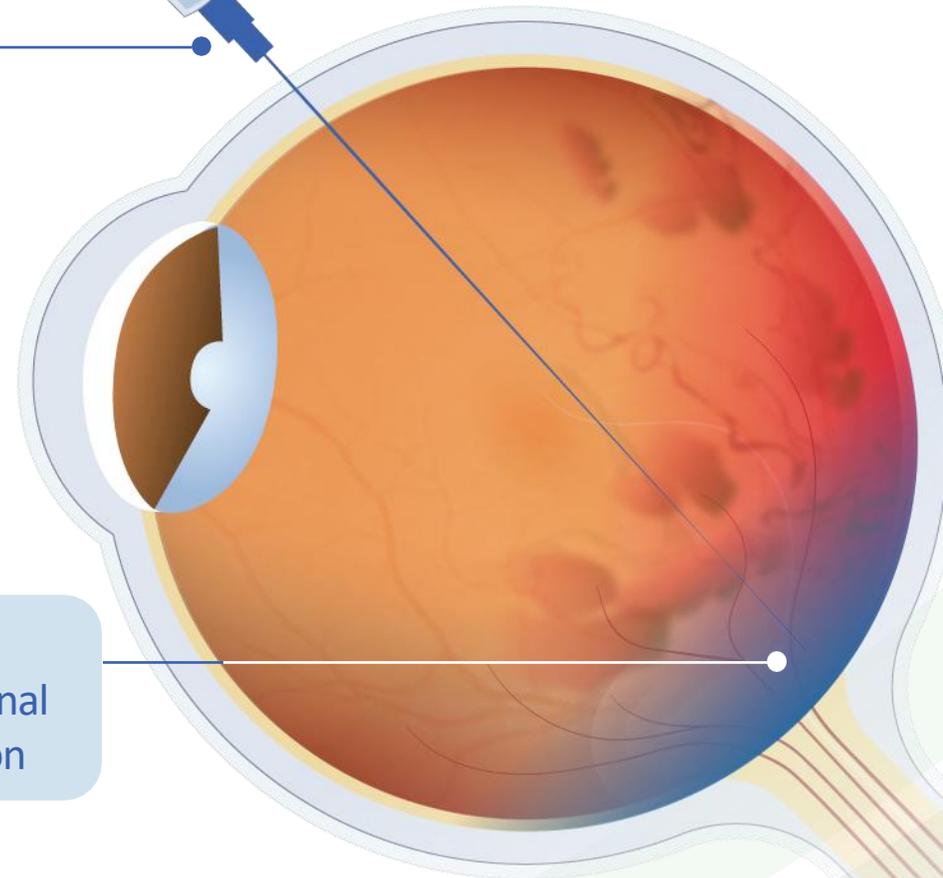
3. Molecular Reset of  
the Gene Network  
Restoration of Molecular and  
cellular homeostasis



4. Retina  
Increased survival of retinal  
cells



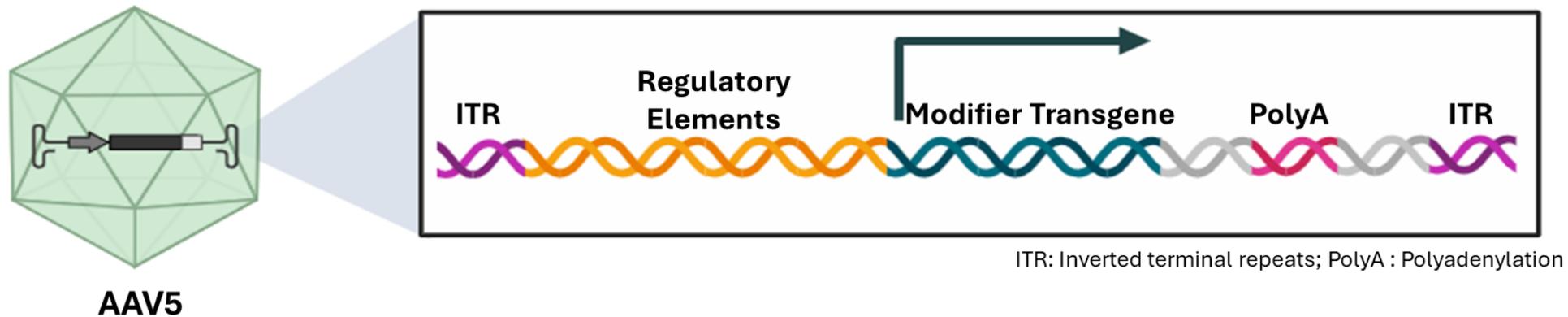
Single  
subretinal  
injection



\* Modifier Genes: *NR2E3* or *RORA*

# Modifier Gene Therapy: A Platform Technology

Based on AAV5 Vector and Nuclear Hormone Receptor Genes



## Key Attributes of AAV5 vector:

- Non-integrating Vector
- Enhanced Tissue Specificity
- High Transduction Efficiency
- Long Term Gene Expression
- Low Immunogenicity

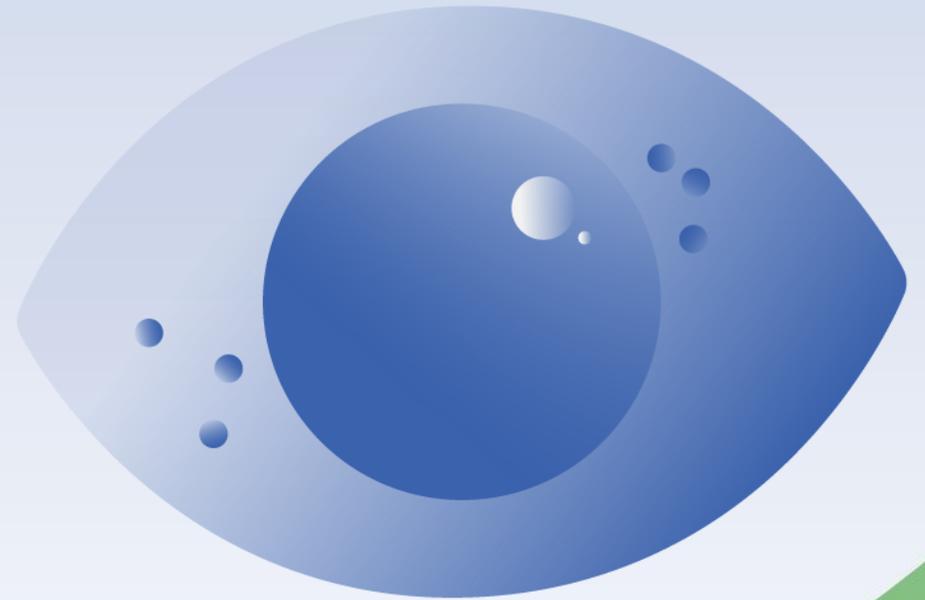
## Product Highlights:

- Novel Gene Agnostic Therapy
- Broad Patient Eligibility
- Durable Benefit
- Favorable Safety & Tolerability Profile
- Proven Clinical Efficacy

# OCU400

## Retinitis Pigmentosa (RP)

Broad indication, gene-agnostic, targets 100+ genes



# First-in-Class Gene Therapy for Retinitis Pigmentosa

## Retinitis Pigmentosa

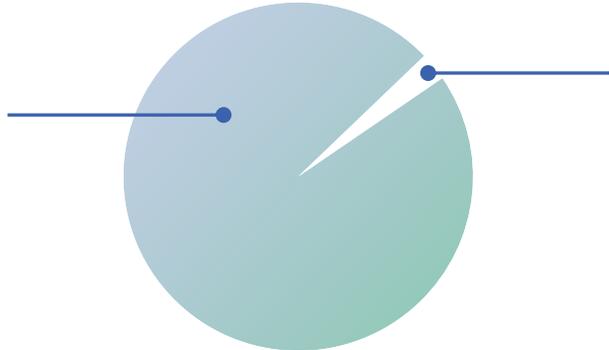
Retinitis Pigmentosa (RP) is a group of rare, inherited retinal diseases caused by mutations in over 100 genes, leading to progressive vision loss and, in many cases, blindness.

**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
- Manufacturing process validation
- Rolling Submission U.S. (BLA)  
Followed by EU (MAA)

### Designations

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

# A Novel Modifier Gene Therapy for RP Patients

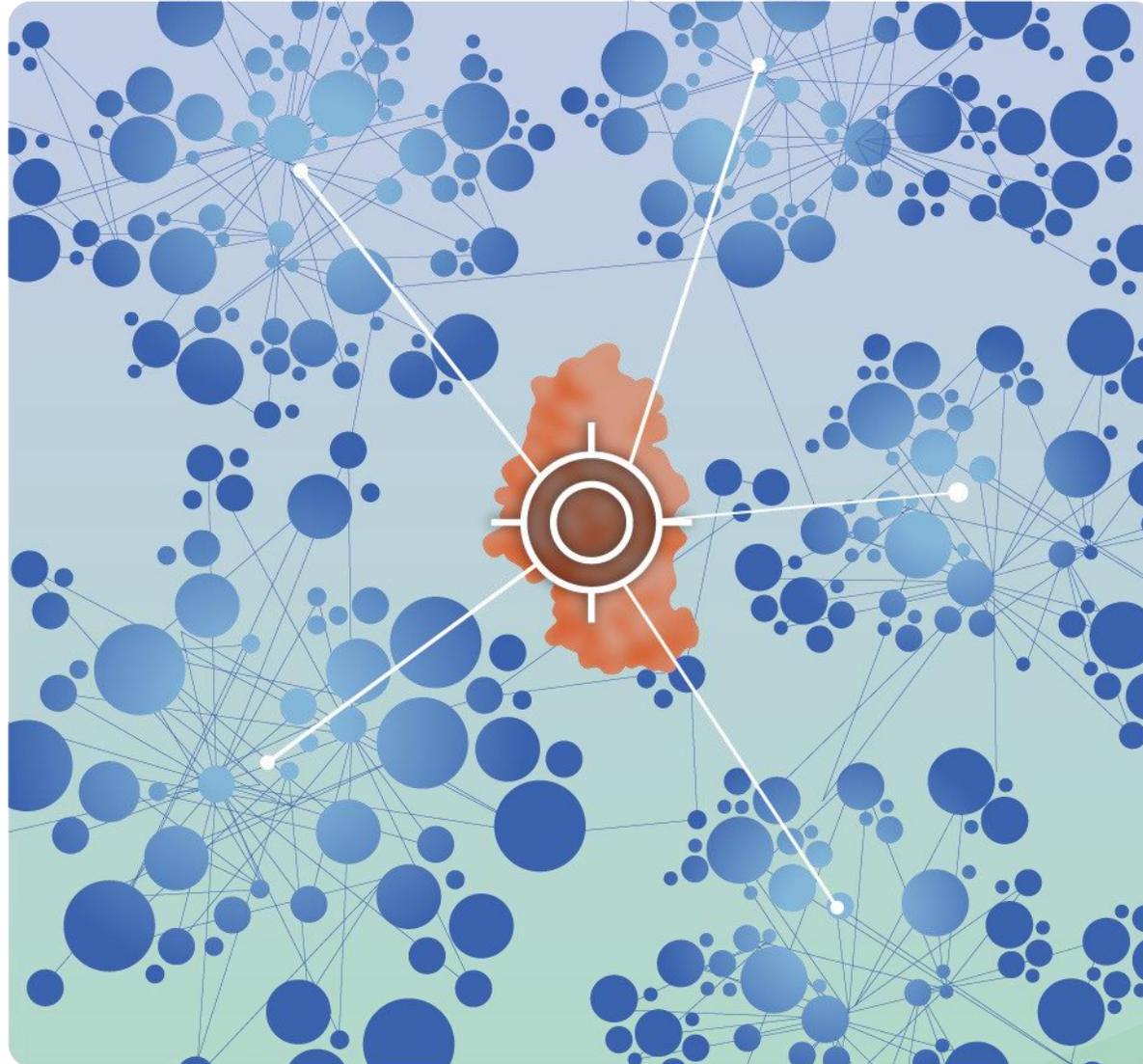
A gene-agnostic,  
broad-spectrum approach  
targeting retinal health at  
the root

Potential curative therapy with a  
single subretinal injection using  
NR2E3



Preclinical data demonstrates  
activity across multiple retinal  
degeneration mouse models

- ✓✓ rd1
- ✓✓ Rho <sup>-/-</sup>
- ✓✓ Rho P23H
- ✓✓ rd16
- ✓✓ rd7



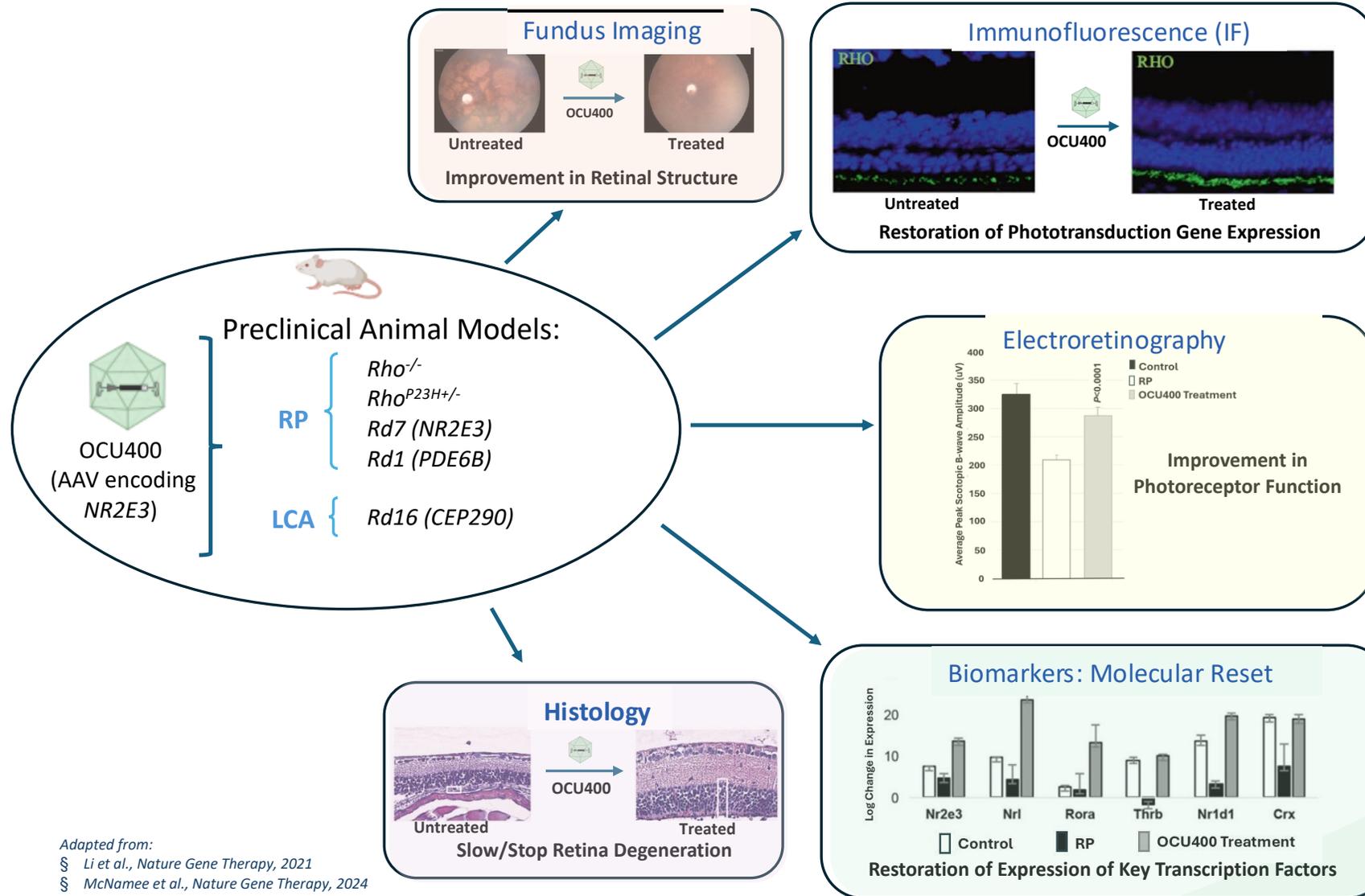
## NR2E3

Retina-specific  
gene expression

"Master Regulator"  
of many genetic  
pathways

Resets transcriptional  
networks and restores  
retinal health

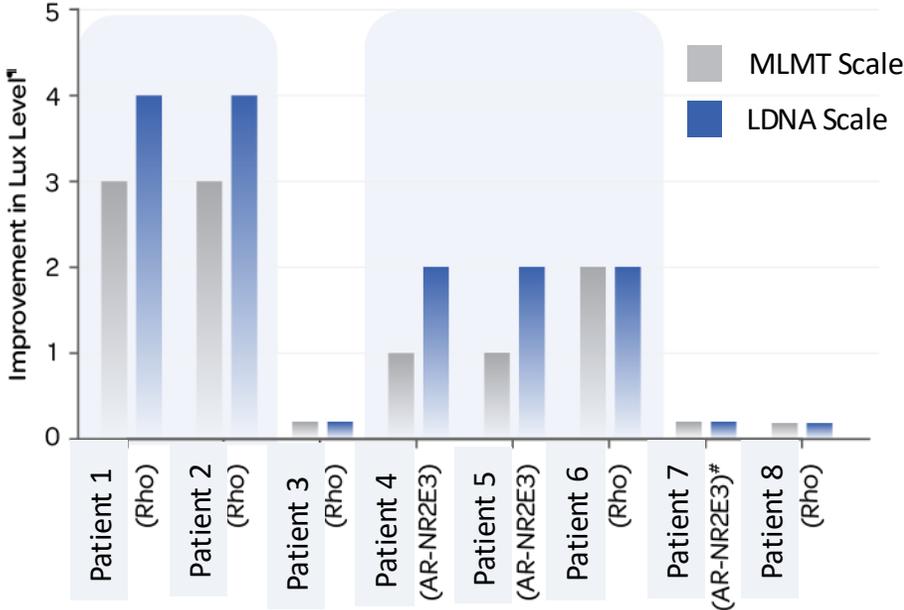
# Preclinical studies demonstrating MOA



Adapted from:  
 § Li et al., *Nature Gene Therapy*, 2021  
 § McNamee et al., *Nature Gene Therapy*, 2024

# Improved Patient Visual Function and Field in Phase 1/2 Clinical Trials

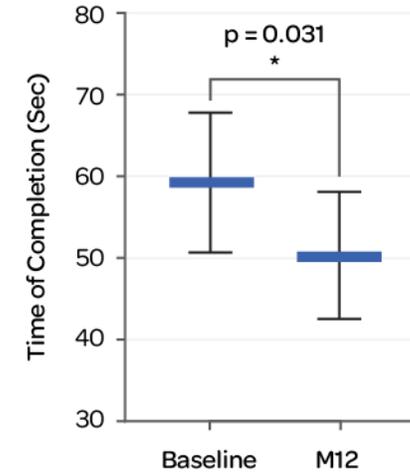
Eye Mobility Under Low Light



**63%**  
of Treated Eyes  
Showed  
Improvement

from baseline after 12 months

Mobility Test Improvement



**10s**  
Improvement  
statistically significant  
improved in MT completion  
in Treated Eyes (p=0.031)

Subject ID (with Mutation)

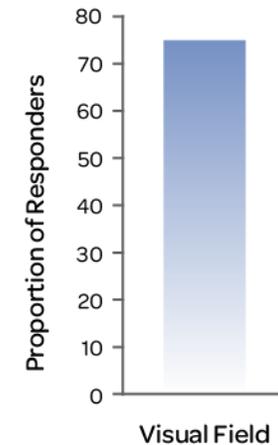
MLMT Scale (Phase 1/2)



LDNA Scale (Phase 3)



Visual Field Improvement



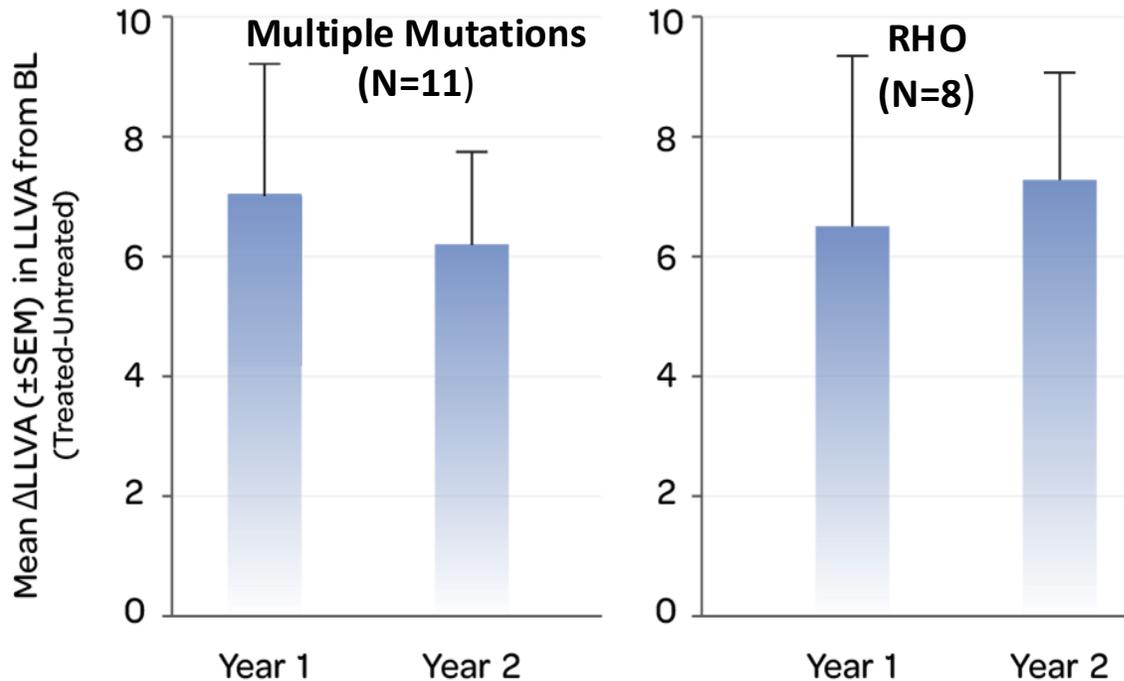
**75%**  
Improvement  
of VF in Treated Eyes in ITT  
subgroup demonstrated  
compared to untreated eyes  
(6/8)

\*RHO +AR-NR2E3 Subjects (- Adverse Events, Sentinel); and Ceiling Effect (RHO) Subjects; ^ ceiling effect (AR-NR2E3)  
# AR-NR2E3 Subjects: Baseline MLMT at 5 Lux level; 1Lux level improvement resulted in ceiling effect on old scale on 0-6 Lux levels

¶ Subjects 001-003, 003-001, 001-005, 002-002 and 003-006 were responders based on the adapted LDNA Scale rounded to the nearest Lux level. Visual field is represented as improvements in VTOT or V30 compared to untreated eyes

# Long-Term Safety & Durability Data

## 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**  
Serious Adverse Events Reported

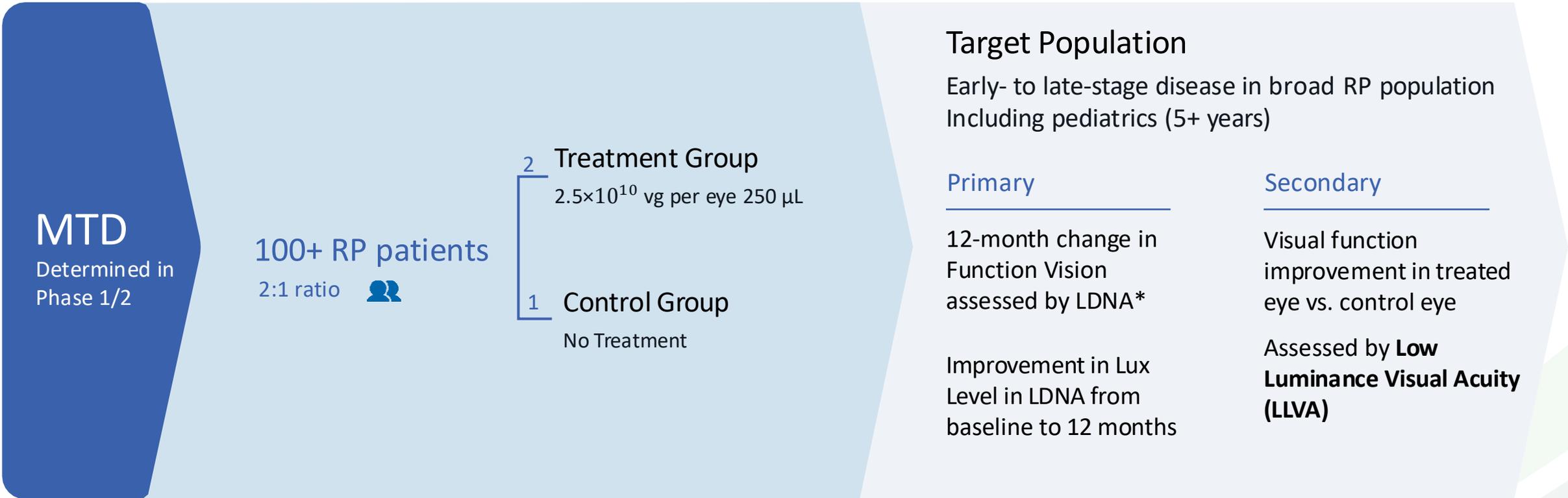
**100%**  
treated evaluable subjects showed improvement or preservation  
in visual function compared to untreated eyes

OCU400 demonstrated a durable and statistically significant (p=0.005) improvement in visual function (LLVA) in all evaluable treated subjects at 2 Yr when compared to untreated eyes

# Phase 3 liMeliGhT Trial—Largest RP Data Set

## Phase 3 Study Design

## Endpoints

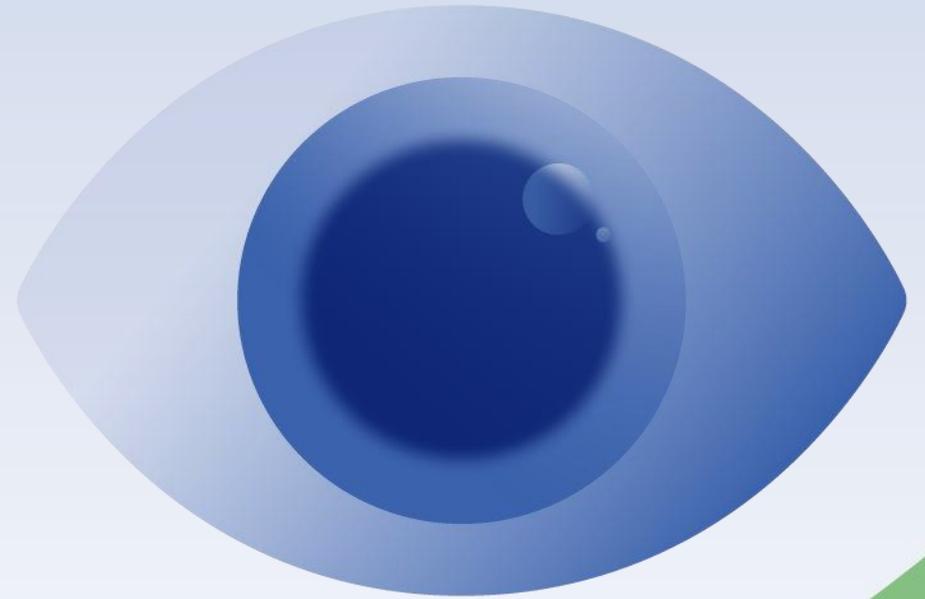


\*LDNA= Luminance Dependent Navigation Assessment is a mobility test administered on a maze under different lux levels

# OCU410ST

## Stargardt Disease

ABCA4 -associated retinopathies >1,200 mutations



# 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

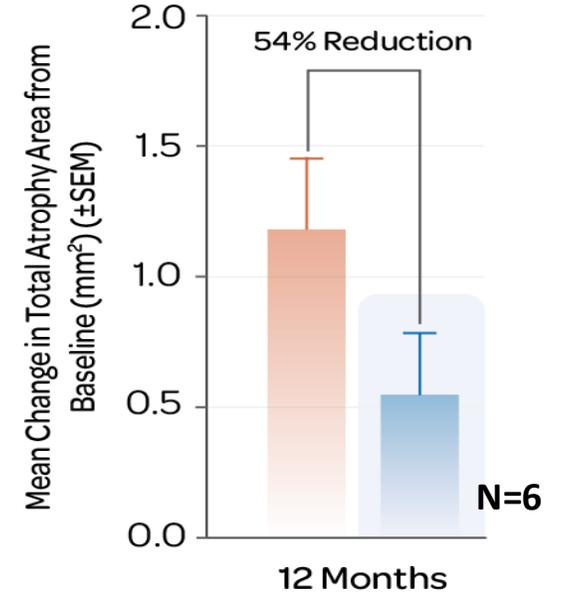
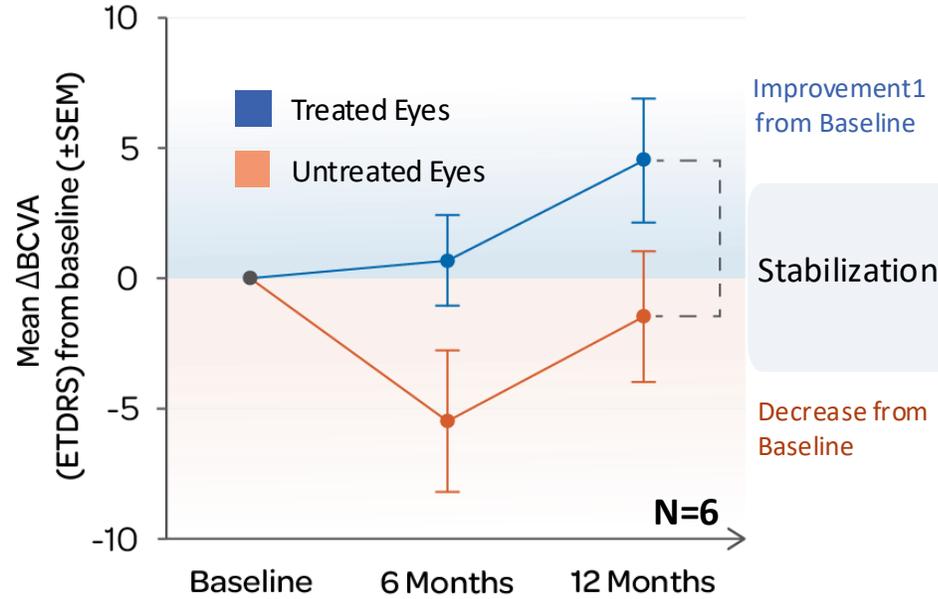
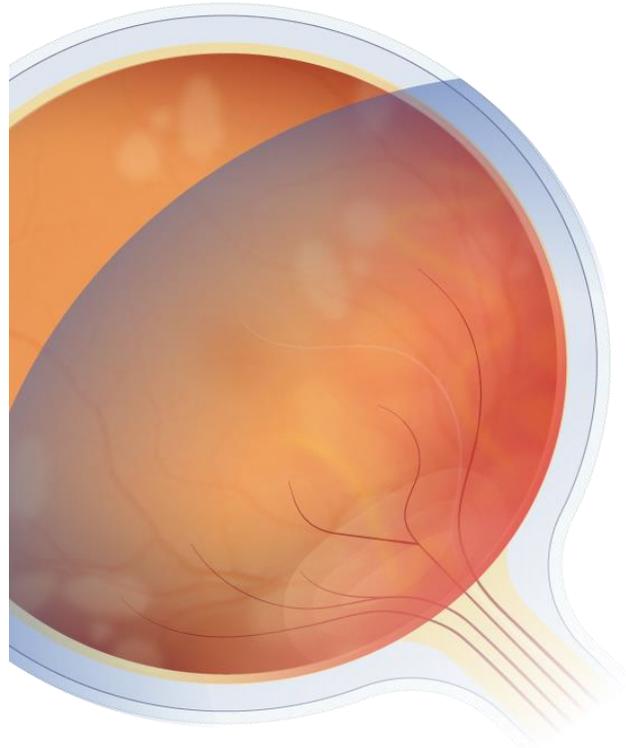
(Completed/anticipated)

- **2025**  
Initiated pivotal Phase 2/3
- **2026**  
Complete enrollment  
Interim analysis
- **2027**  
Topline Data, BLA submission

### Designations

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

# Phase 1 GARDian1 Trial Demonstrated Clinically Meaningful Benefit



Nearly  
**1-line gain**  
In visual acuity compared to untreated eyes

Visual Function\*  
**100%**  
Stabilized or Improved  
compared to untreated eyes

Atrophic Lesion Growth\*  
**54% slower**  
compared to untreated eyes

**No Serious Adverse Events Reported**

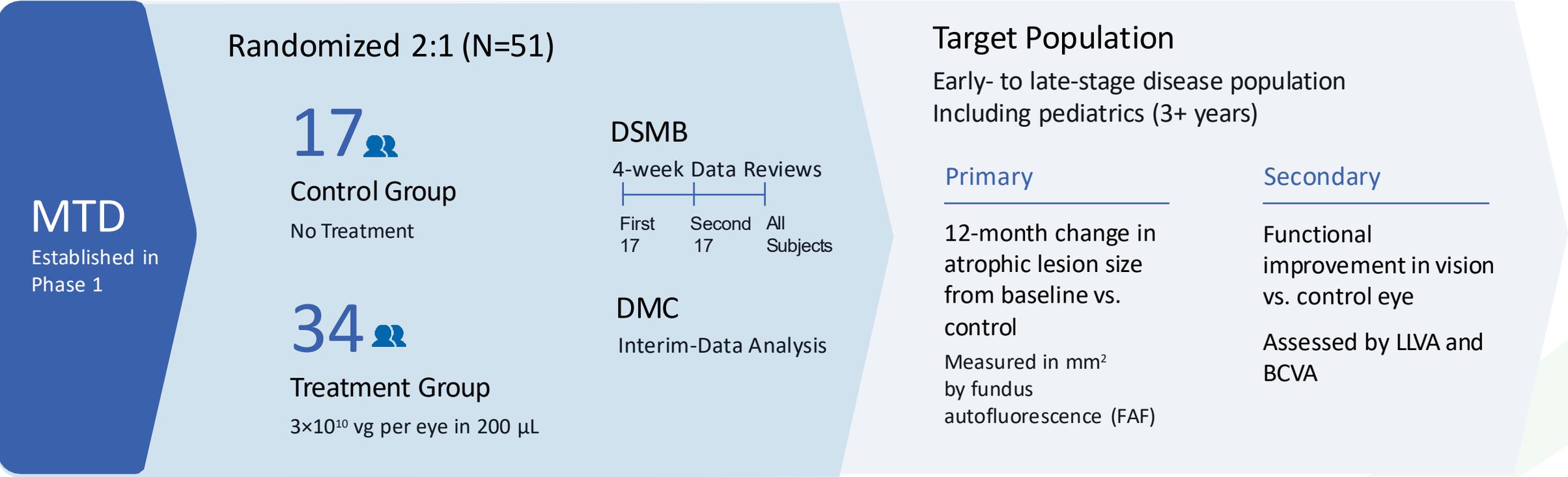
Improvement or Preservation in evaluable Treated Eyes  
Preservation = -/+4 letters from Baseline, Improvement: ≥5 Letters from Baseline

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

# GARDian 3- Phase 2/3 Pivotal Confirmatory Trial

## Trial Design

## Endpoints



Adaptive design with sample size re-estimation [Mid-2026]

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 full data release
- 2026  
Initiate Phase 3
- 2027  
Complete enrollment
- 2028  
Topline data, BLA submission

### Designations

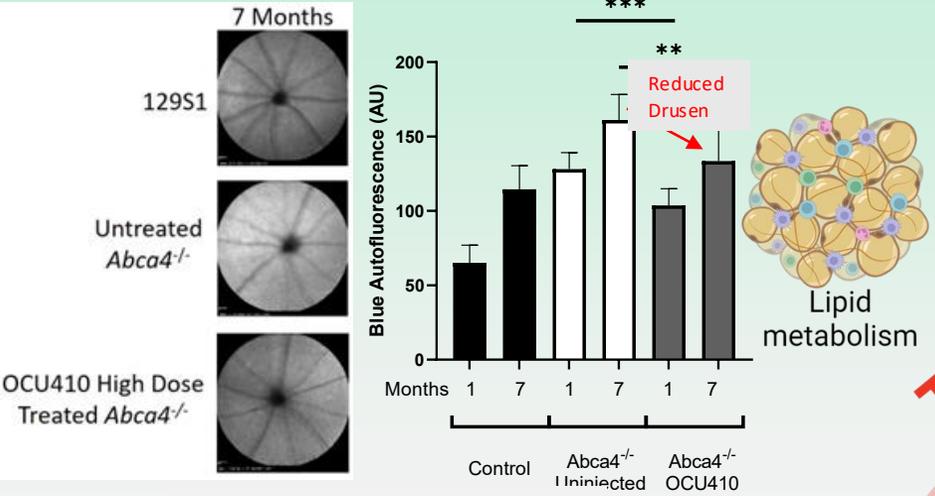
✓✓ EMA (ATMP)

### Recent Milestone

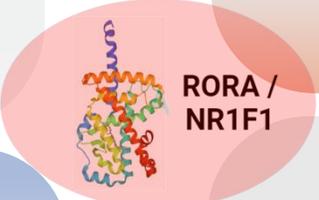
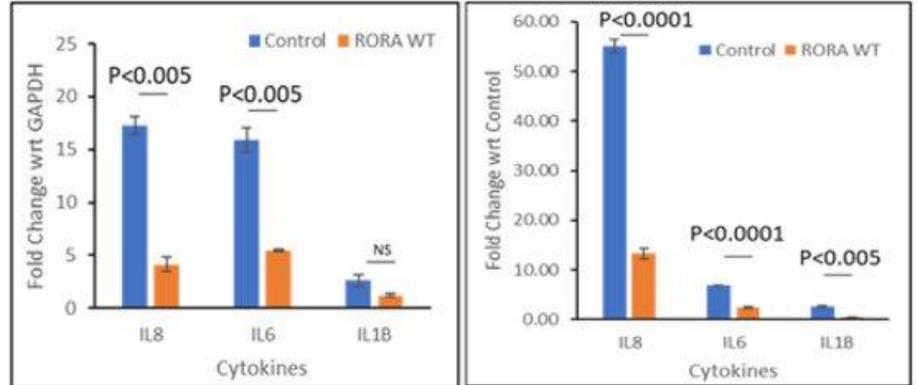
Positive Preliminary 12-month Phase 2 data

# OCU410 (RORA): A Modifier Therapeutic Approach for dAMD

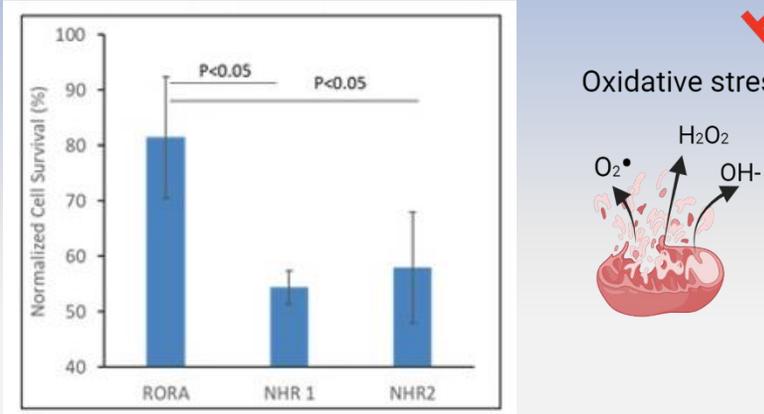
*Anti-drusen activity and improves retinal function*



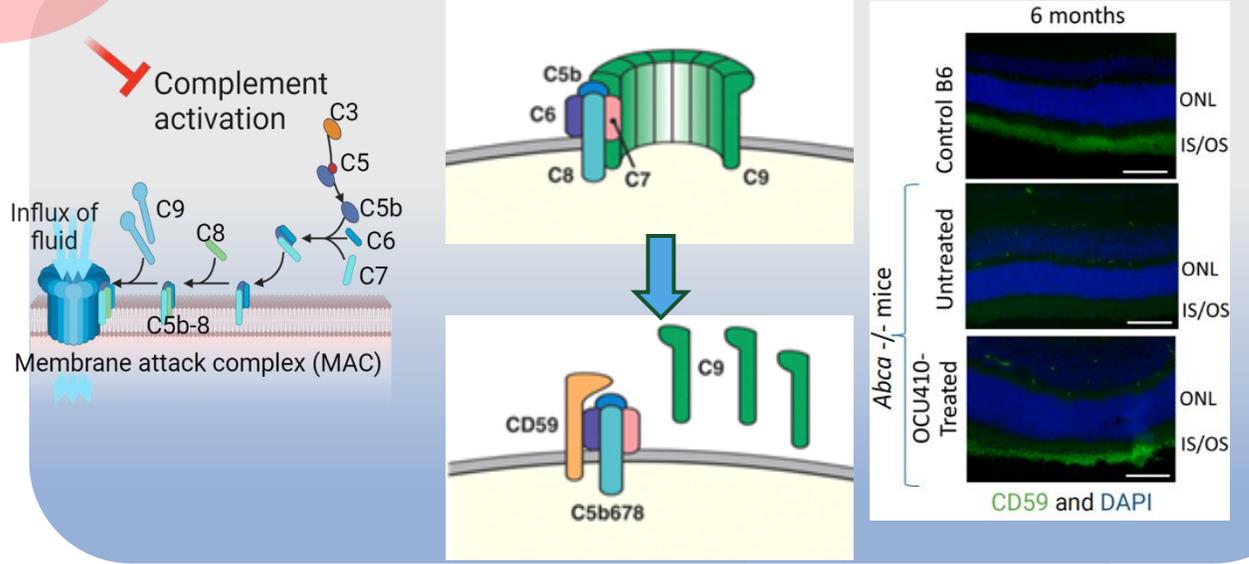
*Anti-inflammatory: Suppresses inflammation in HMC3 cells*



*Anti-oxidative: Improves ARPE19 cells survival*



*Anti-complement: Increased anti-complement (Cd59) protein*



# Phase 1 ArMaDa Trial

## Trial Design

### Dose Escalation (1:1:1)

3 

#### Low Dose Group

2.5x 10<sup>9</sup> vg/ml in 200µL  
(or 5x 10<sup>9</sup> vg per eye)

3 

#### Medium Dose Group

5x 10<sup>10</sup> vg/ml in 200µL  
(or 1x 10<sup>10</sup> vg per eye)

3 

#### High Dose Group

1.5x 10<sup>11</sup> vg/ml in 200 µL  
(or 3x 10<sup>10</sup> vg per eye)

### DSMB

4-week post-OCU410  
Safety Reviews



## Endpoints

### Target Population

GA secondary to dAMD

#### Primary

- Safety, AEs and SAEs
- Indirect ophthalmoscopy
- IOP

#### Secondary

- Humoral/cellular immune response
- Vector shedding

#### Exploratory

- Indirect ophthalmoscopy
- Fundus Autofluorescence
- Drusen volume (mm<sup>3</sup>) measured by SD-OCT
- Incidence of conversion to wet AMD
- MAIA
- Patient Reported Outcomes (NEI-VFQ25)

CNV in fellow eye was not exclusionary

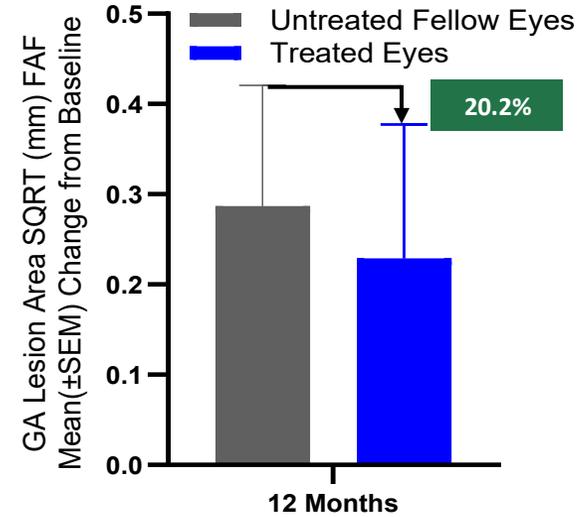
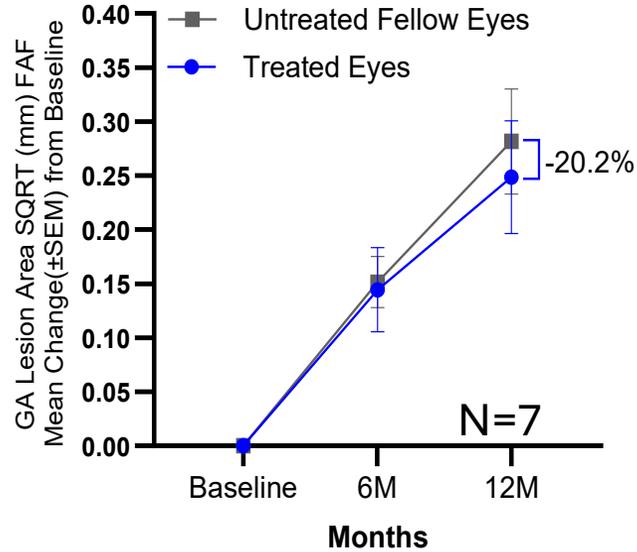
# OCU410 Phase 1—Baseline Characteristics and Safety

	Mean ± SEM Study Eye	Mean ± SEM Fellow Eye
Age (Years)	79 ± 8	
BCVA Letters	46.89 ± 5.98	59.67 ± 8.07
LLVA Letters	34.78 ± 5.71	41.89 ± 7.59
Lesion Size at BL (mm <sup>2</sup> )	7.74 ± 1.54	8.19 ± 1.81

No adverse or severe adverse events related to the study drug were reported including:

- No development of exudation
- No infectious endophthalmitis
- No intraocular Inflammation
- No anterior ischemic optic neuropathy (AION)
- No vasculitis

# Phase 1—OCU410 Treatment shows reduction in GA Lesion Growth



Lesion Size

**20.2% Reduction**

compared to untreated fellow eye

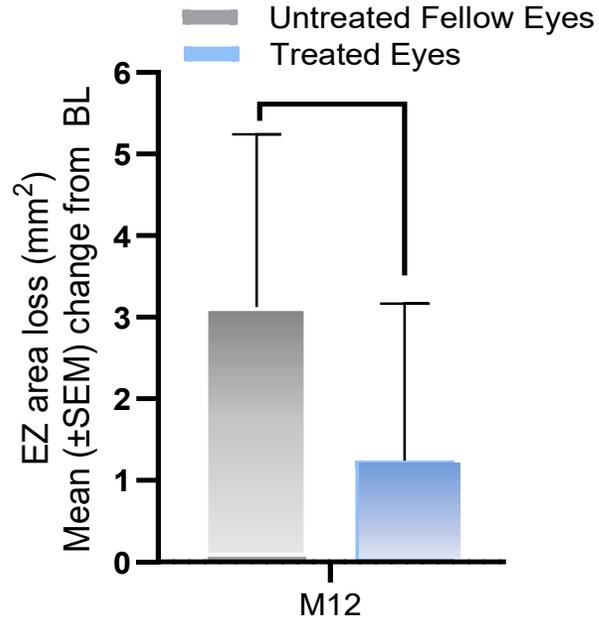
Disease Progression

**Slower Atrophy Growth**

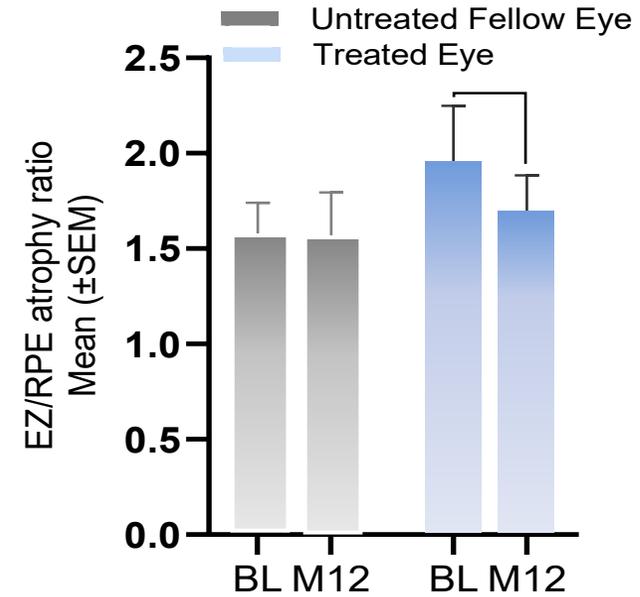
compared to untreated fellow eye

Data points at 12M (N=7, 3 Low Dose, 2 Med Dose, 2 High Dose)  
 1 High dose subject with loss-to-follow up, and 1 Med dose subject with foveal detachment during surgery were not included in the analysis  
 GA= Geographic Atrophy, SQRT= square root; SEM= standard error or mean

# Phase 1—Treatment Provides Retinal Structural Preservation by Reducing EZ Loss & Photoreceptor Degeneration



EZ loss  
**60% slower**  
 compared to untreated fellow eyes



EZ-RPE loss  
**Reduced**  
 compared to untreated fellow eyes  
 potential treatment effect

M=Months  
 Data points at 12M (N=7, 3 Low Dose, 2 Med Dose, 2 High Dose)  
 1 High dose subject with loss-to-follow up, and 1 Med dose subject with foveal detachment during surgery were not included in the analysis  
 Preservation= reduction in the loss of EZ and EZ/RPE (photoreceptors) as a treatment outcome

# Phase 2 ArMaDa Trial

## Trial Design

### MTD

Determined in Phase 1

Randomization  
1:1:1

17 

Control Group

No Treatment

17 

Medium Dose Group

$5 \times 10^{10}$  vg/ml in 200 $\mu$ L  
(or  $1 \times 10^{10}$  vg per eye)

17 

High Dose Group

$1.5 \times 10^{11}$  vg/ml in 200  $\mu$ L  
(or  $3 \times 10^{10}$  vg per eye)

### DSMB

4-week Safety Reviews



## Endpoints

### Target Population

Geographic atrophy secondary to Dry AMD

#### Primary

Change in GA lesion size

Measured in mm<sup>2</sup>  
by fundus  
autofluorescence (FAF)

#### Secondary

Change in LLVA from  
baseline at 12M

Preservation of retinal  
tissue around areas of  
atrophy

## Phase 2—Baseline Characteristics and Safety

<b>Age (Years)</b>	<b>75.96 ± 6.27</b>		
<b>Male</b>	20 (39.2)		
<b>Female</b>	31 (60.8)		
	<b>Mean ± SD Overall Study</b>	<b>Mean ± SD Treated Eye</b>	<b>Mean ± SD Control Eye</b>
<b>BCVA Letters</b>	53.96± 19.27	56.55 ± 18.93	47.15 ± 19.20
<b>LLVA Letters</b>	30.32±17.18	30.41 ± 18.30	30.07 ± 14.50
<b>Lesion Size at BL (mm<sup>2</sup>)</b>	8.67 ± 4.89	8.48 ± 4.59	9.14 ± 5.74

### Side effects

Endophthalmitis and Retinal Detachments

Retinal Vasculitis and/or Retinal Vascular Occlusion

Choroidal Neovascularization

Intraocular Inflammation

Optic ischemic Neuropathy

Treatment Emergent Serious Adverse Events

Treatment Emergent Adverse Events considered severe

### OCU410

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Products with every-other-month and every month injections

✓

✓

✓

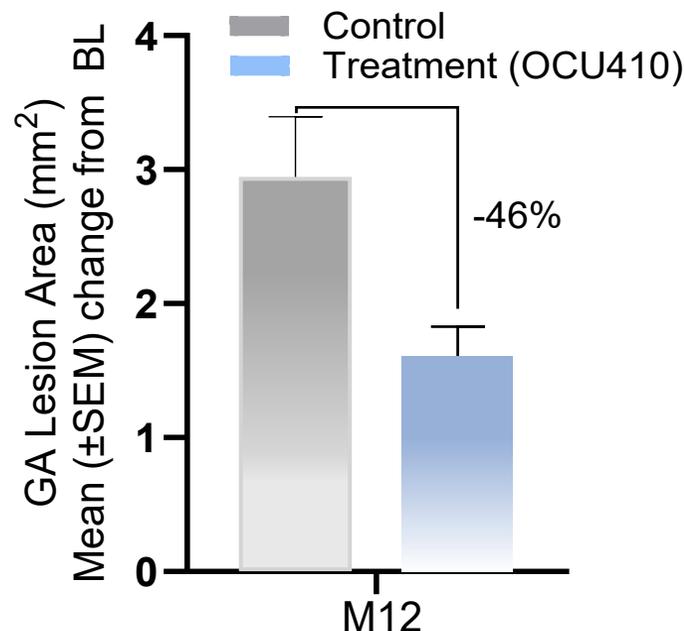
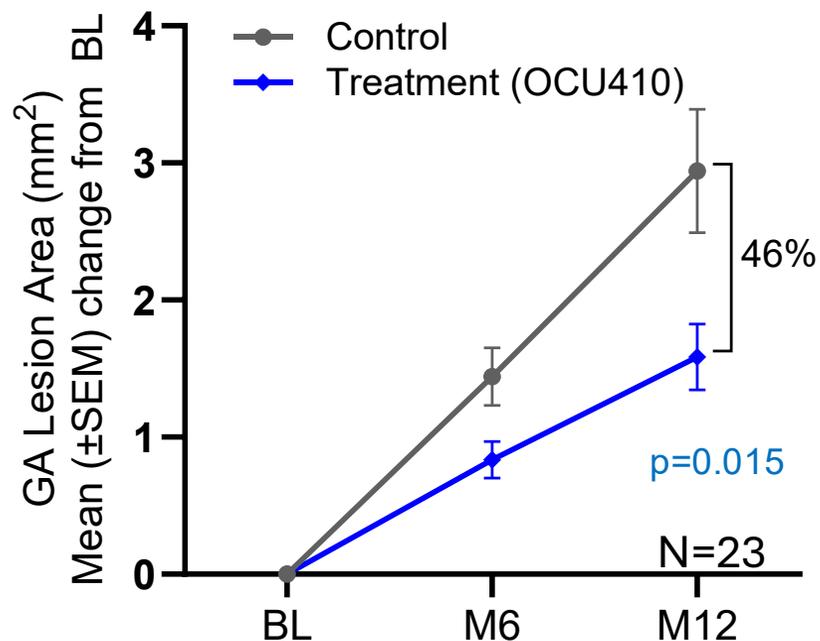
✓

✓

✓

✓

# Phase 2—Treatment Demonstrates Reduction in GA Lesion Growth at 12 Months



Lesion Size

## 46% Reduction

in treatment group compared to controls

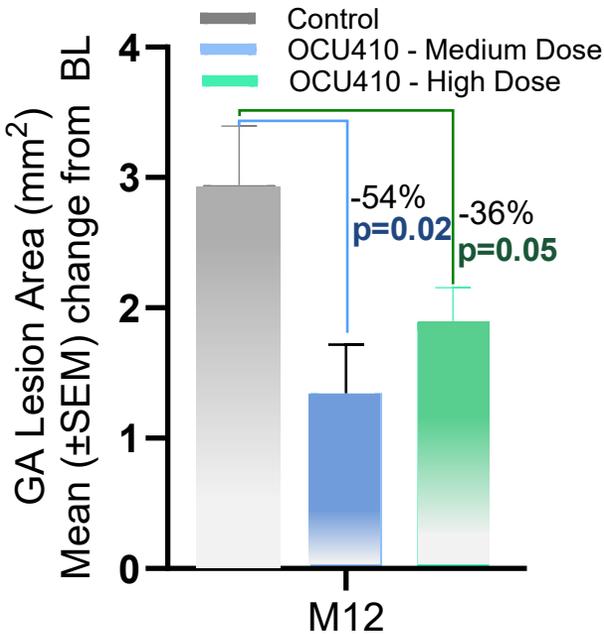
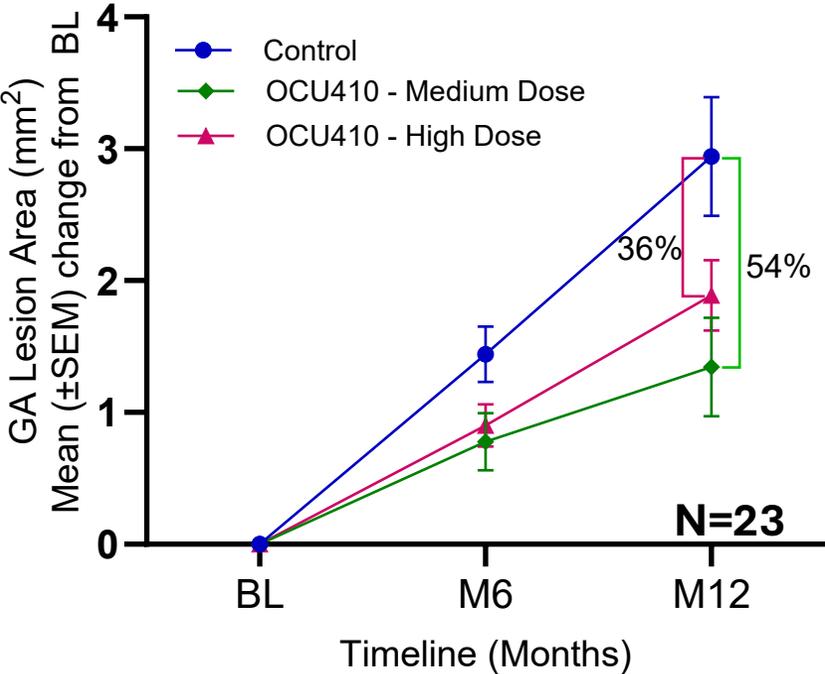
Disease Progression

## Slower Atrophy Growth

compared to controls

Includes both foveal and subfoveal GA; geographic atrophy; Treated Eyes that received OCU410 Medium dose and OCU410 High Dose were combined for analysis  
 Data points for Control, N=5; Treated Medium Dose, N=10; Treated High-Dose, N=8

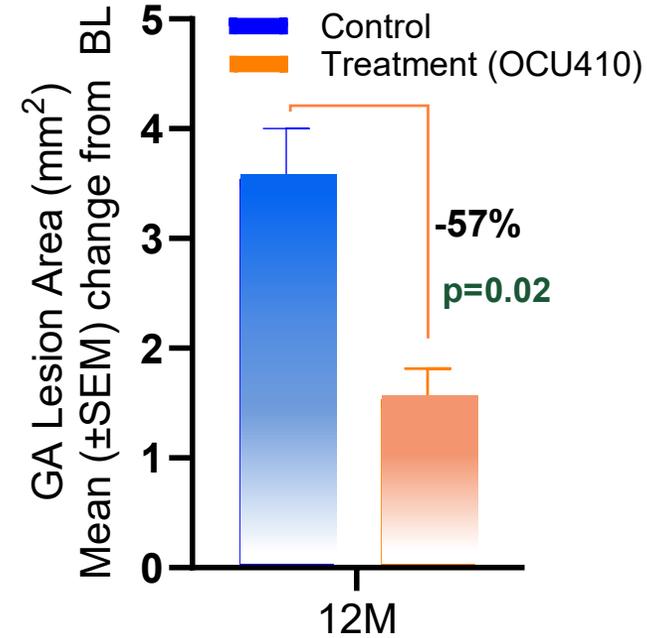
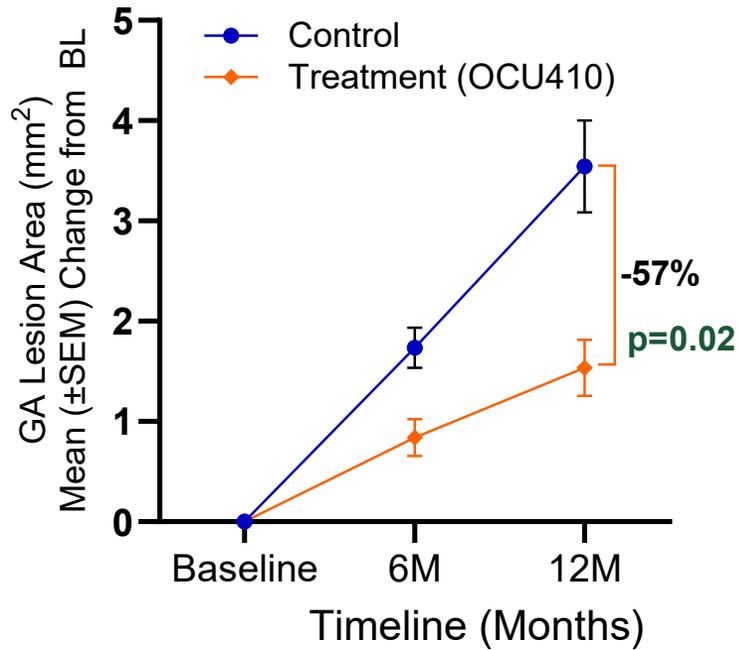
# Phase 2—OCU410 demonstrates reduction in GA Lesion Growth at 12 Months



**Lesion Size**  
**54% vs 36%**  
 Lesion reduction in medium dose and high dose compared to controls

**Disease Progression**  
**Slower Lesion Growth**  
 in both dose groups compared to controls

# Greater Lesion Reduction in Subjects with $\geq 7.5 \text{ mm}^2$ at Baseline



Lesion Size

**57% Reduction**

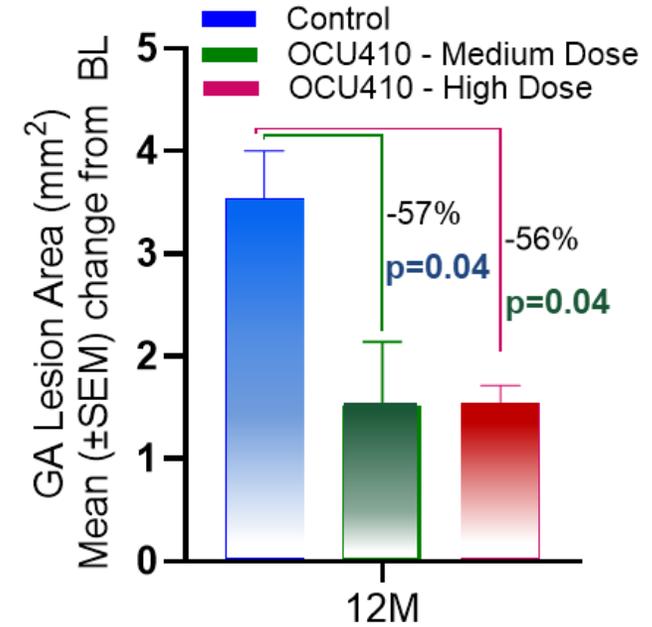
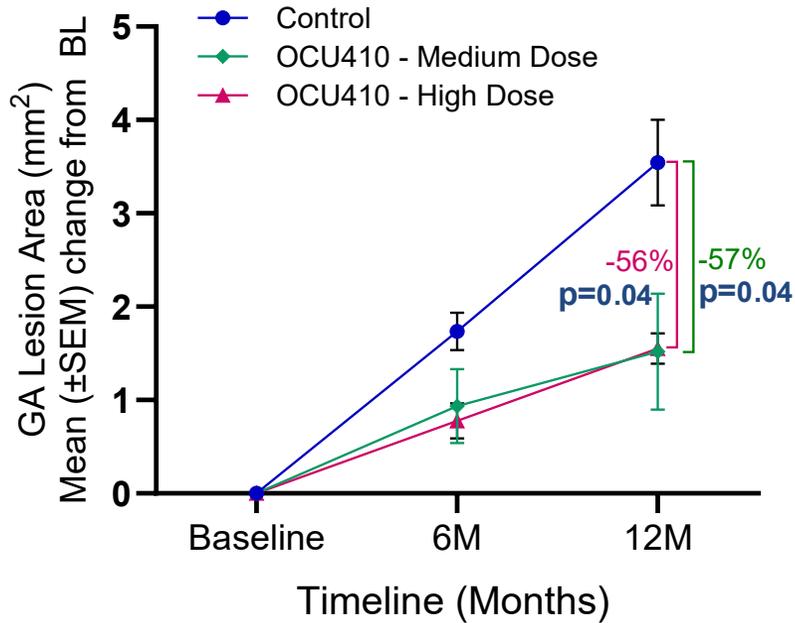
in treated eyes compared to controls

Disease Progression

**Slower Lesion Growth**

in treated eyes compared to controls

# Similar GA Lesion Growth at 12 Months for Medium and High Dose for Lesion Size $\geq 7.5\text{mm}^2$ at Baseline



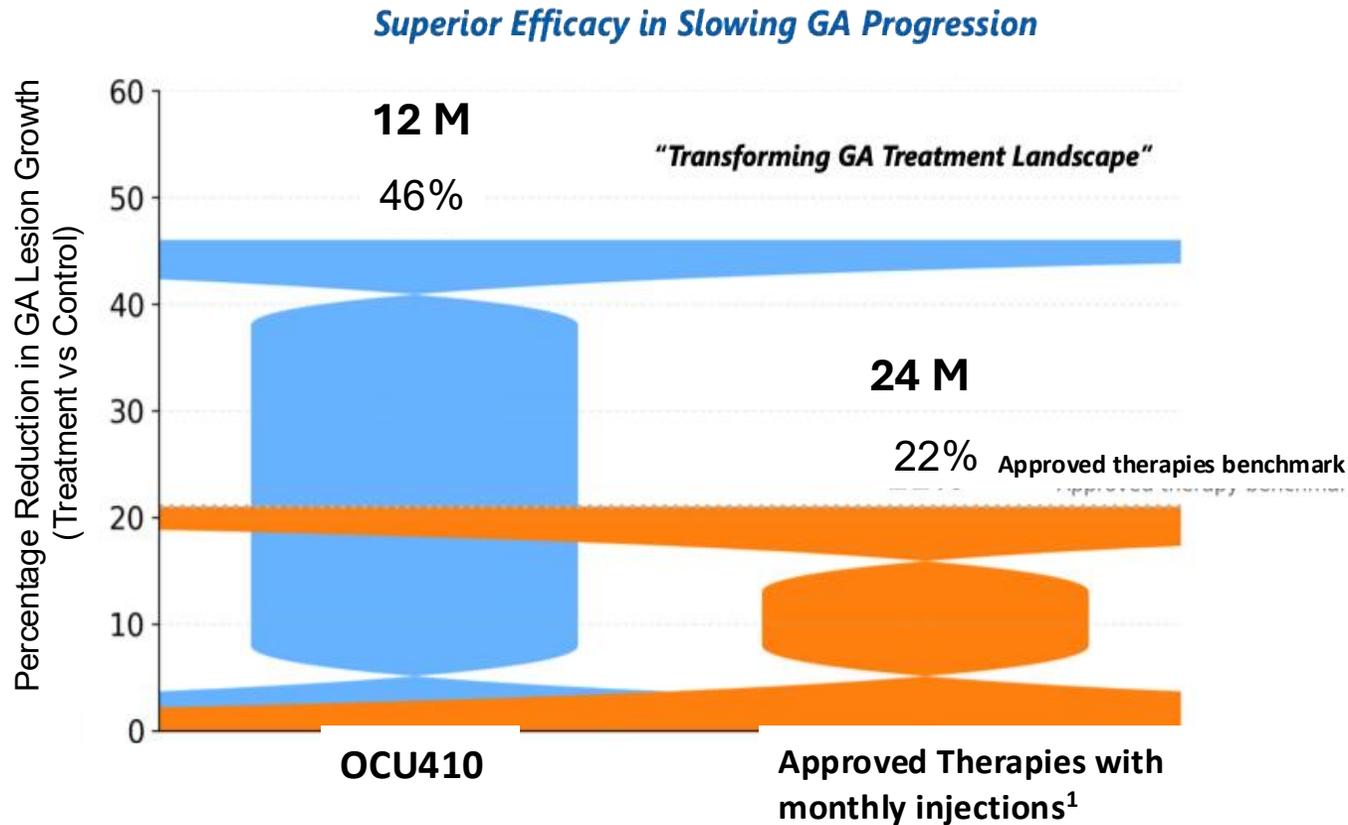
**Lesion Size**  
**57% vs 56%**  
 lesion reduction in medium dose and high dose compared to controls

**Disease Progression**  
**Slower Lesion Growth**  
 in both dose groups compared to controls

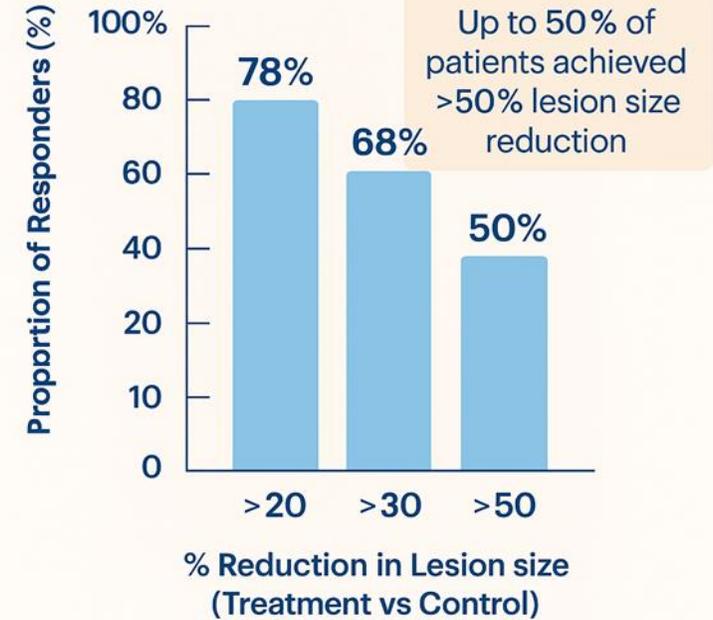
No difference observed between doses

# Phase 2—Demonstrates Superior Clinical Effects in GA Patients

## Significant Lesion Size Reduction Compared to Control



## Responder Rate to OCU410 Treatment



**OCU410**

Oaks and Derby Study reports are obtained from Heier et al., 2023, PMID:37865470

<sup>1</sup> Data presented are based on package inserts of approved therapies. OCU410 remains investigational, and no head-to-head trials have been conducted.

# Transformative Gene Therapy for Geographic Atrophy (GA)

## Comprehensive Mechanism of Action

- Targets Multiple pathways and Demonstrated anti-oxidative, anti-complement, anti-inflammatory, and anti-drusen activity in preclinical models

## Clinical Efficacy Data

- 46% reduction in treated (high + medium dose) vs control at 12M for all lesion sizes (***p value = 0.015***) ; [~22% for approved therapies in 24M]
- 57% reduction in treated (high + medium dose) vs control at 12M for lesion size  $\geq 7.5 \text{ mm}^2$  (***p value = 0.02***)
- No difference between high and medium doses at 12 months for lesion size  $\geq 7.5 \text{ mm}^2$
- Significant responder rates: up to 50% of patients achieved >50% lesion size reduction vs control in 12M

## Favorable Safety and Tolerability Profile

- No serious adverse events related to OCU410 reported to date
- No incidence of retinal detachment, vasculitis, and other complications compared to approved drugs

## Unmet Need & Market Potential

- Addresses large patient population with limited treatment options
- Potential one-time treatment for life vs monthly/bi-monthly injections for approved therapies

# Anticipated Near-Term Targeted Milestones



**OCU400**  
RP

Initiate Rolling BLA  
Submission

Phase 3  
topline data

**OCU410ST**  
Stargardt  
Disease

100% Enrollment  
Completion

Interim data

Phase 3  
top line data

BLA  
Submission

**OCU410**  
GA

Phase 2 Study Results

Initiate Phase 3





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