



Courageous Innovation

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

**OCGN
December 2024**



Forward-Looking Statements

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 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.

We're Here to Make an Impact Through Courageous Innovation

Company Overview

Founded	2013
HQ & Manufacturing	Malvern, PA
Employees	~100
Ticker Symbol	OCGN
India Business Center	Hyderabad
Market Cap	\$300 million



Our Values

- Respect
- Integrity
- Teamwork
- Accountability



Through Courageous Innovation, We are Leveraging Our First-in-Class Platforms to Address Serious Unmet Medical Needs



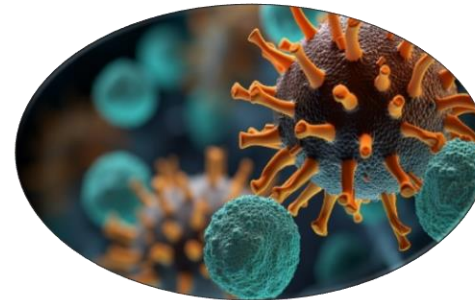
Modifier Gene Therapy Platform *First-in-Class*

- Therapeutic Focus: Inherited retinal diseases & larger blindness diseases with unmet need
- Differentiator: Master gene regulator; gene-agnostic approach
- Pipeline:
 - OCU400* Phase 3 liMeliGHt clinical trial (retinitis pigmentosa)
 - OCU410 Phase 2 ArMaDa clinical trial (geographic atrophy)
 - OCU410ST Phase 1/2 GARDian clinical trial (Stargardt)



Regenerative Cell Therapy Platform *First-in-Class*

- Therapeutic Focus: Articular cartilage lesions
- Differentiator: 3D scaffold
- Pipeline:
 - NeoCart® (Ph3): articular cartilage defects in the knee



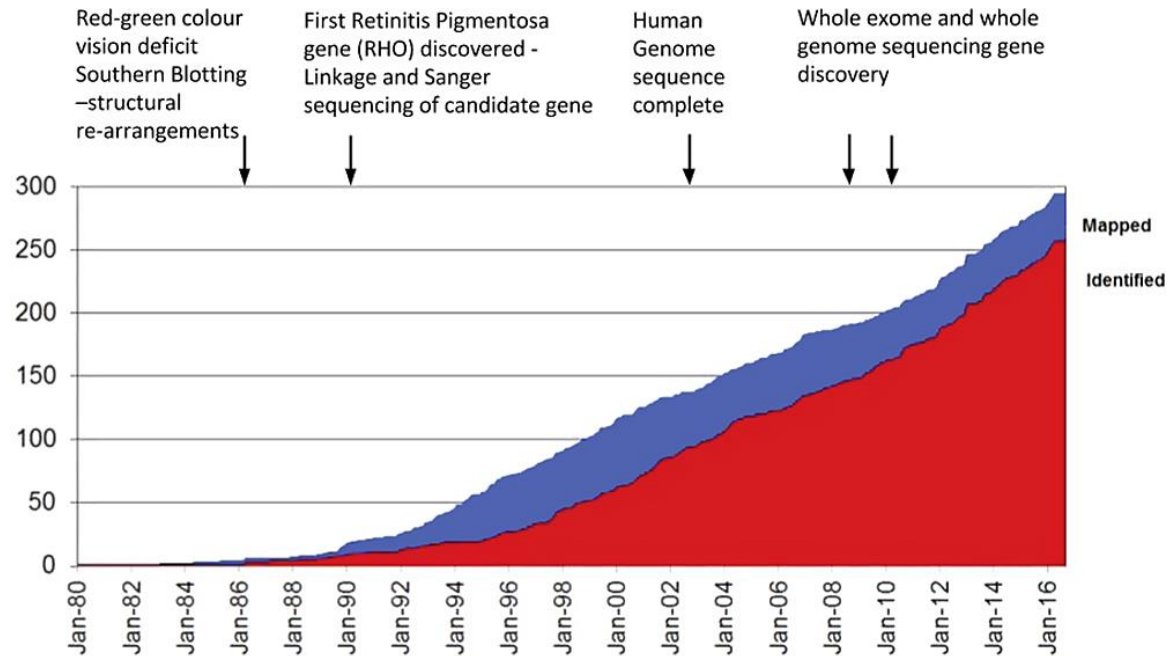
Inhalation Vaccines Platform *First-in-Class*

- Therapeutic Focus: Flu and COVID-19
- Differentiator: Inhalation for improved durability and transmission control
- Pipeline:
 - OCU500 (Pending Ph1): COVID-19 vaccine
 - OCU510 (Preclin): flu quadrivalent
 - OCU520 (Preclin): COVID-19 + flu combo

Modifier Gene Therapy Platform

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

Traditional Gene Therapy is Highly Inefficient for Retinal Diseases



Mapped and Identified Retinal Disease Genes 1980 - 2017

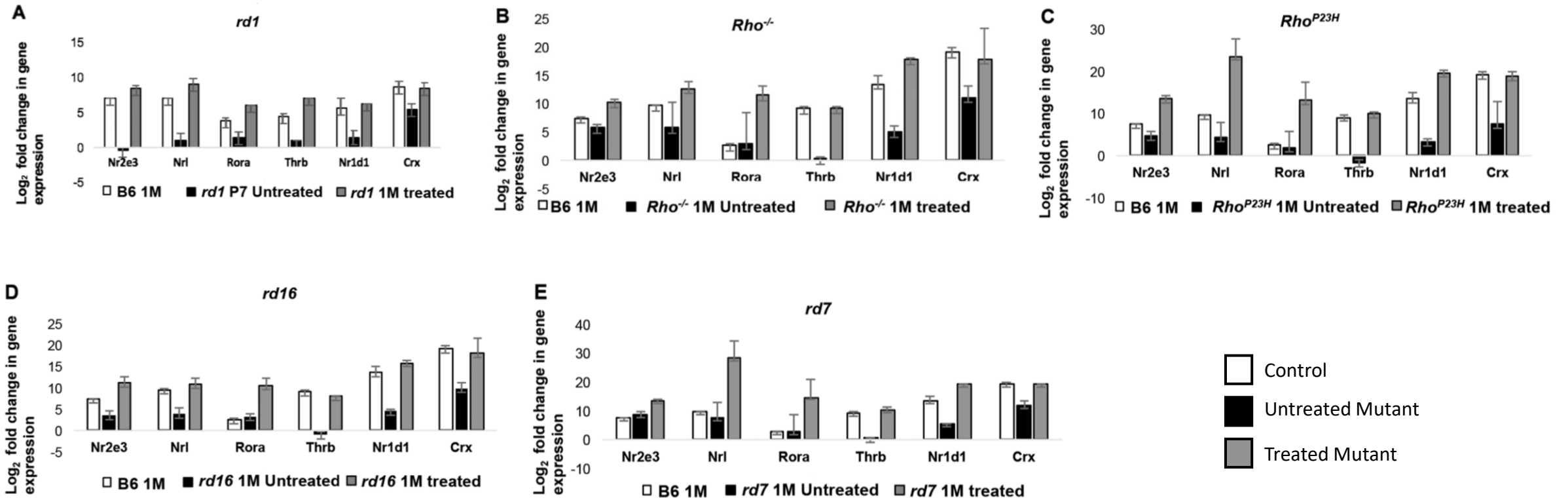
- **13,120 (65%)** of all human proteins expressed in retina
- **785** genes are highly specialized in the retina
- More than **250** mutations have been identified
- **100+** associated with retinitis pigmentosa (RP)
- **1** gene therapy for a single mutation is approved (2017)

Traditional single therapy is not efficient for diseases with multiple mutations (i.e. RP)

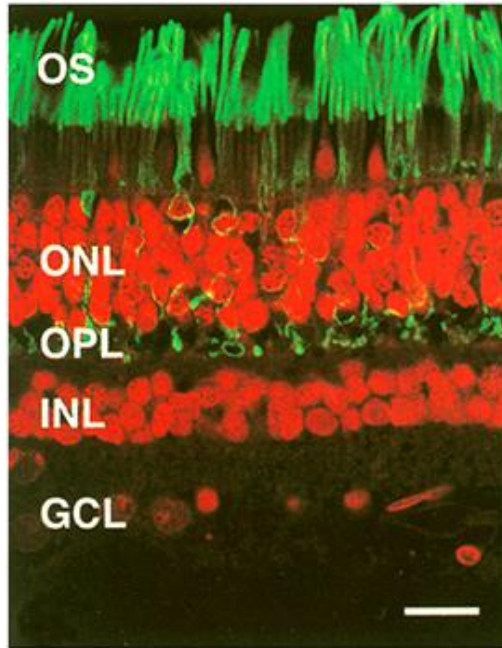
OCU400: *NR2E3* Restores Expression of Key Retinal Transcription Factors

NR2E3 expression results in a “molecular reset”

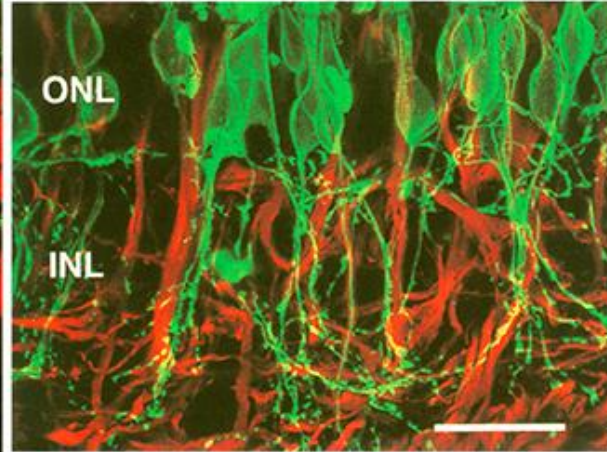
- Restoration of molecular and cellular homeostasis
- Recruitment of transcription factors



The Photoreceptor is Dependent on Cellular Homeostasis Regulated by Key Transcription Factors (TFs) such as *NR2E3* (OCU400)

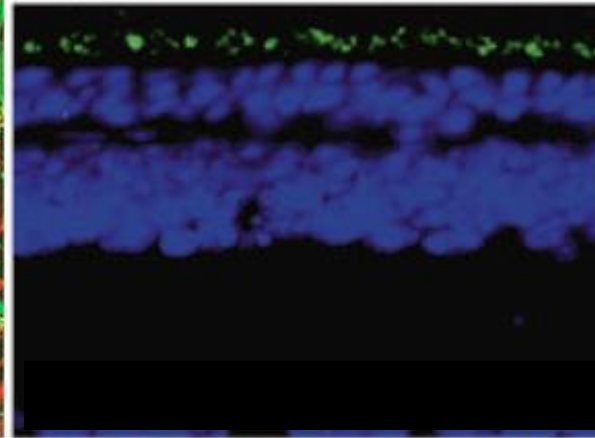


Normal Photoreceptor

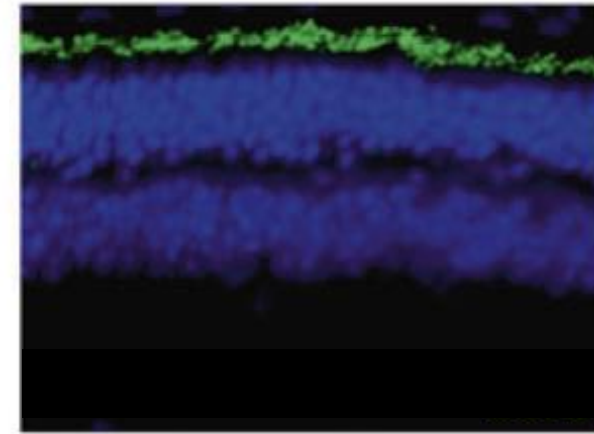


RP Impact on the Photoreceptor

Untreated – RP
(*Rho* mutation)



Treated –OCU400



Recovery after treatment with OCU400

- Immunolabeling of rod photoreceptors with anti-opsin (green) in normal human and in retinitis pigmentosa retinas¹
- Rhodopsin (RHO) immunolabeling shows restored expression and homeostasis in photoreceptors of treated animals²

¹ 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/>

² <https://www.nature.com/articles/s41434-024-00440-6>

OCU400 Modifier Gene Therapy – A Paradigm Shift in Gene Therapy

Prevalence of RP:

- Nearly 310,000 patients in the U.S., EU, & Canada
- ~1.6M patients globally

Currently approved treatment for RP:

- Luxturna[®] was FDA approved based on a mobility test and has achieved the following responder rate in Phase 3 (greater than or equal to 2 Lux levels):
 - 52.4% responder rate
- Based on the intent-to-treat (ITT) analysis of Ph 1/2, the OCU400 Phase 3 outcome is projected to be greater – across multiple mutations
 - 62.5% responder rate

OCU400 status/upcoming anticipated catalysts:

- Phase 3 clinical trial is on track to complete enrollment in 1H2025
- Clinical updates including Phase 3 recruitment for RP
- On track to meet 1H2026 Biologics License Application (BLA) and Marketing Authorization Application (MAA) filing targets



First Phase 3 gene therapy clinical trial to receive broad RP indication from **FDA**

Mobility Test (LDNA*) is Primary Endpoint for Phase 3

- Primary endpoint (Efficacy): Mobility test has been used for an approved product
- Alignment with FDA to demonstrate clinical efficacy (Responder ≥ 2 Lux level improvement)
 - Validated for RP patients
- Phase 1/2 results: More than 60% of the ITT patients from the Phase 1/2 clinical trial (*RHO* and *NR2E3*) meet the responder criteria

Dosing status	Approved Product Phase 3 ¹	OCU400 Phase 1/2
Treated	11/21 (52.4%)	5/8 (62.5%)

- OCU400 Phase 3 provides > 95% power at 50% response rate (N = 150; 2:1 randomization)
- Target BLA/MAA filings 1H2026

OCU410: Addressing Shortcomings of Current Approaches

Prevalence of GA:

- ~2-3 million people in the U.S. & EU
- ~8M patients globally

Currently approved treatment for GA:

- Focused only on the complement system
- Requires frequent intravitreal injections (~6-12 doses per year)
- Structural effect only,
 - Limited effect of GA lesion growth rate
 - No improvement in visual acuity reported
- Safety Considerations
 - 12% of patients experienced nAMD when therapy is administered every month for two years (Syfovre®)

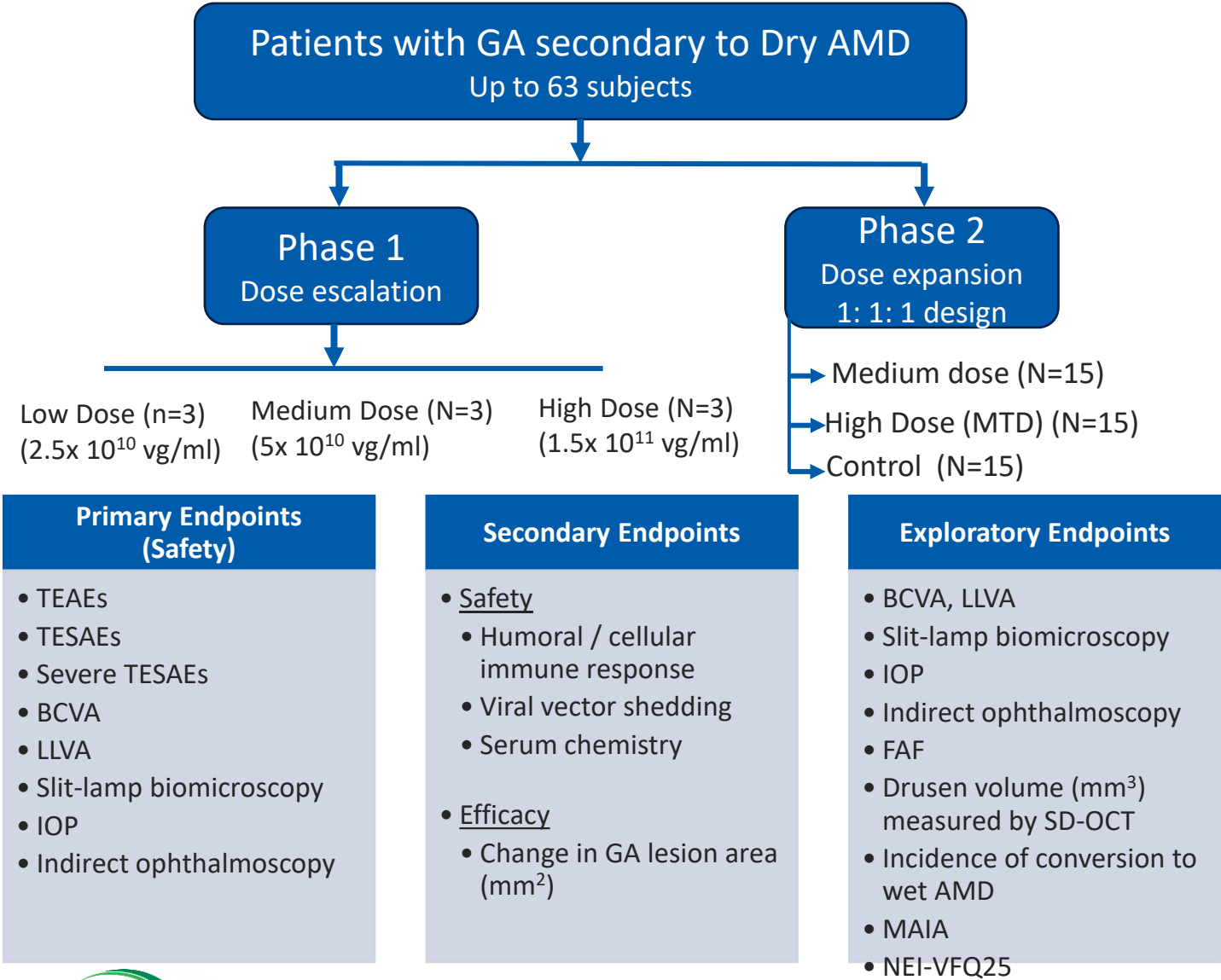
OCU410 status/upcoming anticipated catalysts

- Phase 2 trial is actively enrolling
 - Two treatment groups (medium & high dose) and a control group
- On track to complete Phase 1/2 dosing by early 2025
- 9- and 12-month data updates from Phase 1 clinical trial to be reported



There remains no approved treatment for GA in Europe

OCU410 Phase 1/2 Study : Design and Key Criteria



- Key Inclusion Criteria**
- Age ≥50 years
 - BCVA ≥21 letters ETDRS
 - GA lesion requirements:
 - Total GA area ≥2.0 mm² and ≤ 20.5 mm² ; if multifocal, at least one lesion must be ≥1.25 mm² (0.5 DA)
 - GA lesion within foveal and perifoveal region
 - Prior treatment with Izervay and Syforvire allowed after 3M washout period

- Key Exclusion Criteria**
- Previous treatment with gene or cell therapy
 - GA due to causes other than AMD
 - History or current evidence of wet AMD
 - >6 diopters of myopia, axial length >26 mm, inability to fixate, uncontrolled glaucoma, advanced cataract, or other pathologies that render subject unsuitable
 - History of wet AMD in the study eye

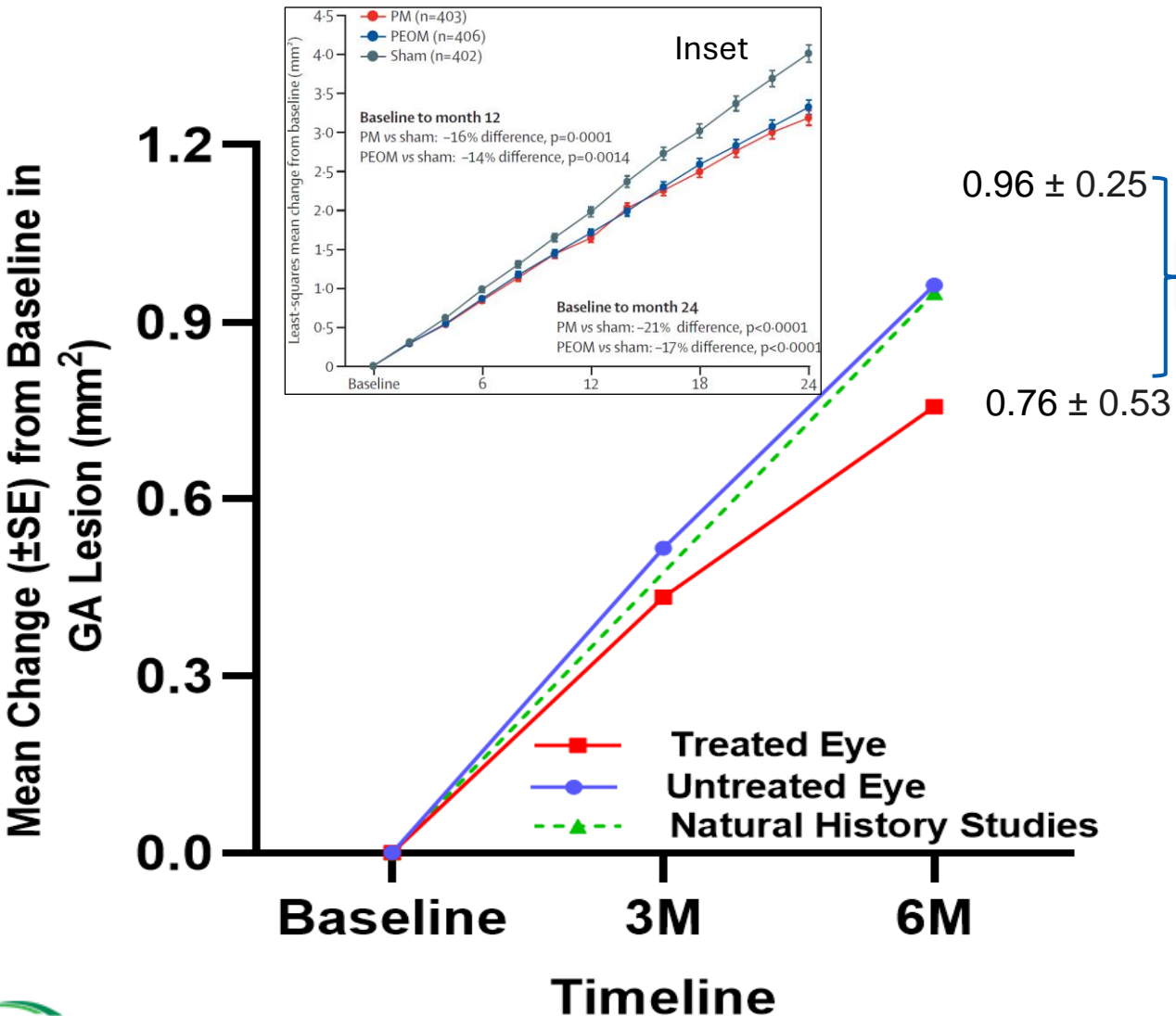
CNV in fellow eye is not exclusionary



☐ Cohorts used for current analysis

Slower GA Lesion Growth Comparable to Pegcetacoplan Treatment

3M (low-dose, N=3 & med-dose, N=2); 6M (low-dose, N=3)

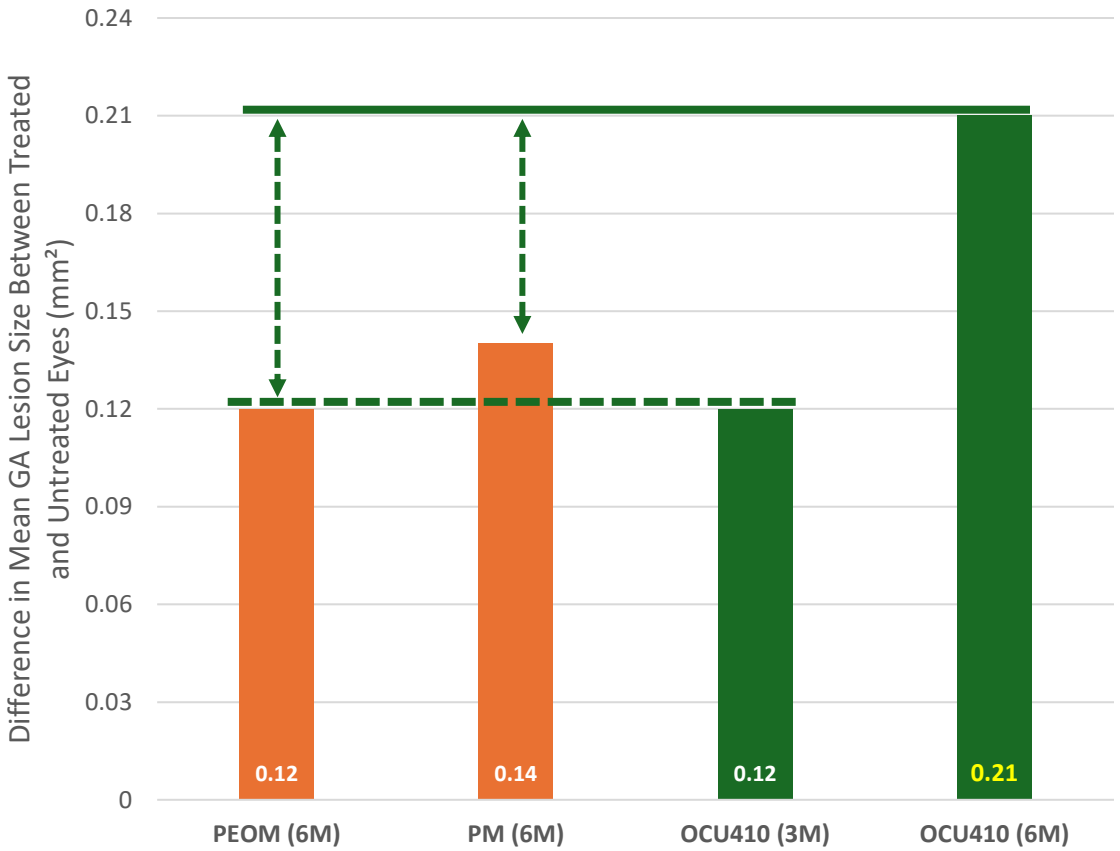


- 21.4% slower GA lesion growth in treated eye versus untreated fellow eye at 6M
- Single sub-retinal injection of OCU410 is similar to 6M compared to PM and PEOM treatment
- The slope of the untreated eye overlaps with natural history of the disease



Inset showing OAKS and DERBY Combined data from Heier JS et al., *Lancet*, 2023; GA: geographic atrophy; SEM: standard error of the mean, PM=pegcetacoplan monthly; PEOM=pegcetacoplan every other month

OCU410 Treatment Preserves Retinal Tissue in GA Lesions



OCU410 low dose treatment preserves more retinal tissue around the GA lesions of treated eyes at 6M compared to PM and PEOM

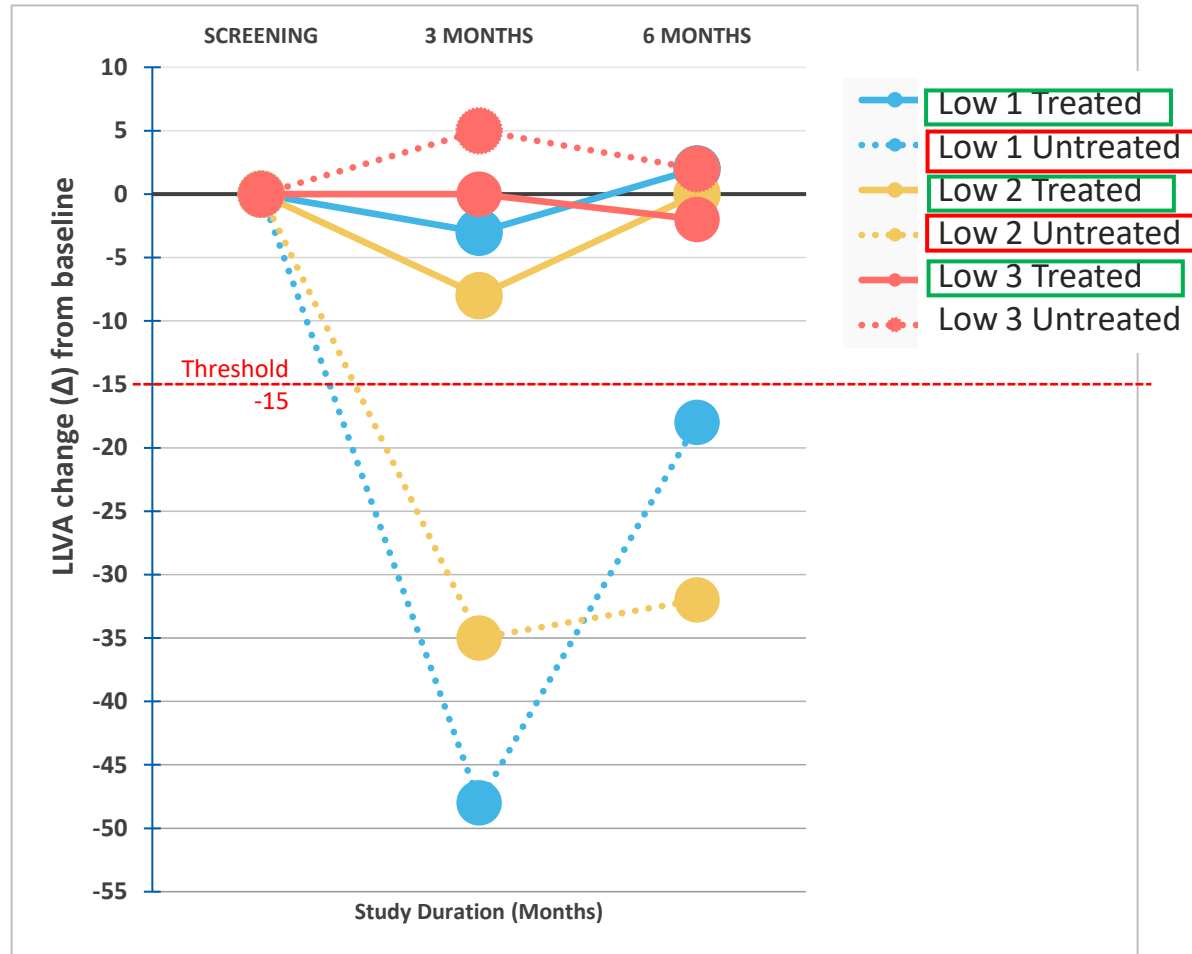
Published data OAKS and DERBY used Sham controls in lieu of untreated.

Includes both foveal and subfoveal GA: geographic atrophy; PM=pegcetacoplan monthly; PEOM=pegcetacoplan every other month
Preservation = Amount to retinal tissue protected from atrophy due to treatment



Preservation of Visual Function (LLVA) in Treated vs. Untreated Eyes

Cohort 1 (Low Dose)



- Stabilization or Improvement
- Decrease from Baseline/Screening

3/3 (100%) of the treated eyes showed stabilization of visual function demonstrating treatment benefit¹

Conclusions

- OCU410 demonstrated a favorable safety and tolerability profile
 - No SAEs or AEs deemed related to study drug or study procedure were reported including ischemic optic neuropathy, vasculitis, endophthalmitis, and choroidal neovascularization
- OCU410 treated eyes showed a **21.4% decrease** in lesion growth versus untreated fellow eyes in subjects followed up to 6M
- All 3 (**100%**) low-dose treated subjects showed stabilization of visual function
- Single subretinal OCU410 treatment preserves more retinal tissue around GA lesions of treated eyes at 6M compared to published PM and PEOM anti-complement treatment supporting the OCU410 MOA to preserve RPE and neurosensory retina

PM=pegcetacoplan monthly; PEOM=pegcetacoplan every other month; MOA= mechanism of Action; Established in preclinical studies
Preservation = Amount of retinal tissue protected from atrophy due to treatment

OCU410ST: A Single-Injection Approach to Address Unmet Need

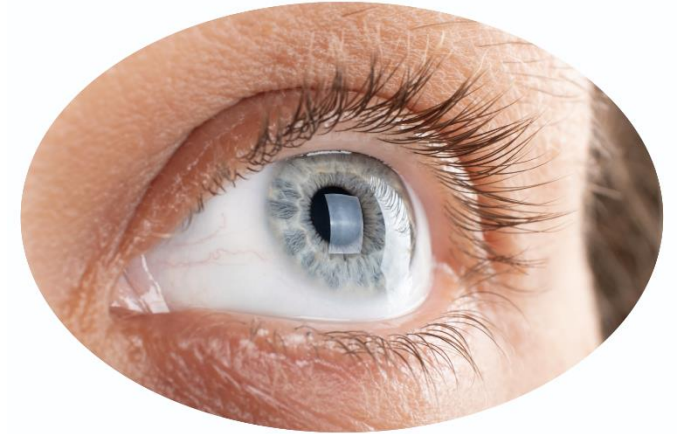
Prevalence of Stargardt disease:

- ~100,000 people in the U.S. and Europe

There is no FDA-approved treatment for Stargardt disease

OCU410ST status/upcoming anticipated catalysts:

- Data and Safety Monitoring Board (DSMB) approved enrollment for Phase 2 of the OCU410ST Phase 1/2 clinical trial
- 9- and 12-month data updates from Phase 1 clinical trial pending
- Ocugen plans to initiate a Phase 2 trial after alignment meeting with FDA in 2025



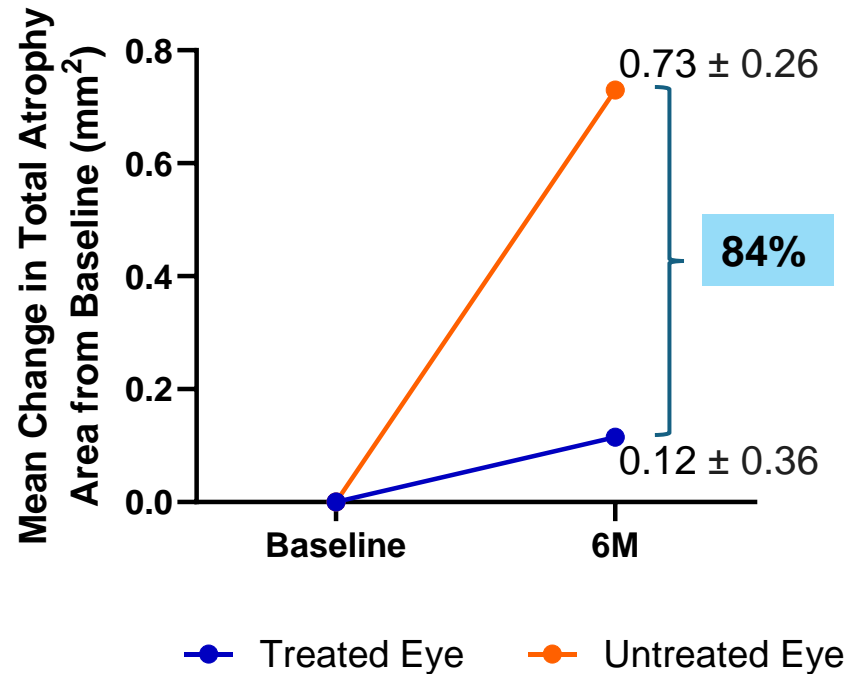
**Received ODD & OMPD
for ABCA4-Associated Retinopathies:
Stargardt, Retinitis Pigmentosa 19 &
Cone-rod Dystrophy 3**

Overall Safety – Phase 1 Trial

- OCU410ST demonstrated a favorable safety and tolerability profile
- No study drug or study procedure related SAEs (deemed related to study drug or study procedure) were reported including:
 - No development of exudation
 - No infectious endophthalmitis
 - No intraocular Inflammation
 - No anterior ischemic optic neuropathy (AION)
 - No vasculitis
- No adverse events of special interests (AESIs)

OCU410ST Ph1 Efficacy: Slower Atrophic Lesion Growth in Treated Eyes

OCU410ST, 6M (Low-dose, N=2, Med-dose, N=1)



84% slower atrophic lesion growth in treated eyes versus untreated fellow eyes at 6M

OCU410ST Stabilizes or Improves Retinal Structure and Visual Function

Parameters for Assessment	Low 1	Low 2	Low 3	Med 2	Med 3	Overall Measures
Atrophic lesion growth (mm ²) compared to untreated eyes	●	N.D	●	●	N.D	
Visual Function Improvement (BCVA)	-	-	●	●	●	3/5 (60%)
Total Retinal Thickness (Change from BL)	●	●	-	●	-	3/5 (60%)
Macular Volume (Change from BL)	●	●	-	●	●	4/5 (80%)

● Parameters showing improvement or preservation in the treated eye

- Structural Improvement
 - Atrophic lesions grew slower by 84% in treated eyes when compared to untreated eyes
 - 4/5 (80%) of treated eyes demonstrated preservation of macular volume
 - 3/5 (60) of treated eyes demonstrated preservation of retinal thickness
- Visual Function (BCVA)
 - 3/5 (60%) treated eyes demonstrated stabilization or improvement in visual function

Stabilization = ± 4 letters from Baseline; Improvement: ≥ 5 Letters from Baseline; Preservation= Visual acuity saved due to treatment; Macular Volume (mm³) is calculated over entire ETDRS Grid using an algorithm ; Central retinal thickness (μm , ILM to anterior border of RPE) measured at the umbo
 N.D= cannot determine atrophy on spectral-domain OCT at baseline

Conclusions

- Demonstrated a favorable safety and durability profile. High dose is the Maximum Tolerated Dose.
- Demonstrated stabilization or improvement in visual function and retinal structure outcomes
- Aligning with FDA for Phase 2 clinical trial design

OCU200

Novel biologic for treating Diabetic Macular Edema (DME), Diabetic Retinopathy (DR) and Wet Age-Related Macular Degeneration (Wet AMD)

OCU200: Phase 1 Clinical Trial Targeting Diabetic Macular Edema (DME)

Novel biologics candidate for sight-threatening conditions

- A recombinant fusion protein of transferrin and tumstatin
- Potential to address diabetic macular edema (DME), diabetic retinopathy (DR), and wet AMD

High prevalence of DME, DR and wet AMD patients

- DME: 21M worldwide
- DR: 162M worldwide
- Wet AMD: 30M worldwide

Limited treatment options available for DME

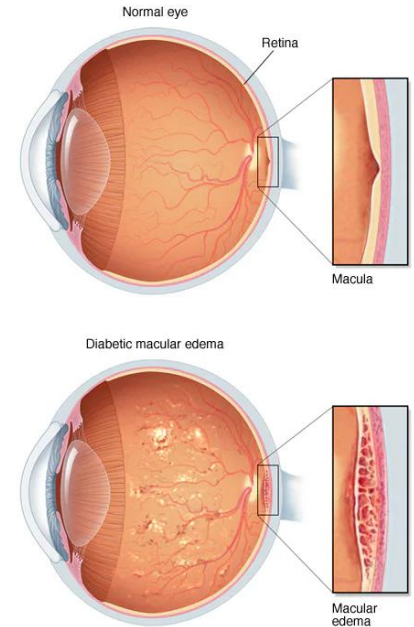
- Current therapies target only one pathway, either angiogenesis or inflammation
- Approximately 30 to 40% of DME patients are refractive to current anti-VEGF therapies

OCU200 potentially addresses shortcomings of current treatments

- OCU200 targets the underlying disease mechanisms through the integrin pathway and holds promise to provide benefit to all DME patients, including non-responders to currently approved therapy

OCU200 Phase 1 study design:

- Initially targeting DME
- Multicenter, open-label, dose-ranging study
 - 3 cohorts in dose-escalation portion assessing safety
 - Fourth cohort: Combination of OCU200 (MTD) with anti-VEGF following sequential intravitreal administration



DME: bulges protrude from the blood vessels, leading to leakage of fluid and blood into the retina; leakage results in swelling (or “edema”), promoting vision loss

Near-Term Targeted Milestones

- Complete OCU400 Phase 3 recruitment and continue to monitor patients to ensure BLA/MAA filings are complete in 1H2026
- Complete OCU410 Phase 2 recruitment and continue to provide clinical trial updates—Phase 1 patients up to 1 year
- Continue to provide OCU410ST clinical trial updates—Phase 1 patients up to 1 year—and initiate Phase 2 after alignment with FDA in 2025
- Initiate OCU200 Phase 1 clinical trial in 4Q 2024

Ocugen™ Vision

Fully integrated, patient-centric biotech company focused on vaccines in support of public health and gene and cell therapies targeting unmet medical needs through **Courageous Innovation**

