



Courageous Innovation

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

Clinical Showcase  
November 12, 2024



## Forward-Looking Statements

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*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.*



# Agenda

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Welcome

Tiffany Hamilton, Head of Corporate Communications

Opening Remarks

Dr. Shankar Musunuri, Chairman, CEO & Co-founder, Ocugen

The Potential of Modifier Gene Therapy

Dr. Arun Upadhyay, Chief Scientific Officer and Head of R&D

Clinical Trial Update

Dr. Huma Qamar, Chief Medical Officer

Investigator Presentations

Dr. Benjamin Bakall, Director of Clinical Research at Associated Retina Consultants (ARC) & Clinical Assistant Professor at University of Arizona, College of Medicine – Phoenix

Dr. Lejla Vajzovic, Professor of Ophthalmology, Pediatrics, & Biomedical Engineering with Tenure at Duke Eye Center and Duke University School of Medicine

Dr. Syed M. Shah, Vitreoretinal Diseases and Surgery/Emplify Health, Vice Chair For Research & Digital Health, Gundersen Health – La Crosse, Wisconsin

Patient Panel

Commercial Update

Mike Shine, SVP, Commercial

Q& A

Ocugen Management Team

Conclusion

Dr. Shankar Musunuri, Chairman, CEO & Co-founder, Ocugen



Placeholder for video



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

### Company Overview

Founded 2013  
Headquarters Malvern, PA  
Manufacturing Facility Malvern, PA



### Our Values

- Respect
- Integrity
- Teamwork
- Accountability



India Business Center Hyderabad, India  
Ticker Symbol OCGN  
Market Cap \$300 Million







We're Driving Science in New Directions and Breaking  
New Ground Through Courageous Innovation

**First-In-Class Modifier Gene Therapy:**

- **OCU400 Phase 3 liMeliGhT Clinical Trial**
  - Retinitis pigmentosa
- **OCU410 Phase 2 ArMaDa Clinical Trial**
  - Geographic atrophy
- **OCU410ST Phase 1/2 GARDian Clinical Trial**
  - Stargardt disease

# The Potential of Modifier Gene Therapy

Arun Upadhyay, PhD  
Chief Scientific Officer and Head of R&D

# Retinal Diseases: Leading Cause of Blindness with Significant Unmet Medical Needs

## Key Retinal Diseases Leading to Blindness

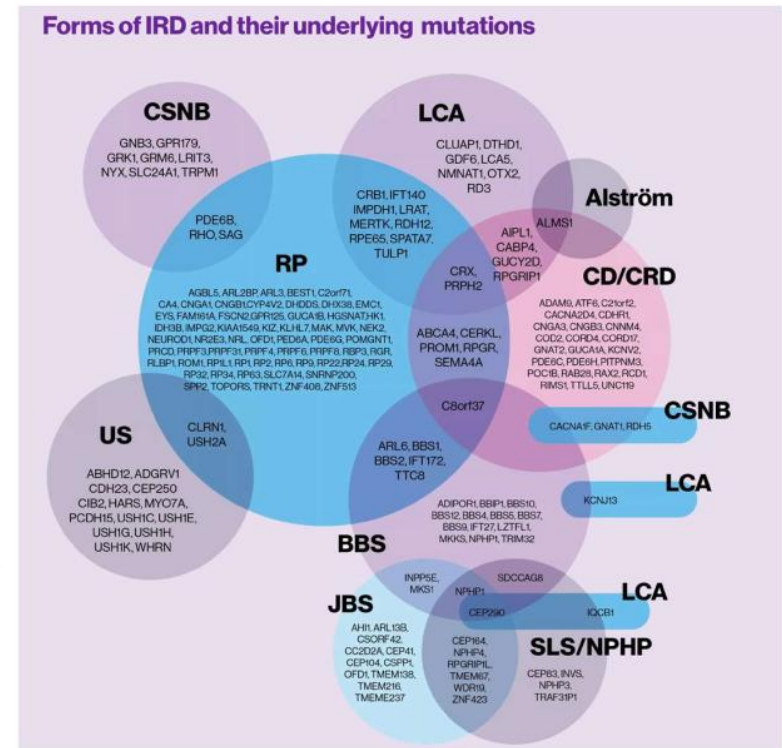
- Inherited retinal diseases (e.g. RP, LCA, Stargardt)
- Multifactorial (Dry AMD; geographic atrophy)

## Prevalence

- RP- U.S. and EU: 300,000; Global: 1.6M
- AMD- U.S. and EU: 19M (GA: 2-3M); Global: 266M
- Stargardt- U.S.: ~44,000

## Significant Unmet Medical Need

- No treatment available for most IRD patients
- Only one product approved for RP, *targeting <2%* patient population
- **No treatment available for Stargardt disease**
- For AMD (GA)- two recently approved products in U.S.
  - *Still a significant unmet medical need, including EU*
  - *Limited treatment benefits; safety and patient compliance concerns*



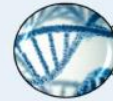
<https://www.discoverird.com/hcp/about-ird>



## Barriers to Effective Treatment in Genetically Diverse & Multifactorial Diseases



High genetic heterogeneity in RP, LCA or STGD limits gene-specific therapy



Gene specific strategy not be applicable for multifactorial/multigenic diseases



Significant cost and effort required to develop and manufacture individual gene therapies



Focusing on a single factor in multifactorial diseases (AMD) falls short of delivering optimal treatment outcomes



Gene Agnostic and  
Multifactorial Therapeutic  
Intervention

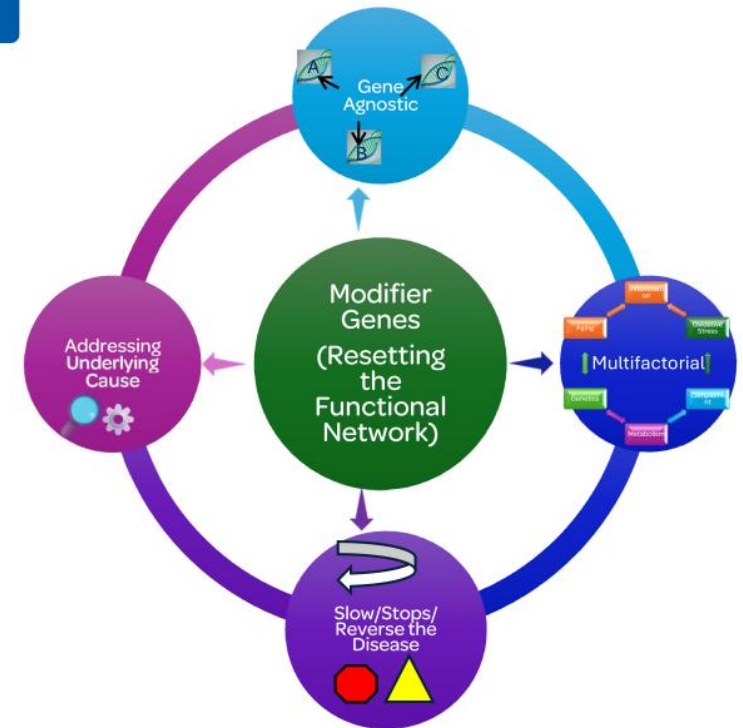
## Modifier Gene Therapy: A Revolutionary Approach to Treat Disease at Roots

### Modifier Genes

Main control system for how cells in the retina survive and function

A disease-modifying treatment for inherited and aging related retinal diseases

- Gene-Agnostic – Targets multiple genotypes and broader patient population
- Multifactorial – Targets multiple pathways linked to a disease
- Alters the course of a disease by addressing its underlying cause
- Decreases need for development of >100 products based on gene augmentation or gene editing



## Modifier Gene Therapy (OCU400): Multiple IRDs with ONE Powerful Solution

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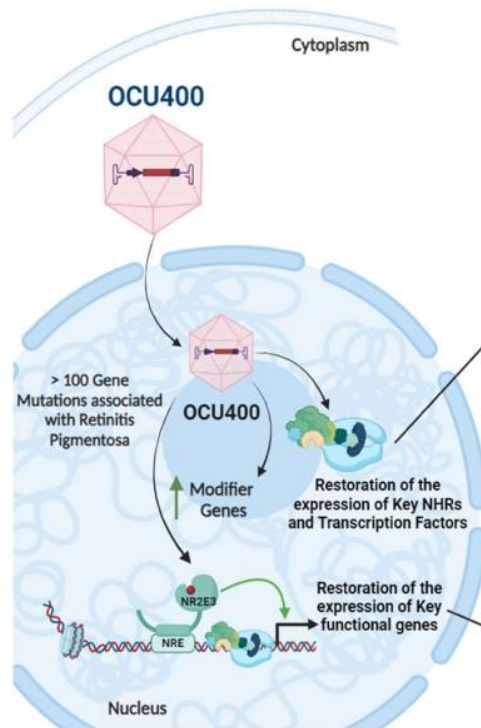
### Therapeutic Development Approaches for IRDs

- **Gene Augmentation**
  - Delivers normal copy of affected gene
  - Limited to treat one gene; monogenic conditions; and recessive gene
- **Gene Editing**
  - Corrects mutation in affected gene
  - Limited to treat one mutation; monogenic conditions
- **Optogenetics**
  - Limited to very advanced stage of the disease (legally blind)
  - Limited efficacy and durability; long-term immune reaction concern because of expression of foreign protein (light capturing protein)
  - Cannot prevent retina from degeneration
- **Cell therapy**
  - Requires early-stage intervention
  - Integration within retina is biggest challenge; immune reactions

### OCU400: Modifier Gene Therapy

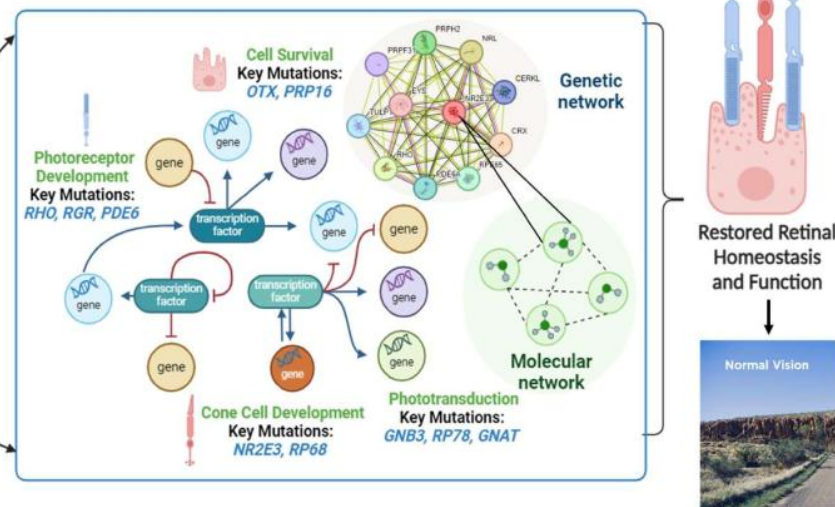
- Gene Agnostic
- Broader Patient Population
- Potential for stabilization/reversal of the diseases
- Generally well-tolerated and durable

## OCU400: A Novel Modifier Gene Therapy Targeting RP and LCA



OCU400 is designed to address shortcomings of current gene therapy approaches

- Broad-spectrum, gene-agnostic approach to genetically diverse IRDs
- Potential curative therapy with a *single* sub-retinal injection

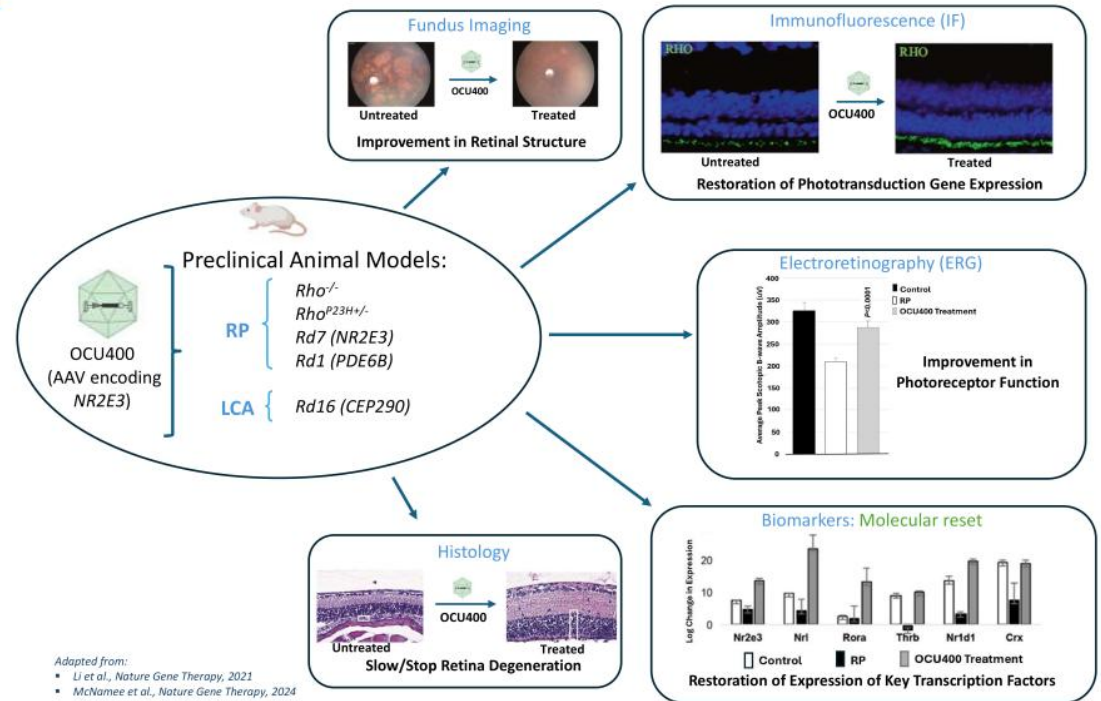





## Animal Proof of Concept – Gene Mutation Agnostic Mechanism

### Key Findings – 5 Animal Models of RP

- Provides structural and functional preservation of degenerating retina
- Resets key NHRs and transcription factors to physiological level
- Improves photoreceptor survival and visual functions
- No off-target effects or excess expression





OCU410/OCU410ST (*RORA*) for the  
Treatment for Geographic Atrophy and  
Stargardt Diseases

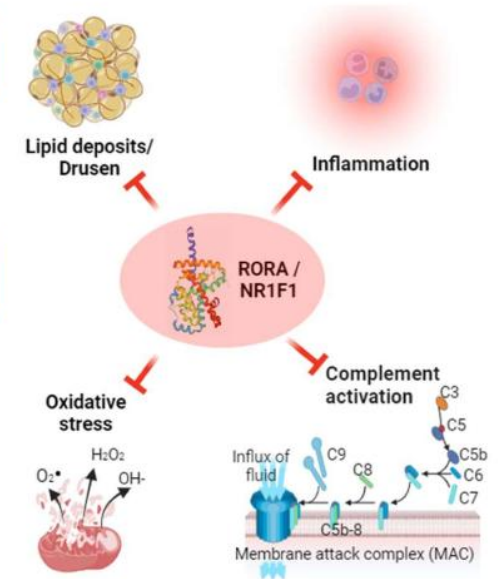
## Regulation of Multiple Pathways by *RORA*

### *RORA*: a nuclear hormone receptor (NHR) and important modulator

- Plays a role in development and function of rod and cone photoreceptor cells
- Regulates genes and pathways involved in onset and progression of AMD

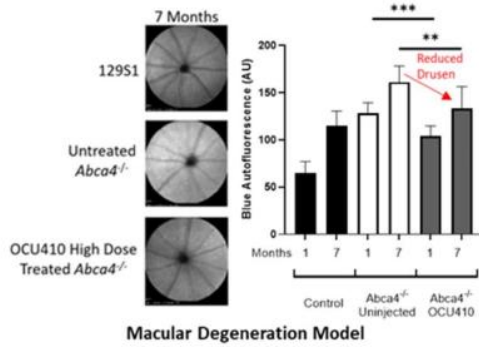
### *RORA* regulates genes involved in:

- Photoreceptor development like *OPN1SW*, *OPN1MW*, and *ARR3*
- Lipid metabolism such as apolipoproteins (*APOA1*, *APOA5*, *APOC3*) and steroids
- Regulation of inflammation
- Hypoxia signaling like *HIF-1 $\alpha$*  and oxidative stress response machinery
- Regulating expression of cellular *CD59* protein, which suppresses the complement complex's assembly and prevents cell damage

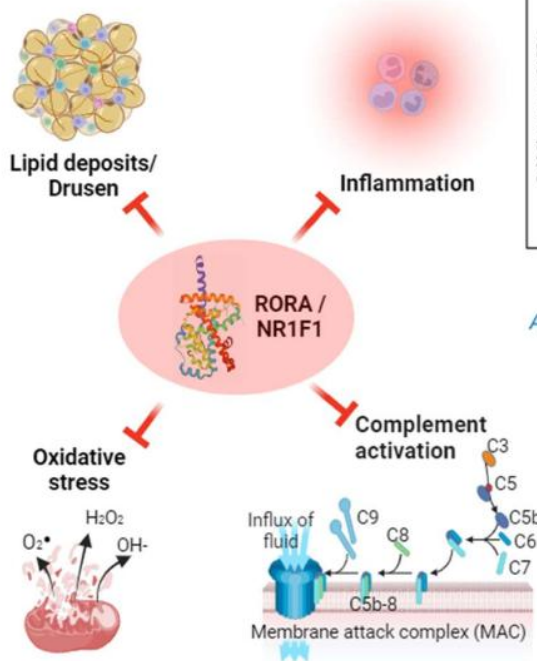
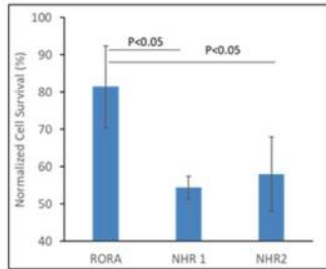


# OCU410 (RORA): Proof of Concept Summary

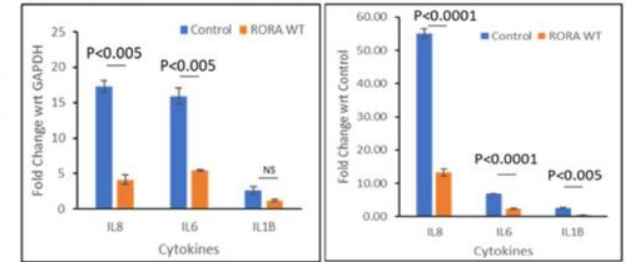
*Anti-drusen activity and improves retinal function*



*Anti-oxidative: Improves ARPE19 cell survival*



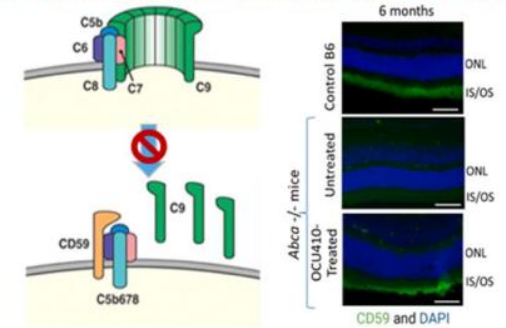
*Anti-inflammatory: Suppresses inflammation in HMC3 and ARPE19 cells*



**HMC3**

**ARPE19**

*Anti-complement: Increased anti-complement (CD59) protein*





## OCU410 (RORA): A Single-Injection Approach to Addressing Unmet Needs in dAMD BEYOND the Complement System

- Phase 2 OCU410 ArMaDa study for GA currently underway
  - Limited options for dAMD, presenting significant unmet medical need
    - U.S. and EU: nearly 19M (GA: 2-3M)<sup>1</sup>
    - Worldwide: 266M<sup>2</sup>
  - Distinct 4-way MOA:  
Addresses multiple regulator pathways involved with the disease including:
    - Lipid Metabolism
    - Regulation of Inflammation
    - Oxidative Stress
    - Membrane Attack Complex (Complement)
  - Optimal delivery and durability:
    - A single subretinal injection designed to eliminate patient compliance concerns and the treatment burden associated with multiple injections
  - Improved retinal function:
    - Improved photoreceptor function in OCU410 treated eyes with all doses\*
- Advancement from recently approved therapies for GA: Potential to address limitations of recently approved therapies for GA focused only on the complement system, including:
    - Patient Compliance
      - Frequent intravitreal injections (~6-12 doses per year)
    - Observed Structural Impact
      - Limited effect of GA lesion growth rate
    - Safety Considerations
      - 12% of patients experienced nAMD when therapy is administered every month for two years (Syfovre®)

Potential for a one-time therapy for life with a single sub-retinal injection to address the unmet needs and treatment burden in patients with dAMD



MOA: Mechanism of action | nAMD: Neovascular age-related macular degeneration

<sup>1</sup>DB Rein et al., *JAMA Ophthalmol*, 2022. doi: 10.1001/jamaophthalmol.2022.4401

<sup>2</sup>CJ Thomas et al., *Med Clin North Am*, 2021. doi: 10.1016/j.mcna.2021.01.003 (2021).

\*As demonstrated in peak scotopic B-wave amplitudes, N≥5 biological replicates

## OCU410: The Opportunity for a One-Time Procedure Designed to Address All Mechanistic Factors Driving Geographic Atrophy BEYOND the Complement System

	Anti-Complement	Anti-Inflammatory	Anti-Drusen Activity	Anti-Oxidative
<b>OCU410</b>	✓ <i>Anti-complement: Increased anti-complement (Cd59) protein</i>	✓ <i>Anti-inflammatory: Suppresses inflammation in HMC3 cells</i>	✓ <i>Anti-drusen activity: Improves retinal function</i>	✓ <i>Anti-oxidative: Improves ARPE19 cells survival</i>
<i>Marketed Products:</i>	✓ Syfovre – C3 Inhibitor Izervay – C5 Inhibitor			
<i>Select Late Stage Assets in Development:</i>	✓ C1q inhibitor: ANNEXON			✓ <i>ORAL Visual Cycle Modulators</i> Belite Bio (Tinarebant) Alkeus (Gildeuretino)



## OCU410ST: Received ODD for *ABCA4*-Associated Retinopathies: Stargardt, Retinitis Pigmentosa 19 & Cone-rod Dystrophy 3

### *ABCA4*-associated retinopathies—Genetic Rare Disease

- *ABCA4* gene produces an ATP-binding cassette (ABC) superfamily transmembrane protein involved in clearance of all-trans-retinal aldehyde, a byproduct of the retinoid cycle, from photoreceptor cells
- Mutation in *ABCA4* gene results in Stargardt disease. Different *ABCA4* alleles have been identified to cause other retinopathies such as cone-rod dystrophy type 3 (CORD 3), retinitis pigmentosa type 19 (RP 19)

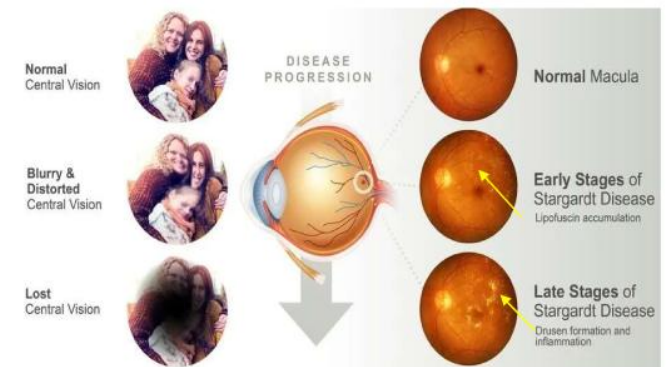
### No treatment options exist

- U.S.: 44,000 patients\*

### Modifier gene therapy platform addresses shortcomings of current approaches

- AAV delivery platform delivers *RORA* (RAR Related Orphan Receptor A)
- Broad-spectrum, gene-agnostic approach
- Potential one-time, curative therapy with a single sub-retinal injection

Completed dosing patients in Phase 1 of Phase 1/2 GARDian clinical trial and the Data and Safety Monitoring Board approved enrollment in Phase 2



\*P Kohli et al., StatPearls, 2024. <https://www.ncbi.nlm.nih.gov/books/NBK587351/>

# Clinical Trial Updates

Huma Qamar, MD, MPH  
Chief Medical Officer



## OCU400 Modifier Gene Therapy – A Paradigm Shift in Gene Therapy

- ✓ Health Canada approved initiation of OCU400 Phase 3 liMeliGhT trial for the treatment of retinitis pigmentosa (RP)
- ✓ Received FDA approval of expanded access program (EAP) for adult patients with RP

*Upcoming anticipated catalysts:*

- *Clinical updates including Phase 3 recruitment for RP*
- *Phase 3 clinical trial is on track to complete enrollment in 1H2025*
  - *New data updates from Phase 1/2 RP & LCA*



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

*OCU400 for the treatment of RP remains on track to meet 1H 2026  
Biologics License Application (BLA) and Marketing Authorization  
Application (MAA) filing targets.*

## OCU410: A Single-Injection Approach to Address Unmet Need

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✓ Dosing is underway in the OCU410 Phase 2 ArMaDa clinical trial, following the completion of Phase 1 low, medium, and high dose cohorts, which involved nine patients with geographic atrophy (GA)

- *The Phase 2 trial is actively recruiting a larger patient group randomized into either of two treatment groups (medium or high dose) or control untreated group*
- *Plan to complete dosing by 2H 2025*

*Upcoming anticipated catalyst:  
Preliminary safety and efficacy update from ongoing  
Phase 1/2 clinical trial*



Dry AMD affects nearly 19 million people in the U.S. & EU

GA affects ~2-3 million people in the U.S. & EU – a significant market opportunity

*OCU410 is positioned to transform the landscape of GA with its potential one-time therapy with a single sub-retinal injection—compared to other treatment options that require approximately 6-12 intravitreal injections annually.*

## OCU410ST: Modifier Gene Therapy Addressing Shortcomings of Current Approaches

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- ✓ Data and Safety Monitoring Board (DSMB) approved enrollment for the Phase 2 of the OCU410ST Phase 1/2 clinical trial

*Upcoming anticipated catalysts:*

- *Preliminary safety and efficacy update from ongoing Phase 1/2 clinical trial*
- *Seeking alignment with FDA for Phase 2 initiation*



Stargardt disease affects  
~100,000 people in the U.S. and  
Europe

*The safety and tolerability profile of OCU410ST remains encouraging as clinical development progresses and continues to bring hope to patients who have no FDA-approved treatment available.*

## Ocular Programs : Key Highlights

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### OCU400 for Retinitis Pigmentosa

- Excellent trending efficacy and favorable safety profile in 18 RP subjects, published results
- Benefits early, middle to advanced RP
- Only Phase 3 product for broad RP indication
- U.S. and Health Canada Phase 3 trial approval
- FDA-approved expanded access program
- On track for 1H 2026 BLA and MAA filing

### OCU400 for Leber Congenital Amaurosis

- Enrollment complete
- Treated eyes showed:
  - Stabilization or preservation (67%) in visual function (BCVA) in peds (9M) and adults (12M)
  - Stabilization or improvement (100%) in overall cone response (FST) in peds (9M) and adults (12M)
  - Improved QoL (NEI VFQ-25) in all adult subjects (9M, 12M)





## Ocular Programs : Key Highlights

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### OCU410 for Geographic Atrophy

- Phase 1 complete and Phase 2 enrollment on track
- Excellent safety profile with single sub-retinal injection. Reduces treatment burden and compliance issues.
- Slows GA lesion growth in treated eye vs. untreated eye at 6M
- Low-dose demonstrates treatment benefit by stabilization of visual function (LLVA) at 6M

### OCU410ST for Stargardt

- No current therapies for Stargardt
- Phase 1 dosing complete. Excellent safety profile.
- Aligning with FDA for Phase 2
- Treated eyes show 84% slower lesion growth at 6M compared to untreated eyes
- Up to 80% preservation of retinal structure at 6M and visual function (60% BCVA) at 6M

# Treatment Outcomes from Novel Modifier Gene Therapy (OCU400) in LCA Subjects with *CEP290* mutation

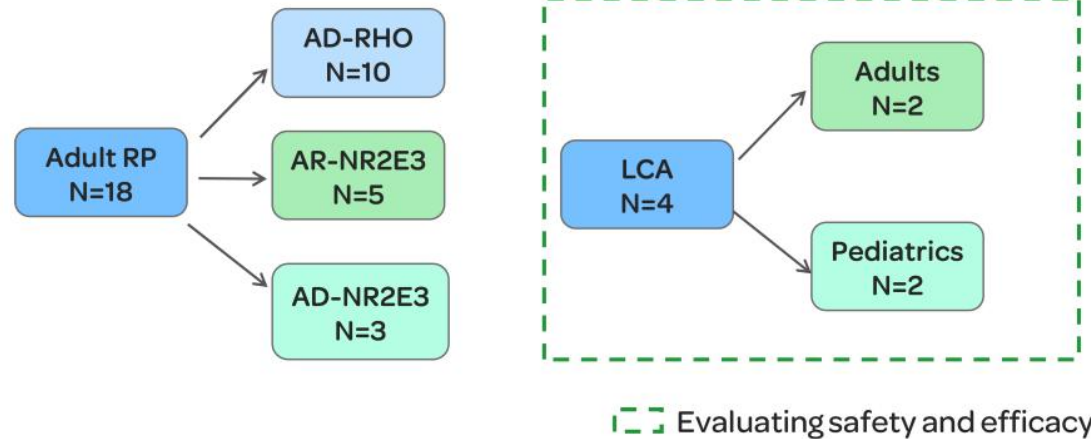
Benjamin Bakall, MD, Ph.D.  
Director of Clinical Research at Associated Retina Consultants (ARC) &  
Clinical Assistant Professor at University of Arizona,  
College of Medicine – Phoenix



NCT05203939

## OCU400 Clinical Program (Phase 1/2 Study—Completed Enrollment)

- OCU400 demonstrated an excellent safety and tolerability profile in Phase 1/2 retinitis pigmentosa patients with *RHO* and *NR2E3* mutations
- Evaluating safety and efficacy of OCU400 in Leber Congenital Amaurosis with mutation(s) in *CEP290* gene



AR-NR2E3= Autosomal Recessive NR2E3; AD-NR2E3= Autosomal dominant NR2E3; AD-RHO= Autosomal dominant RHO  
LCA=Leber Congenital Amaurosis

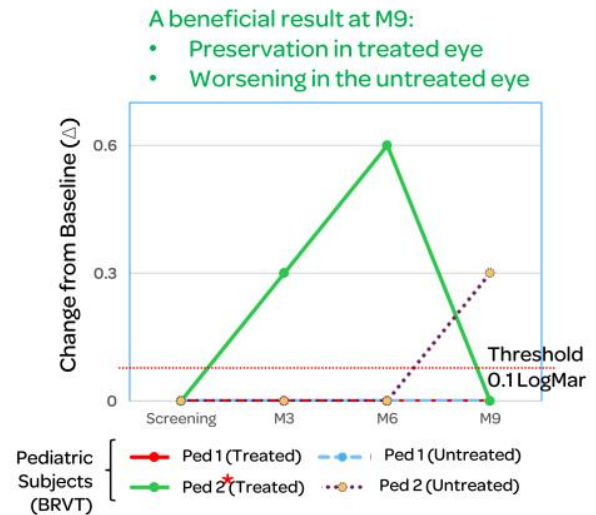
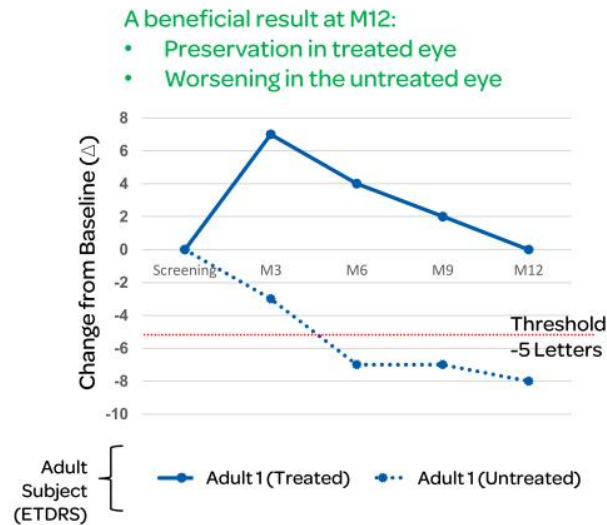
## LCA Open Label Study

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- OCU400 is novel Modifier Gene Therapy – Strong Preclinical Data
- A single subretinal delivery of OCU400 (Medium Dose,  $3.33 \times 10^{10}$  vg/mL) in LCA subjects to overexpress *NR2E3*
- Safety outcomes evaluated in all LCA subjects
- Month 9 efficacy is presented for all subjects
  - Additional Month 12 efficacy data available for 1 of 4 subjects (adult)
- Efficacy outcomes presented (Change from Baseline in 1 Adult and 2 Pediatric subjects):
  - Best Corrected Visual Acuity (BCVA)
  - NEI-VFQ25 Quality of Life/Patient Reported Outcome
  - Full-field stimulus threshold (FST)
  - Mobility Test



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



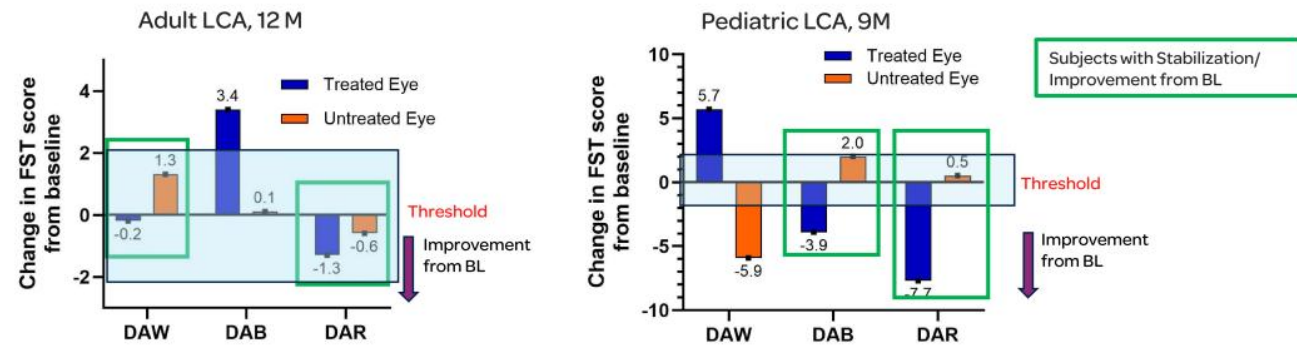
2/3 (67%) of LCA treated eyes showed stabilization or preservation<sup>1</sup> of visual function by BCVA



<sup>1</sup>Stabilization of BCVA when compared to baseline; Threshold = Decrease of 5 letters when compared to baseline  
ETDRS= Early Treatment of Diabetic Retinopathy Study; BRVT= The Berkeley Rudimentary Vision Test

\* No change was observed in the treated and untreated eye at M9, 1 Adult LCA subject not evaluable for efficacy

## Retinal Sensitivity Assessments with FST Demonstrate Improved Cone Response by Dark-Adapted Light Testing



2/2 (100%) subjects showed stabilization or improvement in overall cone response in treated eyes compared to untreated eyes



Stabilization =  $\pm 2$ dB from Baseline, marked as Threshold band; Improvement =  $\geq 3$ dB from Baseline  
 FST = Full-field stimulus test; DAW = Dark-adapted white light; DAB = Dark-adapted blue light; DAR = Dark-adapted red light

## Conclusions

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- OCU400 is generally safe and well tolerated in LCA subjects with *CEP290* mutation
  - No serious adverse events (SAEs) deemed related to study drug reported
- One adult LCA subject showed:
  - 1 Lux level improvement in the Mobility Test (from 130 lux at BL to 50 lux at 12M)
  - Stabilization in visual acuity (BCVA) at 12M
  - Improvement in overall cone response at 12M
- 2/3 (67%) LCA subjects showed stabilization or preservation of visual function (BCVA) in treated eyes
- 2/2 (100%) LCA subjects (1 Adult, 1 Pediatric) demonstrated improvement or stabilization in overall cone response in treated eyes in full-field stimulus test (FST)
- 2/2 (100%) adult LCA subjects showed overall composite score (3-4 overall score) improvement in NEI VFQ-25

# Treatment Outcomes from Novel Modifier Gene Therapy (OCU410) from Phase 1/2 Study in GA Subjects secondary to Dry AMD

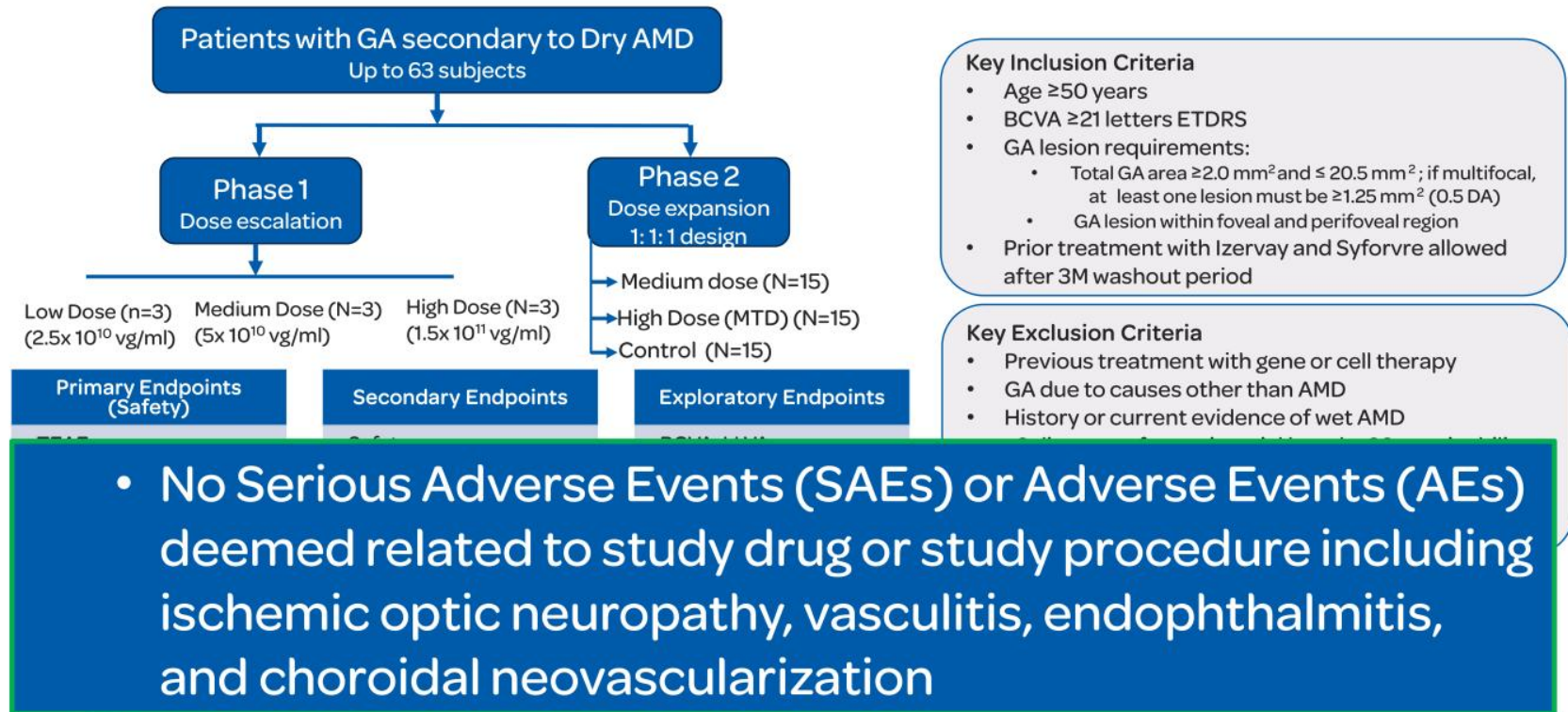
Syed M. Shah, MD, FACS  
Vitreoretinal Diseases and Surgery/Emplify Health, Vice  
Chair For Research  
& Digital Health, Gundersen Health – La Crosse, Wisconsin



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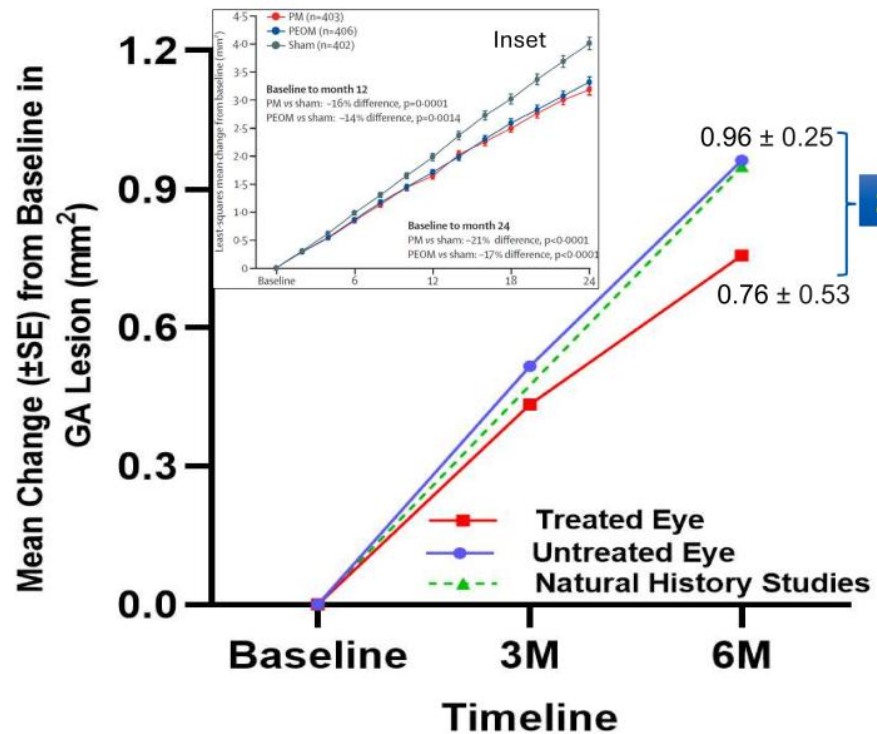


## OCU410 Phase 1/2 Study : Design and Key Criteria



## Slower GA Lesion Growth in Treated Eyes Comparable to Pegcetacoplan Treatment

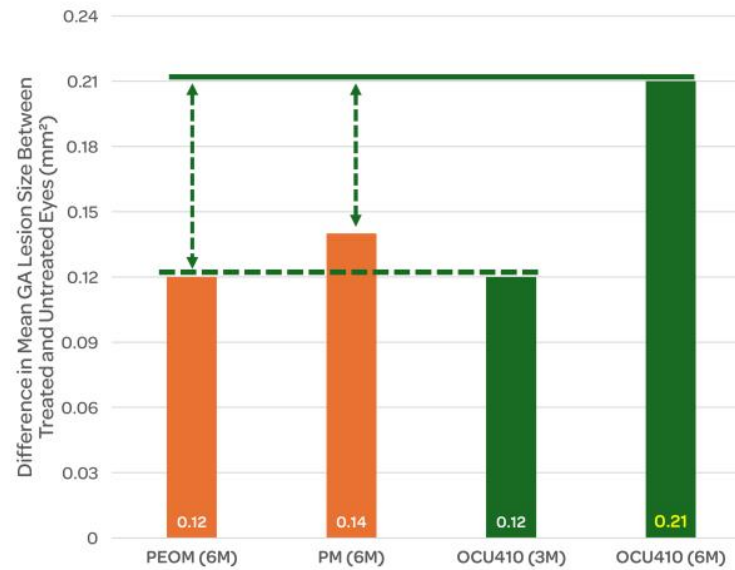
3M (low-dose, N=3 & med-dose, N=2); 6M (low-dose, N=3)



- 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

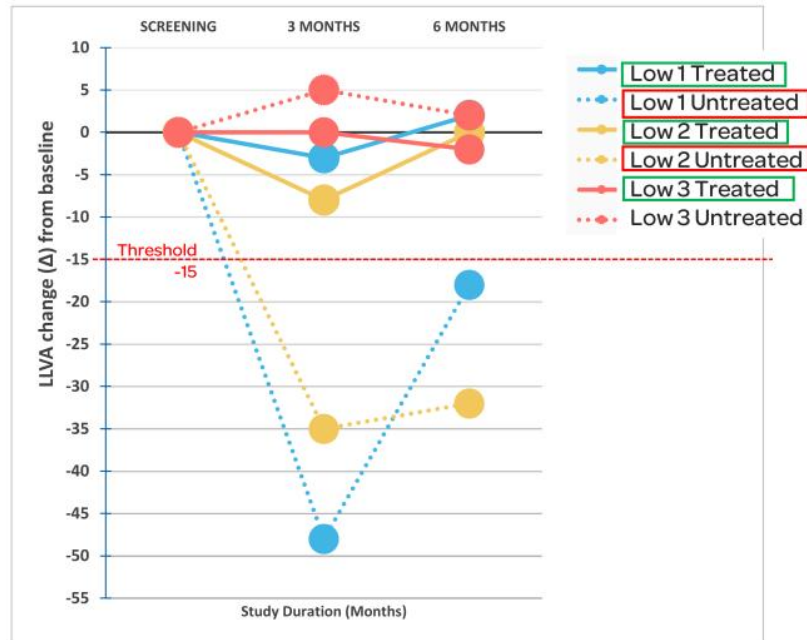


Published data OAKS and DERBY used Sham controls in lieu of untreated.

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)



**3/3 (100%)** of the treated eyes showed stabilization of visual function demonstrating treatment benefit<sup>1</sup>

  Stabilization or Improvement  
  Decrease from Baseline/Screening



Stabilization : ±4 letters from Baseline; Threshold= Loss of 15 letters or more in the untreated eyes from baseline  
 Preservation= Visual acuity saved due to treatment

<sup>1</sup>33% of untreated eyes demonstrated stabilization

## Conclusions

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- 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





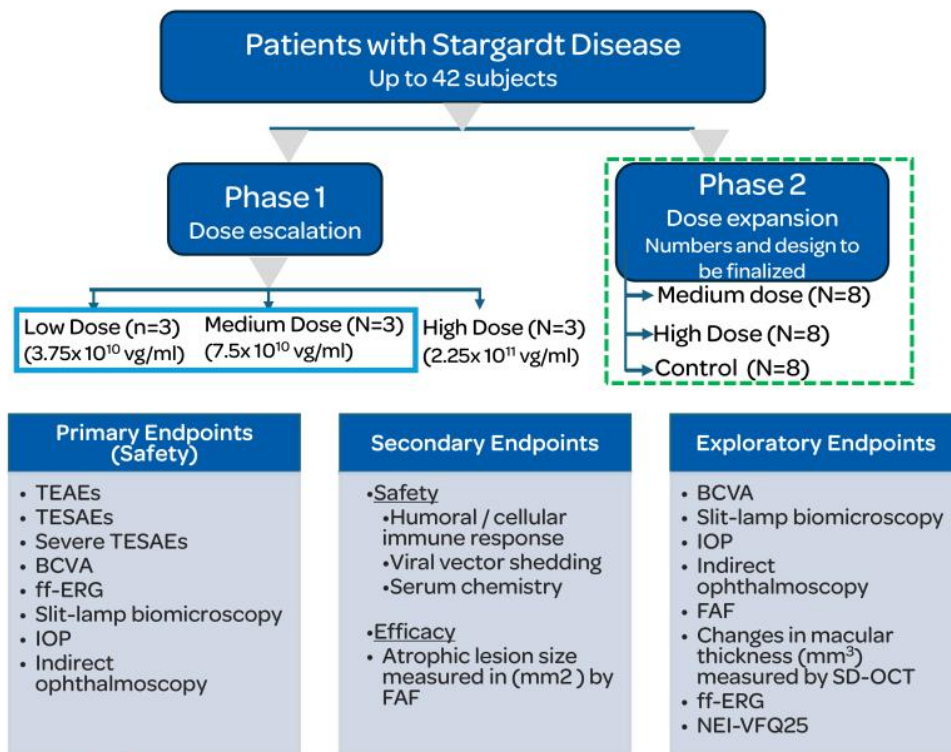
# Trending Positive Outcomes from Novel Modifier Gene Therapy (OCU410ST) from Phase 1/2 Study in Stargardt Disease

Lejla Vajzovic, MD, FASRS  
Director, Duke Surgical Vitreoretinal Fellowship Program,  
Professor of Ophthalmology with Tenure, Adult and Pediatric  
Vitreoretinal Surgery and Disease, Duke University Eye Center,  
and Retina Scientific Advisory Board Chair of Ocugen



NCT06018558

## OCU410ST Phase 1/2 Study : Design and Key criteria



### Key Inclusion Criteria for Adults

- Age 18-75 years
- BCVA ≤ 50 letters ETDRS
- Atrophic lesion requirements:
  - Minimum diameter of ≥ 300 microns for area of atrophy by FAF
  - All lesions together must add to ≤ 18 mm<sup>2</sup> (7 DA)
- Presence of two pathogenic mutations in the ABCA4 gene

### Key Exclusion Criteria for Adults

- Previous treatment with gene or cell therapy
- Concurrent retroviral therapy that would inactivate the investigational product
- Uveitis or endophthalmitis or laser therapy in the macular region
- Choroidal neovascularization, uncontrolled glaucoma, advanced cataract, or other pathologies that render subject unsuitable

**DSMB approved to initiate Phase 2**



Cohorts used for current analysis

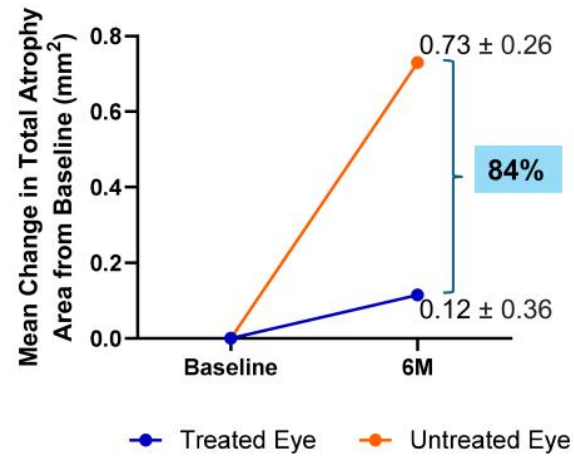
## Overall Safety

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- 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)

## 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



SEM: standard error of the mean; Two subject has no well demarcated area of atrophy on FAF  
One subject with worsening cataract not use for efficacy



## 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 <sup>2</sup> ) 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 =  $\pm 4$  letters from Baseline; Improvement:  $\geq 5$  Letters from Baseline; Preservation= Visual acuity saved due to treatment; Macular Volume (mm<sup>3</sup>) is calculated over entire ETDRS Grid using an algorithm; Central retinal thickness ( $\mu$ m, ILM to anterior border of RPE) measured at the umbo  
N.D.= cannot determine atrophy on spectral-domain OCT at baseline

## Conclusions

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- 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
- Currently aligning with FDA for Phase 2 confirmatory trial

# Patient Panel

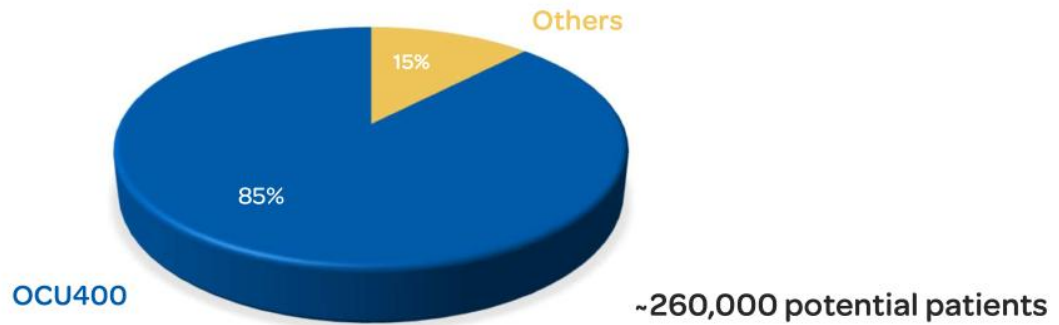
# Commercial Update

Mike Shine, MBA  
SVP Commercial

## Retinitis Pigmentosa: Projected U.S. & EU Market Opportunity for OCU400

Projected Market Landscape at Time of Approval/Launch

\$61B Opportunity



2017

**LUXTURNA**  
vrodigene nepravovectry  
RPE65

Anticipated 2025 – 2026

**Johnson & Johnson**  
Innovative Medicine  
XLRP

**nanoscope**  
THERAPEUTICS  
Severe RP only  
(legally blind)

2026

**ocugen. OCU400**  
Broad RP – mutation agnostic

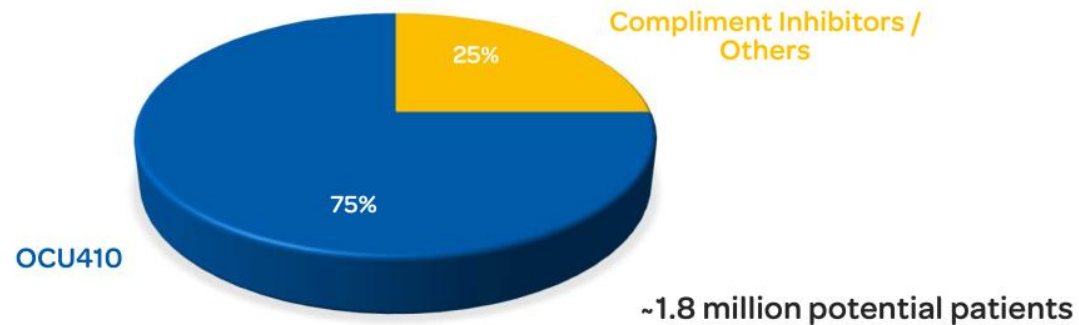




## Geographic Atrophy: Projected U.S. & EU Market Opportunity for OCU410

Projected Market Landscape at Time of Approval/Launch

\$115B Opportunity



2023

**SYFOVRE**  
(pegcetacoplan injection)  
15 mg/0.3 mL  
C3 inhibitor\*

2023

**izervay**  
(avacincaptad pegol  
intraocular solution) 2 mg  
C5 inhibitor\*

Anticipated 2025 - 2027

**ANNEXON**  
biosciences  
C1q inhibitor\*

**Belite**  
BIO  
Retinoid  
Inhibitor

2028

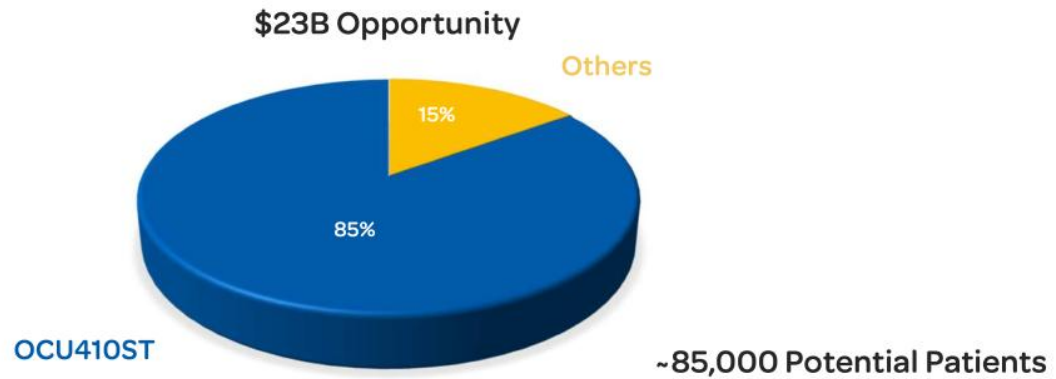
**ocugen. OCU410**  
4-way pathway Modifier  
Gene Therapy approach



\* Monthly / Every other month injections

## Stargardt: Projected US & EU Market Opportunity for OCU410ST

Projected Market Landscape at Time of Approval/Launch



Anticipated 2025 – 2027

  
Retinoid  
Inhibitor

  
Severe ST only  
(Legally Blind 12+)

  
 **OCU410ST**  
Novel Modifier Gene  
Therapy approach



## Ocugen Vision

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We're here to make an impact. At Ocugen, we approach drug development with a sense of urgency, resolve, ingenuity, and boldness. We consider patients in everything we do. **Courageous innovation** means driving science in new directions and breaking new ground.

