# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

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# FORM 8-K

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**CURRENT REPORT**
**Pursuant to Section 13 OR 15 (d)**
**of The Securities Exchange Act of 1934**

Date of Report (Date of Earliest Event Reported): **November 12, 2024**

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# OCUGEN, INC.

(Exact Name of Registrant as Specified in its Charter)

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| **Delaware** | **001-36751** | **04-3522315** |
|---|---|---|
| (State or Other Jurisdiction of Incorporation) | (Commission File Number) | (I.R.S. Employer Identification Number) |

**11 Great Valley Parkway**
**Malvern, Pennsylvania 19355**
**(484) 328-4701**
(Address, including zip code, and telephone number, including area code, of principal executive office)

**N/A**
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8–K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a–12 under the Exchange Act (17 CFR 240.14a–12)

☐ Pre–commencement communications pursuant to Rule 14d–2(b) under the Exchange Act (17 CFR 240.14d–2(b))

☐ Pre–commencement communications pursuant to Rule 13e–4(c) under the Exchange Act (17 CFR 240.13e–4(c))

**Securities registered pursuant to Section 12(b) of the Act:**

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common Stock, $0.01 par value per share | OCGN | The Nasdaq Stock Market LLC<br>(The Nasdaq Capital Market) |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

**Item 7.01 Regulation FD Disclosure.**

Attached as Exhibit 99.1 hereto and incorporated herein by reference is a presentation that Ocugen, Inc. will present at an in-person Clinical Showcase at the Nasdaq Market Site in Times Square, New York City on November 12, 2024, and may use from time to time in presentations or discussions with investors, analysts, and other parties. The information in this Item 7.01, including Exhibit 99.1, is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, and shall not be deemed to be incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 9.01 Financial Statements and Exhibits.**

The following exhibits are being furnished herewith:

**(d) Exhibits**

| Exhibit No. | Document |
|---|---|
| 99.1 | Ocugen, Inc. Presentation |
| 104 | Cover Page Interactive Data File (embedded within the Inline XBRL document). |

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: November 12, 2024

OCUGEN, INC.

By: /s/ Shankar Musunuri

Name: Shankar Musunuri

Title: Chairman, Chief Executive Officer, & Co-Founder

ocugen™

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

*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 fo 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 tha 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

| | |
|---|---|
| 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 (A<br>Clinical Assistant Professor at University of Arizona, College of Medicine – Phoenix<br>Dr. Lejla Vajzovic, Professor of Ophthalmology, Pediatrics, & Biomedical Engineering w<br>Tenure at Duke Eye Center and Duke University School of Medicine<br>Dr. Syed M. Shah, Vitreoretinal Diseases and Surgery/Emplify Health, Vice Chair For Re<br>& 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 |

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Placeholder for video

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

### Company Overview

| | |
|---|---|
| Founded | 2013 |
| Headquarters | Malvern, PA |
| Manufacturing Facility | Malvern, PA |

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

**Our Values**

- Respect
- Integrity
- Teamwork
- Accountability


ocugen
We're Driving Science in New Directions and Breaki
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

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

## Key Retinal Diseases Leading to Blindness

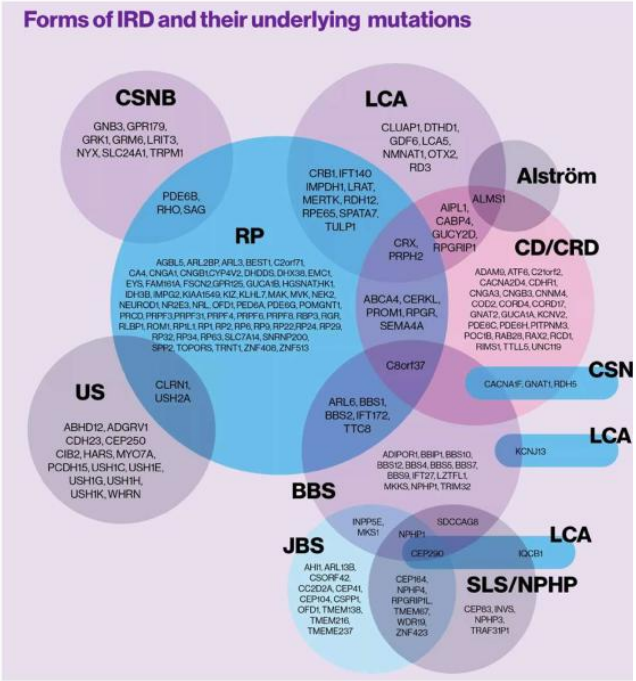
- Inherited retinal diseases (e.g. RP, LCA, Stargardt)
- Multifactorial (Dry AMD; geographic atrophy)

## 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

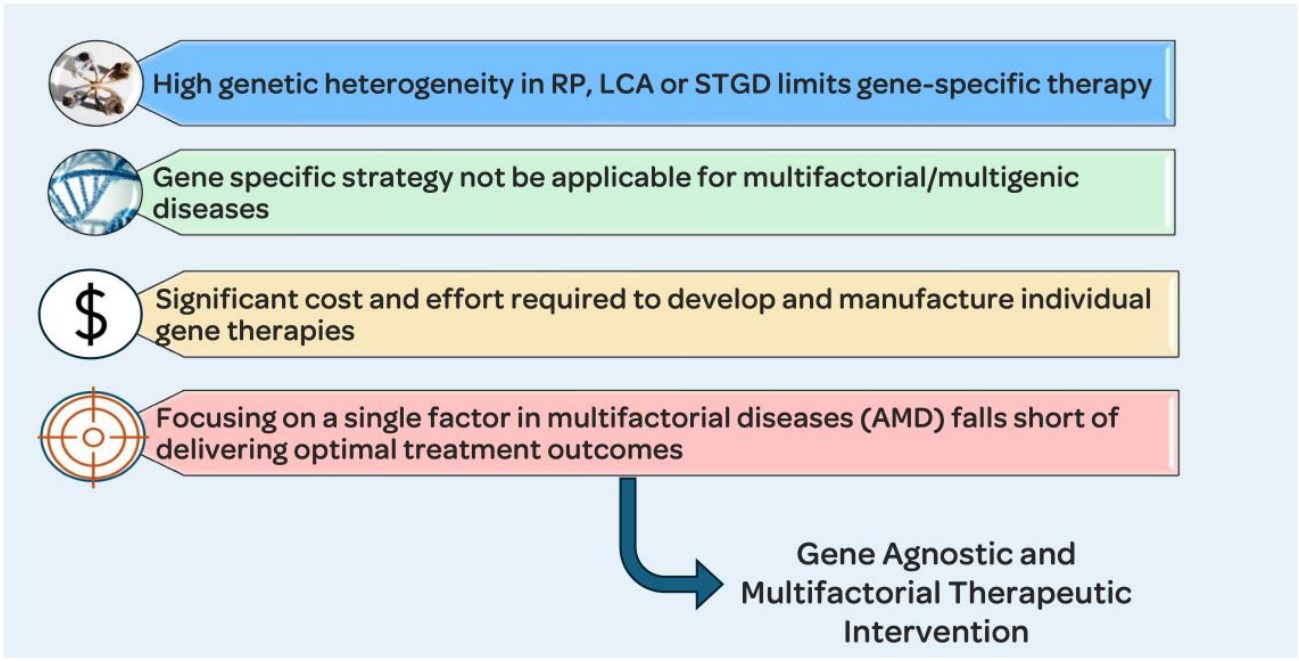
## Significant Unmet Medical Need

- 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



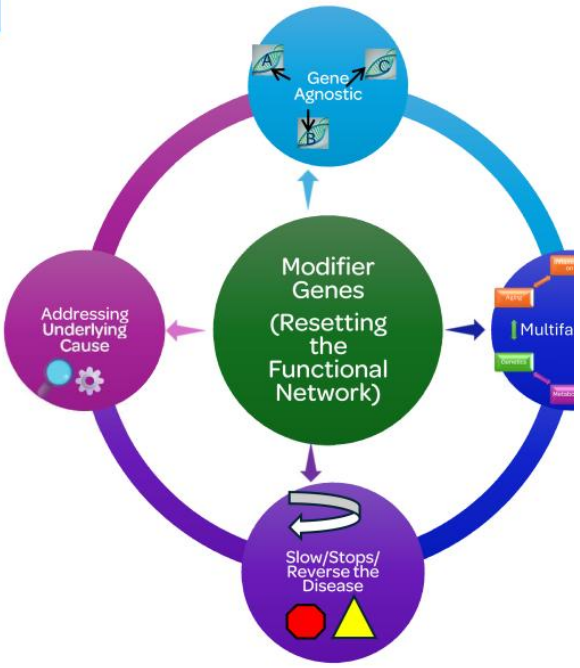
# Modifier Gene Therapy: A Revolutionary Approach to Treat Disease at Roots

## 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

## 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

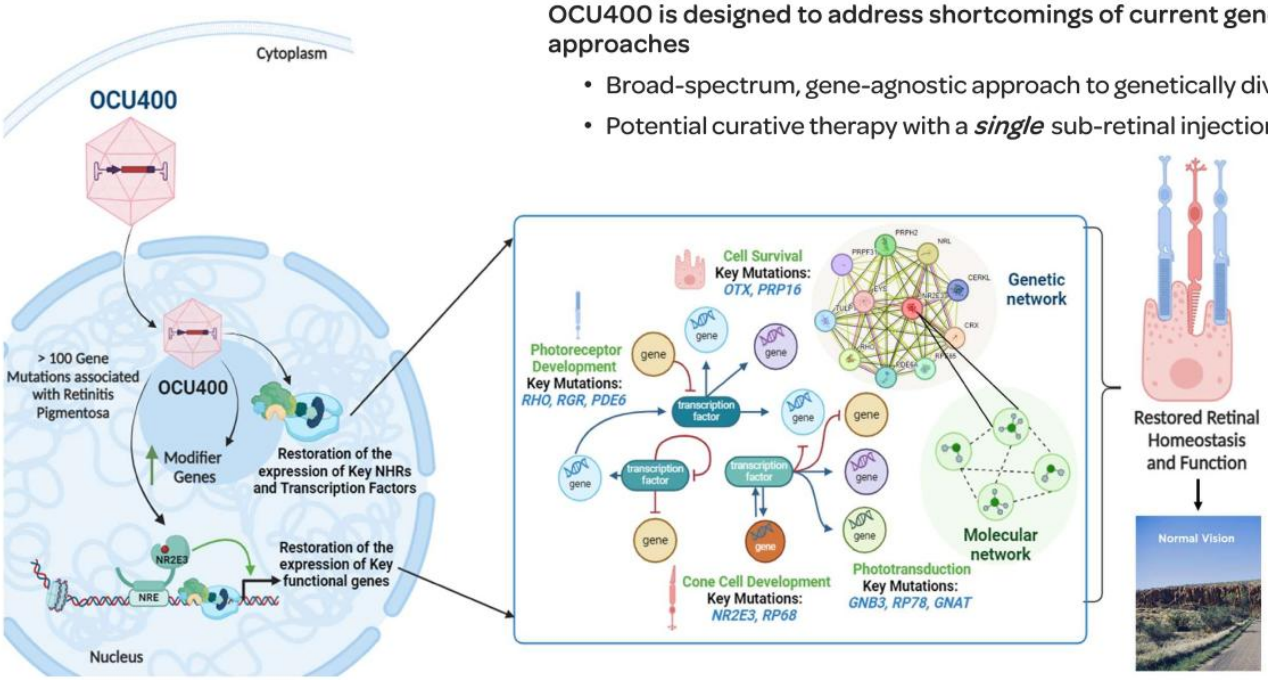
**OCU400: Modifier Gene Therapy**

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

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

**OCU400 is designed to address shortcomings of current gene therapy approaches**

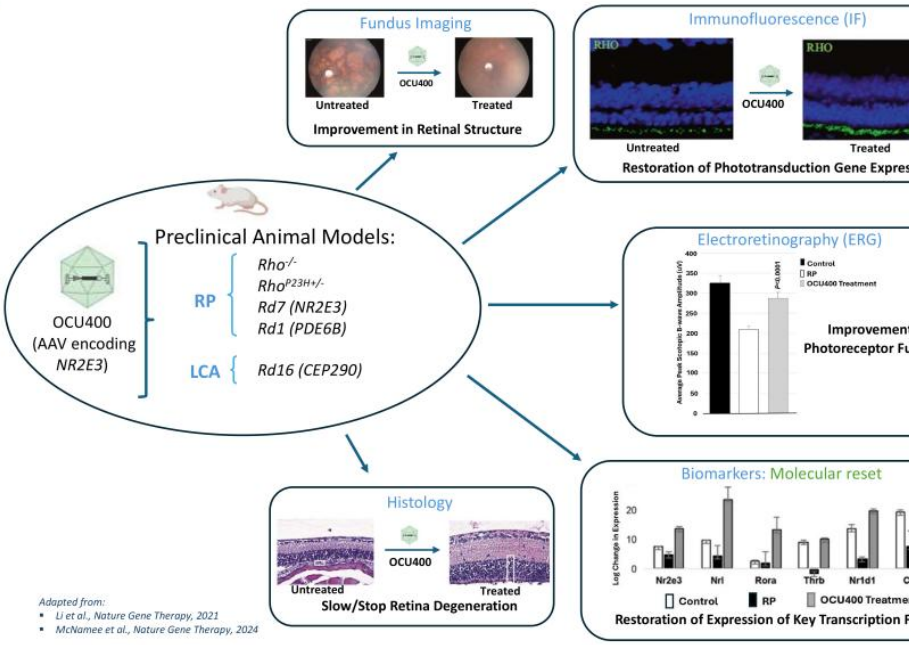
- Broad-spectrum, gene-agnostic approach to genetically diverse IRDs
- Potential curative therapy with a ***single*** sub-retinal injection



# 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




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

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

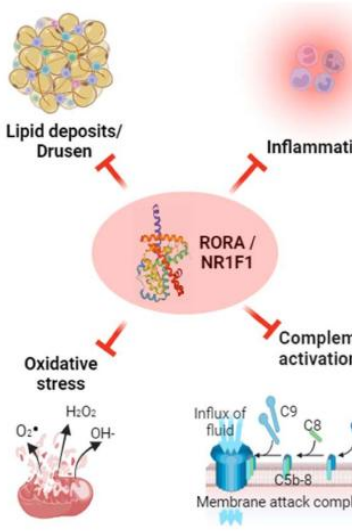
# Regulation of Multiple Pathways by *RORA*

## *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

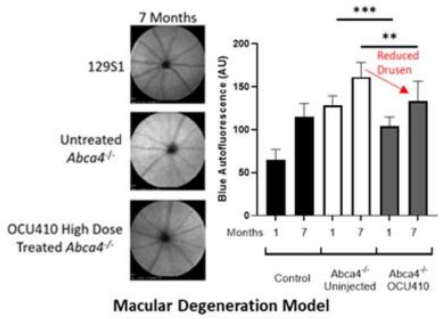
## *RORA* regulates genes involved in:

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



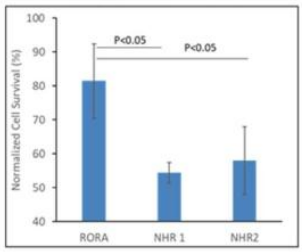
# OCU410 (RORA): Proof of Concept Summary

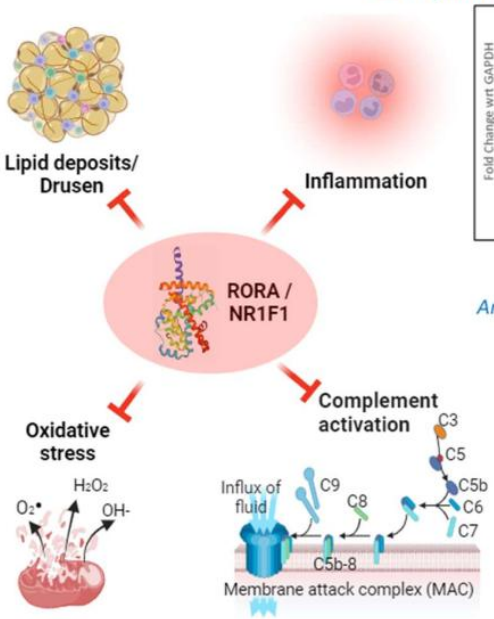
*Anti-drusen activity and improves retinal function*



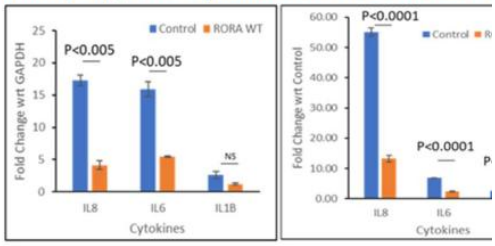

**Macular Degeneration Model**

*Anti-oxidative: Improves ARPE19 cell survival*



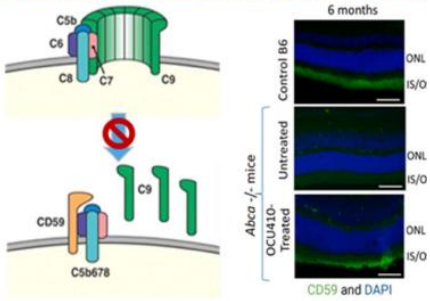




*Anti-inflammatory: Suppresses inflammation in HMC3 and ARPE*




HMC3 ARPE19

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



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

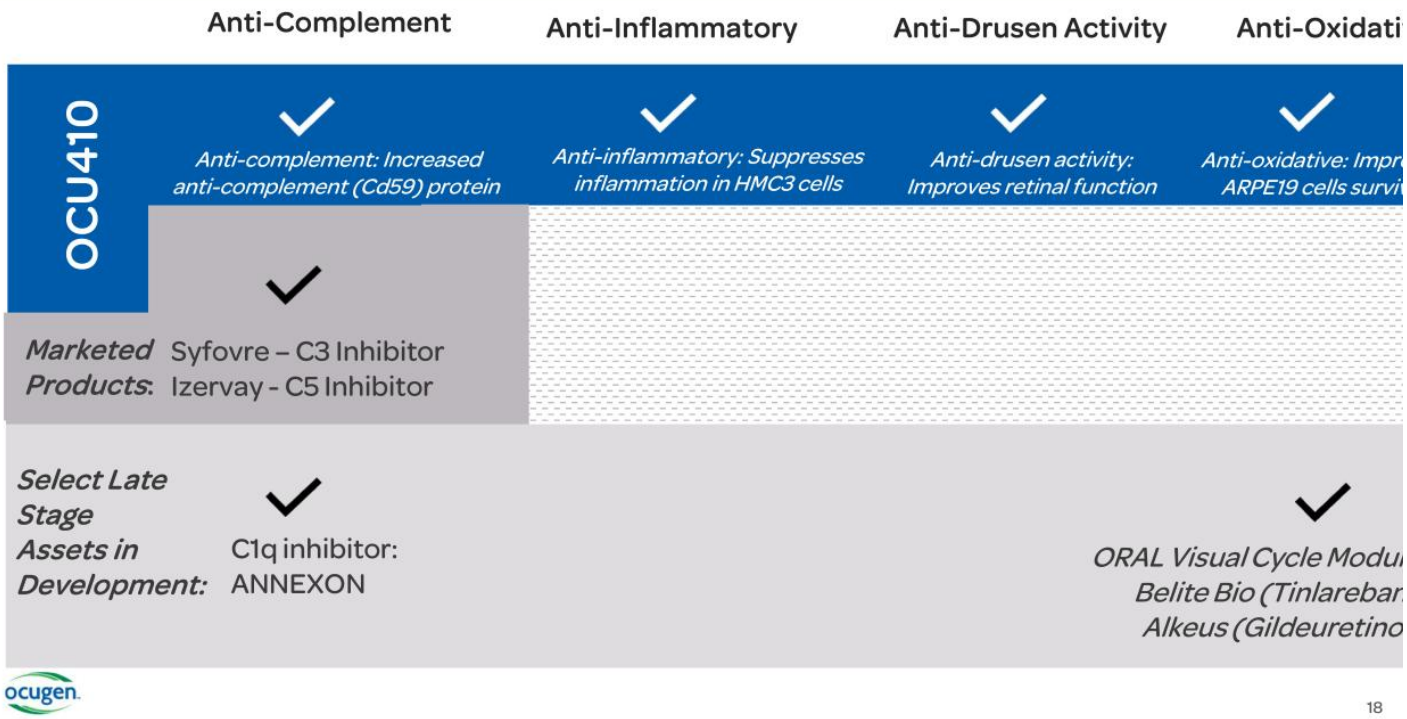
- **Phase 2 OCU410 ArMaDa study for GA currently underway**
- **Limited options for dAMD, presenting significant unmet medical need**
  - U.S. and EU: nearly 19M (GA: 2-3M)[1]
  - Worldwide: 266M[2]
- **Distinct 4-way MOA:**
  Addresses multiple regulator pathways involved with the disease including:
  - 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 therapie 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 whe therapy is administered every month f 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
[1]DB Rein et al., *JAMA Ophthalmol*, 2022. doi: 10.1001/jamaophthalmol.2022.4401
[2]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 Syste

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# 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)

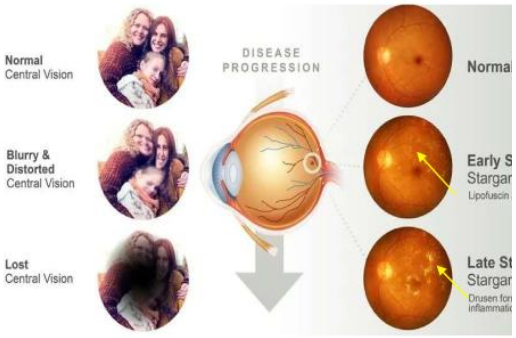
**No treatment options exist**

- 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.***

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

- ✓ 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.*

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# OCU410ST: Modifier Gene Therapy Addressing Shortcomings of Current Approaches

- ✓ 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

## 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 Canda 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)

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## Ocular Programs : Key Highlights

### 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

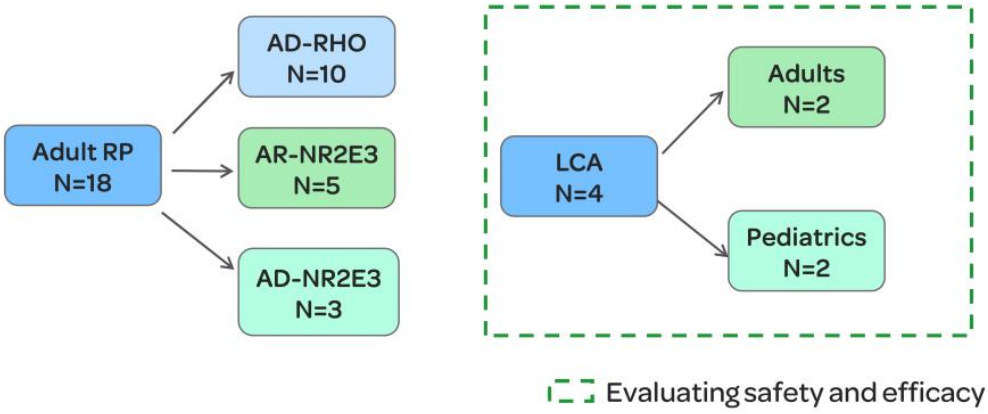
- No current therapies for Stargardt
- Phase 1 dosing complete. Excellent safety profile.
- Aligning with FDA for Phase 2
- Treated eyes show 84% slower lesion growth at 6M compared to untreated eyes
- Up to 80% preservation of retinal structure at 6M and visual function (60% BCVA) at 6M

# 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

## 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



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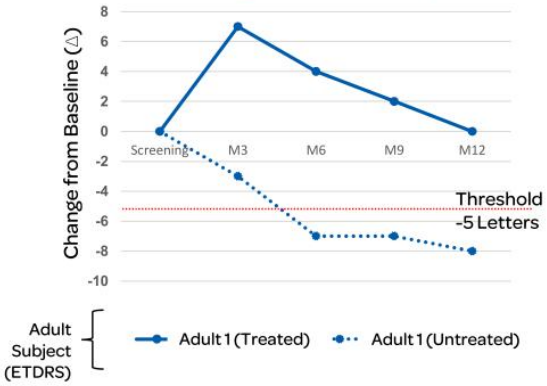
## LCA Open Label Study

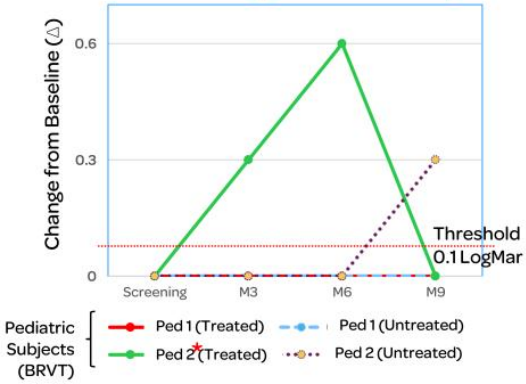
- OCU400 is novel Modifier Gene Therapy – Strong Preclinical Data
- A single subretinal delivery of OCU400 (Medium Dose, $3.33 \times 10^{10}$ vg/mL) in LCA subjects to overexpress *NR2E3*
- Safety outcomes evaluated in all LCA subjects
- Month 9 efficacy is presented for all subjects
  - Additional 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

A beneficial result at M12:
- Preservation in treated eye
- Worsening in the untreated eye



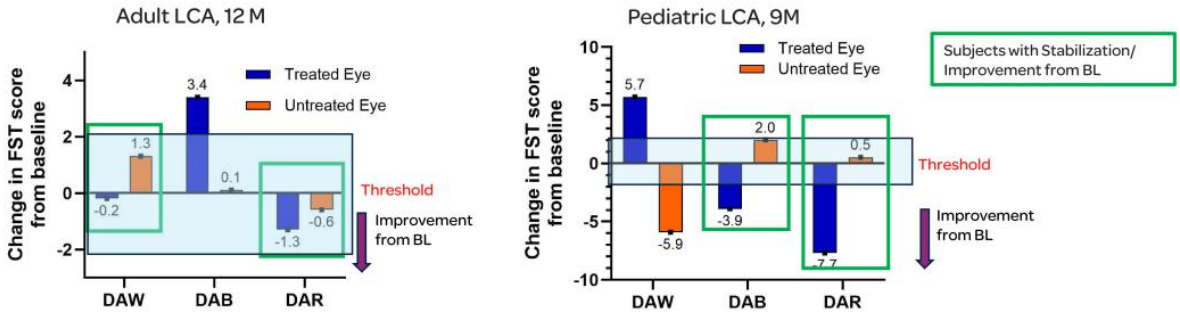

A beneficial result at M9:
- Preservation in treated eye
- Worsening in the untreated eye




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

[1] 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 = ±2dB from Baseline, marked as Threshold band ; Improvement= ≥3dB from Baseline
FST = Full-field stimulus test; DAW = Dark-adapted white light; DAB = Dark-adapted blue light; DAR = Dark-adapted red light

## Conclusions

- 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-2

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

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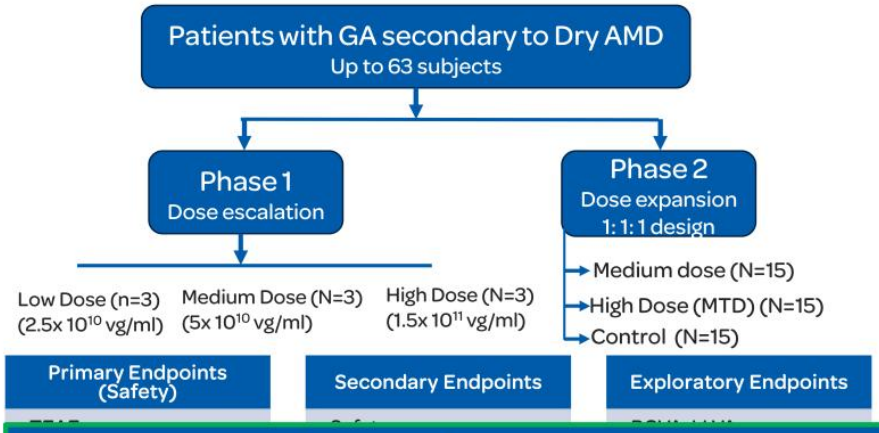
Vitreoretinal Diseases and Surgery/Emplify Health, Vice Chair For Research
& Digital Health, Gundersen Health – La Crosse, Wisconsin

ocugen

NCT06018558

# 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 $mm^2$ and ≤ 20.5 $mm^2$; if multifo at least one lesion must be ≥1.25 $mm^2$ (0.5 DA)
  - GA lesion within foveal and perifoveal region
- Prior treatment with Izervay and Syforvre 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

- No Serious Adverse Events (SAEs) or Adverse Events (AEs deemed related to study drug or study procedure includin ischemic optic neuropathy, vasculitis, endophthalmitis, and choroidal neovascularization

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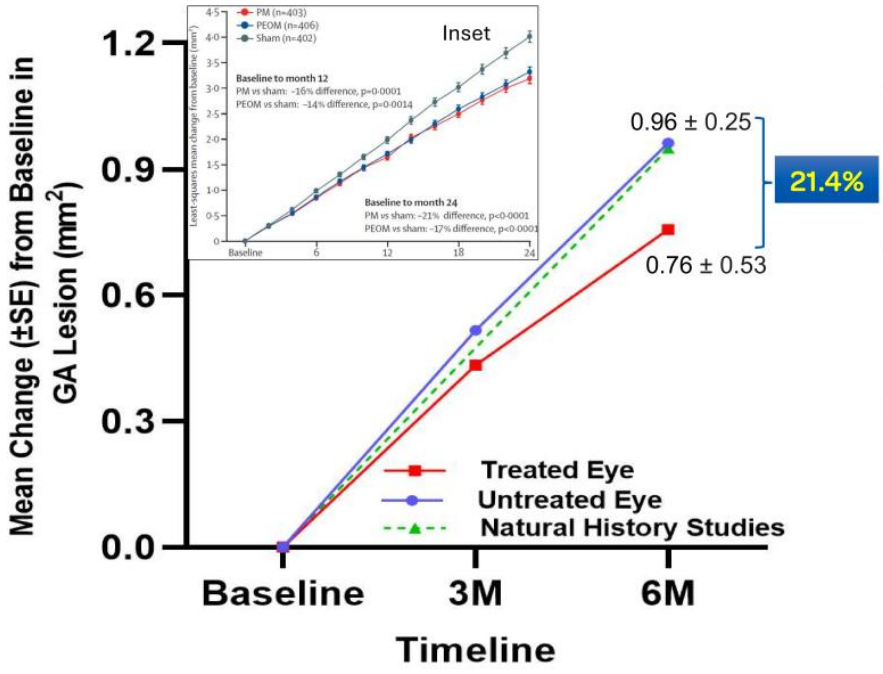
Cohorts used for current analysis

Phase 2 Enrollment – Q4, 2024 completion

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

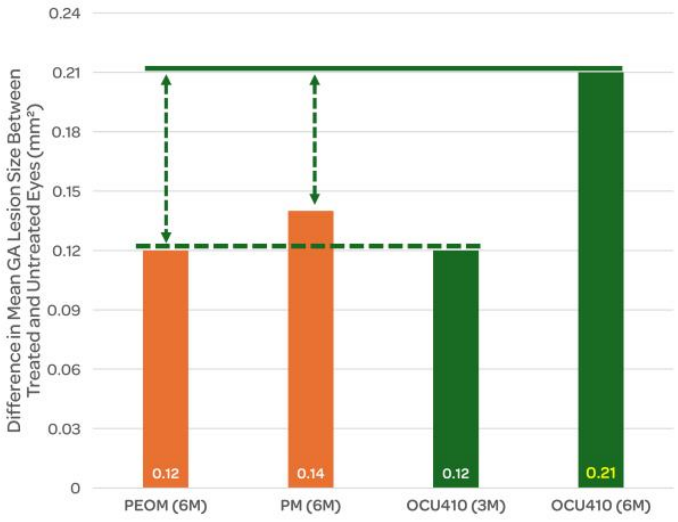
3M (low-dose, N=3 & med-dose, N=2); 6M (low-dose, N=3)




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

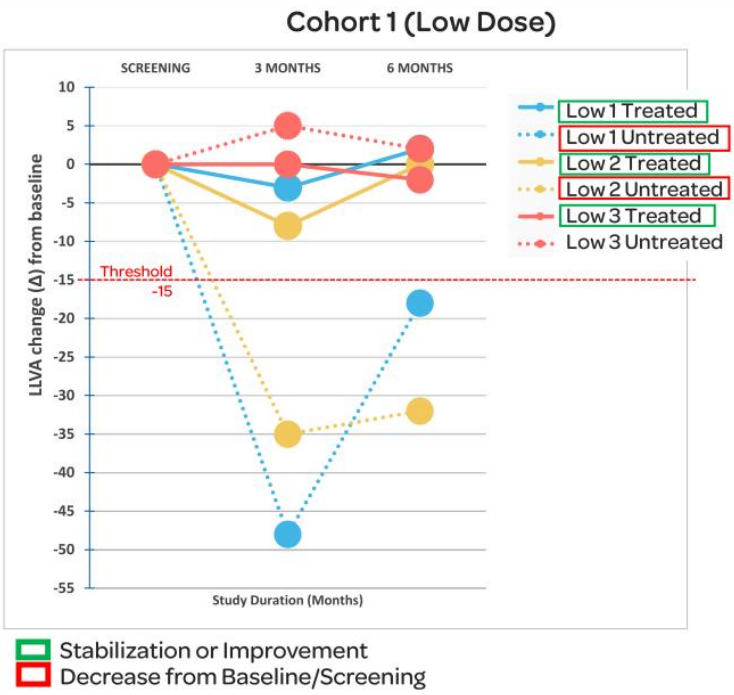



**OCU410 low-dose treatment preserves more retinal tissue around the GA lesions of treated eyes at 6M compared to PM and PEOM**

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




**3/3 (100%)** of the treated eyes showed stabilization of visual function demonstrating treatme benefit[1]

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

[1]33% of untreated eyes demonstrated stabilization

## 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

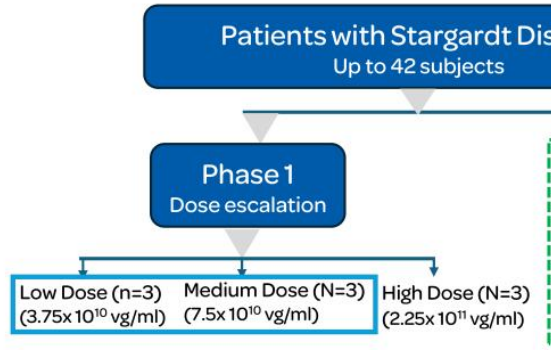
PM=pegcetacoplan monthly; PEOM=pegcetacoplan every other month; MOA= mechanism of Action; Established in preclinical studies
Preservation = Amount of retinal tissue protected from atrophy due to treatment

# 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




**Primary Endpoints (Safety)**

- TEAEs
- TESAEs
- Severe TESAEs
- BCVA
- ff-ERG
- Slit-lamp biomicroscopy
- IOP
- Indirect ophthalmoscopy

**Secondary Endpoints**

- Safety
  - Humoral / cellular immune response
  - Viral vector shedding
  - Serum chemistry
- Efficacy
  - Atrophic lesion size measured in (mm2 ) by FAF

**Exploratory Endpoints**

- BCVA
- Slit-lamp biomicroscopy
- IOP
- Indirect ophthalmoscopy
- FAF
- Changes in macular thickness ($mm^3$) measured by SD-OCT
- ff-ERG
- NEI-VFQ25

**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 mm2 (7 DA)
- Presence of two pathogenic mutations in the ABC gene

**Key Exclusion Criteria for Adults**

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

**DSMB approved to initiate Phase 2**

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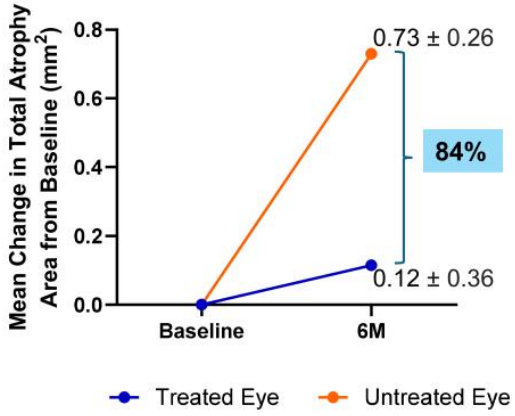
Cohorts used for current analysis

## Overall Safety

- 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 ($mm^2$) 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

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Stabilization = ±4 letters from Baseline; Improvement: ≥5 Letters from Baseline; Preservation= Visual acuity saved due to treatment; Macular Volume ($mm^3$) 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

- 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

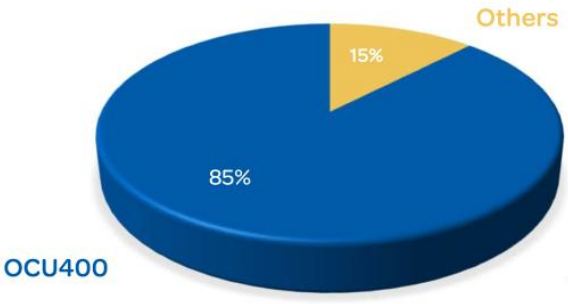
# Commercial Update

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**




~260,000 potential patients

**2017**

LUXTURNA (voretigene neparvovec-rzyl)

RPE65

**Anticipated 2025 – 2026**

Johnson & Johnson Innovative Medicine

XLRP

nanoscope

**Severe RP only (legally blind)**

**2026**

ocugen OCU400

Broad RP – mutation agnostic

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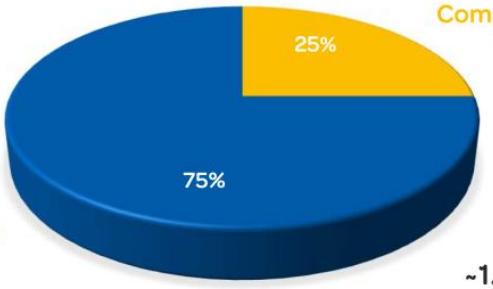
# Geographic Atrophy: Projected U.S. & EU Market Opportunity for OCU410

**Projected Market Landscape at Time of Approval/Launch**

**$115B Opportunity**




**~1.8 million potential patients**

**2023**

SYFOVRE (pegcetacoplan injection) 15mg/0.1mL

C3 inhibitor*

**2023**

izervay (avacincaptad pegol intravitreal solution) 2 mg

C5 inhibitor*

**Anticipated 2025 - 2027**

ANNEXON biosciences

C1q inhibitor*

Belite BIO

Retinoid Inhibitor

**2028**

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4-way pathway Modifier Gene Therapy approach

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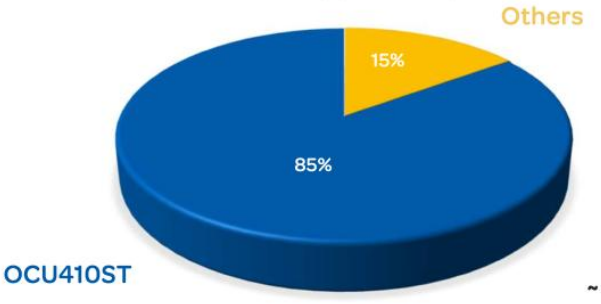
* Monthly / Every other month injections

# Stargardt: Projected US & EU Market Opportunity for OCU410ST

**Projected Market Landscape at Time of Approval/Launch**

**$23B Opportunity**




**~85,000 Potential Patients**

**Anticipated 2025 – 2027**

Belite BIO
Retinoid Inhibitor

nanoscope
Severe ST only (Legally Blind 12+)

2027
ocugen OCU410ST
Novel Modifier Gene Therapy approach

ocugen

# Ocugen Vision

**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 scienc new directions and breaking new ground.