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

April 16, 2024
NASDAQ: OCGN

Forward Looking Statements

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, 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 include, but are not limited to, statements regarding our clinical development activities and related anticipated timelines. 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. These and other risks and uncertainties are more fully described in our 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.

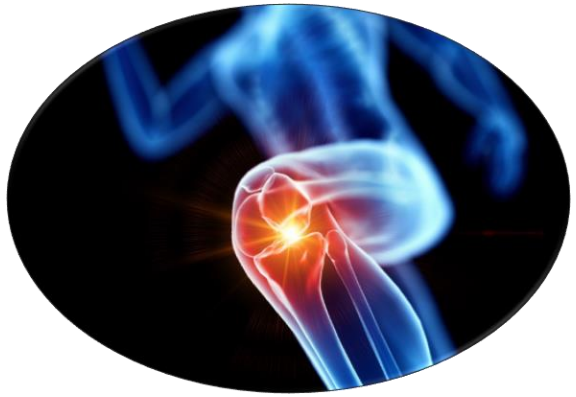
Through Courageous Innovation, We are Leveraging Our First-in-Class Platforms to Address Serious Unmet Medical Needs

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 (Ph3)** RP* & **(Ph1/2)** LCA**:Orphan drug designation from FDA/EMA, RMAT from FDA. **First gene therapy with broad RP indication.** EMA provided acceptability of the U.S.-based trial for submission of a Marketing Authorization Application (MAA)
  - **OCU410 (Ph1/2)**: dry AMD (geographic atrophy)
  - **OCU410ST (Ph1/2)**: Stargardt; Orphan drug designation from FDA

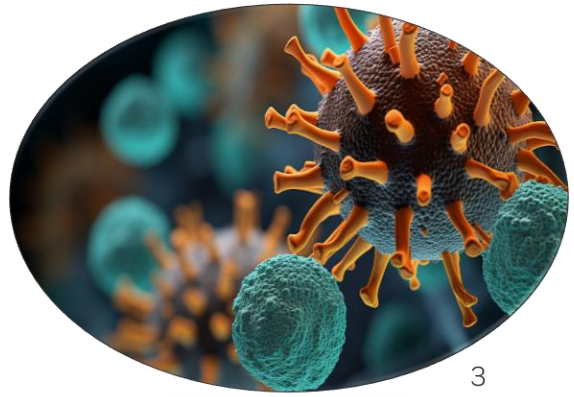
Regenerative Cell Therapy Platform
First-in-Class

- **Therapeutic Focus:** articular cartilage lesions
- **Differentiator:** 3-D scaffold
- **Pipeline:**
  - NeoCart® (Ph3): articular cartilage defects in the knee
    - **Ph3 target: 2H2024**
    - **cGMP manufacturing facility construction completed**
    - RMAT designation from FDA



Inhalation Vaccines Platform
First-in-Class

- **Therapeutic Focus:** Flu and COVID-19
- **Differentiator:** inhalation for improved durability and transmission control
- **Pipeline:**
  - OCU500 (Preclin): COVID-19 vaccine (**NIH/NIAID Nextgen Collaboration – Ph1 planned for mid-2024**)
  - OCU510 (Preclin): flu quadrivalent
  - OCU520 (Preclin): COVID-19 + flu combo



*RP, retinitis pigmentosa; **LCA, Leber congenital amaurosis

Pipeline Overview

	Asset/Program	Indication	Current Status
Gene therapies	**OCU400** * AAV-hNR2E3	*Retinitis pigmentosa (RP)—RHO Mutations*	• Phase 3: RP • Phase 1/2.: LCA. Expect to expand Phase 3 development in LCA patients in 2H 2024. • FDA granted RMAT** designation for RP indication associated with *NR2E3* and *RHO* mutations • EMA provided acceptability of the U.S.-based trial for submission of a Marketing Authorization Application (MAA)
		RP—gene-agnostic indication (other than RHO mutations)	
		Leber congenital amaurosis (LCA)	
	OCU410 AAV-hRORA	Dry Age-Related Macular Degeneration (Geographic Atrophy)	• Phase 1/2 ongoing • Cohort 1 dosing completed (low dose) . Initiation of enrollment in Cohort 2 (medium dose).
	OCU410ST AAV-hRORA	Stargardt disease (orphan disease)	• Phase 1/2 ongoing • Cohort 1 dosing completed (low dose) . Initiation of enrollment in Cohort 2 (medium dose).
Biologics	**OCU200** Transferrin – Tumstatin	Diabetic Macular Edema	• Continue to work with FDA to address comments to lift clinical hold
		Diabetic Retinopathy	• IND-ready
		Wet Age-Related Macular Degeneration (Wet AMD)	• IND-ready
Cell therapies (Regenerative Medicine)	**NeoCart®** (Autologous chondrocyte-derived neocartilage) RMAT	Treatment of Articular Cartilage Defects in the Knee	• Phase 3 clinical trial is planned for 2H 2024, contingent on funding availability • cGMP facility construction completed
Vaccines	**OCU500 Series**		
	OCU500: COVID-19	For Prevention of Disease Caused by COVID-19	• OCU500 IND planned for mid-2024 in collaboration with NIAID
	OCU510: Flu (Quadrivalent)	For Prevention of Disease Caused by Flu	
	OCU520: Flu + COVID-19	For Prevention of Diseases Caused by Flu and COVID-19	

*Broad, gene-agnostic, ORPHAN DRUG DESIGNATIONS FOR RP/LCA FROM FDA AND EMA

**Regenerative Medicine Advanced Therapy Designation

Modifier Gene Therapy Platform

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

Ocugen's Gene Therapy – Fundamentally Changing Gene Therapy

20th Century Disruptive Biotechnologies

- Penicillin
- Polio vaccine
- Recombinant DNA
- Production of monoclonal antibodies
- Genetically engineered bacteria

21st Century Biotechnology Innovations

- Human Genome fully sequenced
- Gene and cell therapies/CRISPR
- First commercial gene therapies
- mRNA vaccines

Ocugen's Modifier Gene Therapy (MGT) – First Broad Mutation Agnostic and Multifactorial Gene Therapy

- Current gene therapy/CRISPR: Costly, mutation-specific and typically addresses ultra rare patient groups
- Ocugen MGT: Potential to treat broad cohorts of patients with inherited retinal diseases with a single therapy
- Being studied in diseases that affect millions (dry AMD) as a potential one-time therapy for life

Ocugen's Modifier Gene Therapy is a First-in-Class Platform Technology Designed to Restore Homeostasis and Preserve Vision

Modifier Gene Therapy Platform
First-in-Class

- **Therapeutic Focus:** Inherited retinal diseases and larger blindness diseases with unmet need

- **Differentiator:** "*Master Gene Regulator*"; gene-agnostic approach
  - OCU400 (***NR2E3***) has been shown in humans (Ph1/2 for RP) to improve vision in patients with ***RHO gene*** mutations—supporting its gene-agnostic mechanism of action
    - RP is associated with over 100 gene mutations and affects more than 110,000 people in the U.S.
  - OCU410 (***RORA***) has been shown to beneficially modify the four pathways which directly contribute to dry age-related macular degeneration (dAMD) in multiple animal models
    - dAMD affects approximately 10 million people in the U.S. The advanced form of dAMD, geographic atrophy (GA), affects ~one million Americans.

RP and LCA—Unmet need and Treatment Benefit Target

- IRDs, such as RP and LCA, are a group of heterogenous genetic disorders that affect the retina, the light-sensitive tissue at the back of the eye
- They often lead to a gradual loss of vision over time and can ultimately result in blindness
- ***Preservation of vision is crucial*** for patients with RP and LCA due to the progressive and degenerative nature of these diseases
- ***Preservation of remaining vision, slowing disease progression, or improving the vision*** can significantly impact patients' quality of life. Such outcomes not only can enhance the quality of life for affected individuals but also provide hope that future treatments that could ultimately lead to vision restoration.
- Comprehensive care, early diagnosis, and access to emerging therapies are essential components of a ***strategy to preserve vision in RP and LCA patients***

OCU400: A Modifier Gene Therapy Targeting RP and LCA - High Unmet Medical Need

Received Regenerative Medicine Advanced Medicine (RMAT) Designation from the FDA

FDA/EMA granted expanded Orphan Drug Designations for all retinitis pigmentosa (RP) and Leber congenital amaurosis (LCA) mutations

Despite its prevalence, RP and LCA patients have very limited treatment options

- US: RP & LCA affect 110,000 and 15,000 people, respectively
- Worldwide: conditions affect approximately 1.6M people

Current approved and in-development gene therapies focus on individual gene

- More than 125 mutated genes associated with RP and LCA
- Developing a single therapy to treat each mutation is not feasible

OCU400 addresses shortcomings of current gene therapy approaches

- Broad-spectrum, gene-agnostic approach to genetically diverse inherited retinal diseases
- Potential one-time, curative therapy with a ***single*** sub-retinal injection, using *NR2E3*

Status:

- Released Phase 1/2 safety and efficacy results
- Initiating Phase 3 limelight clinical trial for RP with a sample size of 150 patients
    - FDA granted RMAT designation for RP indication associated with *NR2E3* and *RHO* mutations
    - Luminance Dependent Navigation Assessment (LDNA)—that includes a wider range of light intensity—is the primary endpoint
- Expect to expand Phase 3 development in LCA patients in 2H 2024

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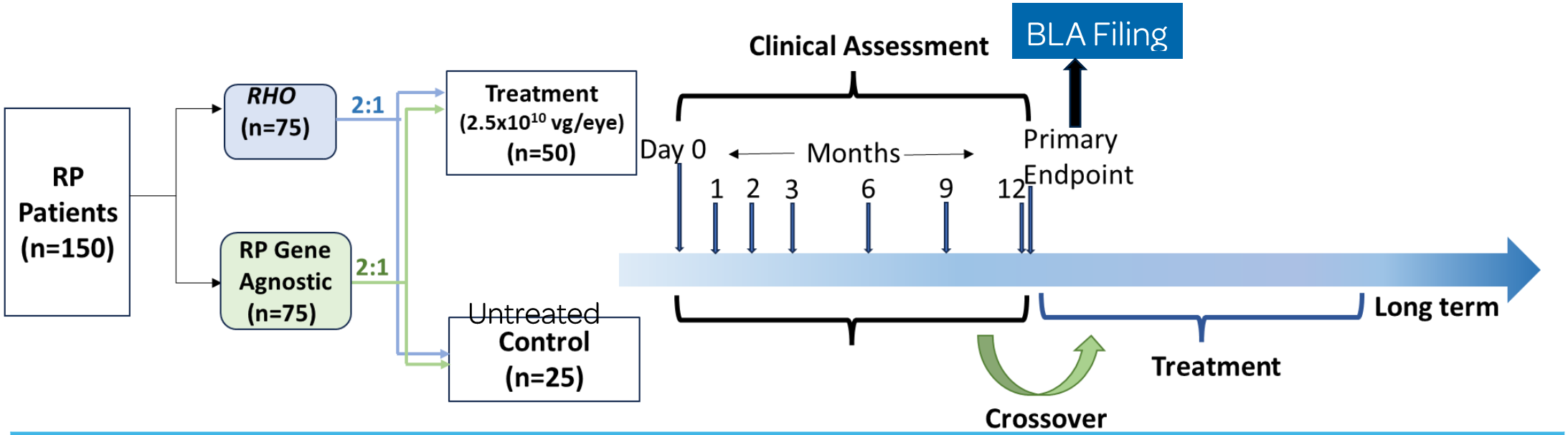
Phase 1/2 Safety and Efficacy Summary

- OCU400 continued to be generally safe and well-tolerated in subjects across different mutations and dose levels
- Efficacy measurements suggest ***positive trends*** in Best-Corrected Visual Acuity (BCVA) and Multi-Luminance Mobility Testing (MLMT), and Low-Luminance Visual Acuity (LLVA) among treated eyes
- 89% (16/18) of subjects demonstrated ***preservation or improvement*** in the treated eye either on ***BCVA or LLVA or MLMT*** scores from baseline
- 78% (14/18) of subjects *demonstrated* ***preservation or improvement*** in treated eyes in MLMT scores from baseline
- 80% (8/10) *of RHO* mutation subjects experienced *either preservation or improvement in MLMT scores* from baseline
- Treatment effect in *RHO* patients supports the gene-agnostic mechanism of action of OCU400

Phase 3 Study Design

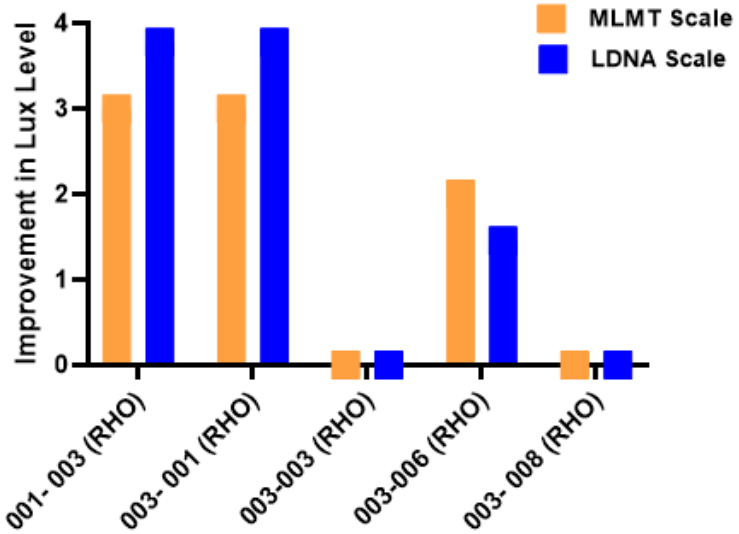


Study Design	
Population	• Patient with ≥8 years of age with Clinical and Molecular Diagnosis of Retinitis Pigmentosa
Key Eligibility Criteria	• BCVA ≤ 75 letters and ≥35 letters (ETDRS Chart) • Able to perform LDNA at ≤ 500 Lux but unable to pass the LDNA at ≤ 0.35 at the Screening visit • Presence of photoreceptors
Endpoints	
Primary	• Proportion of responder (**LDNA ≥ 2 Lux Level from Baseline- Study Eyes**) in treatment vs control arm
Secondary	• Proportion of responder EYES (**LDNA ≥ 2 Lux Level from Baseline**) in treatment vs control • Proportion of responder (LLVA score change of 0.3logMAR from Baseline) in treatment vs control

LDNA: Luminance Dependent Navigation Assessment

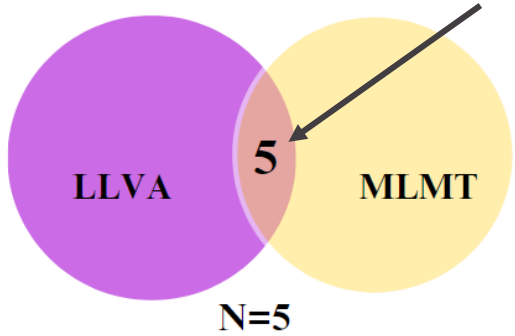
Phase 1/2 Patient Population Meeting "Intent to Treat Population" Criteria for Phase 3 Study*

Demonstrate Gene Agnostic Effect in RHO Patients



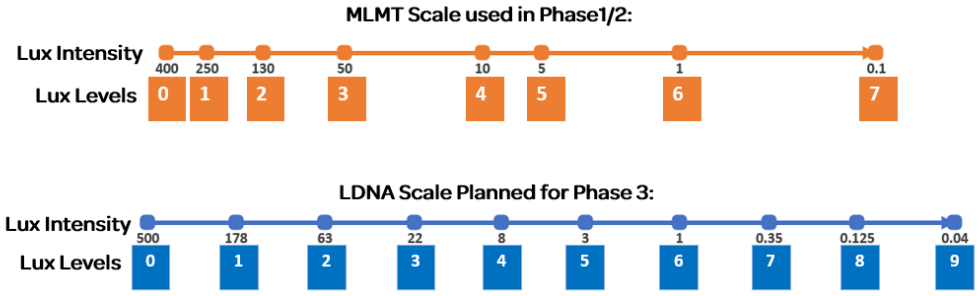
More than 50% ITT RHO Patients Meet Responder Criteria

*Showed **stabilization** or **improvement** on both the parameters from Baseline*



Stabilization:
LLVA: ± 4 letters change
MLMT: 0 change in Lux Level

Improvement:
LLVA: ≥ 5 letters change
MLMT: ≥1 change in Lux Level

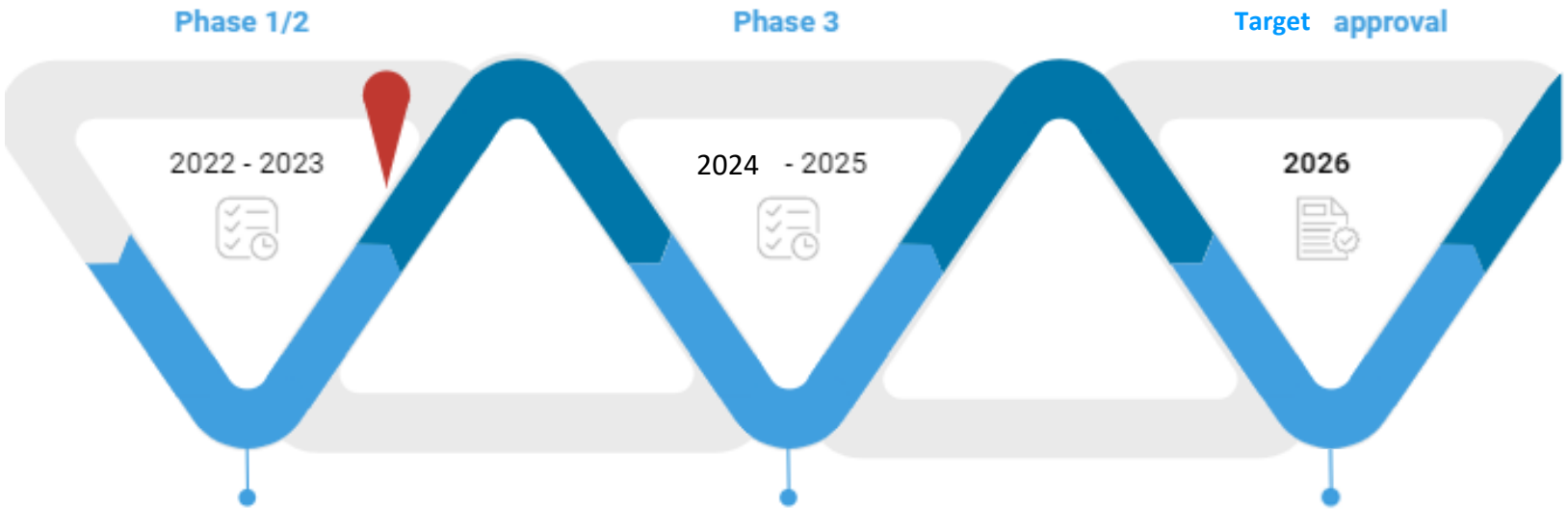


LDNA: Luminance Dependent Navigation Assessment (Updated Mobility Course)

- Wide range of light intensity (0.04-500 Lux) and Lux Levels (0-9)
- Uniform correlation between Lux level and Lux intensity

*Primary Endpoint: Proportion of responder (LDNA ≥ 2 Lux Level from Baseline- Study Eyes) in treatment vs control arm

OCU400: Expected Pathway to Clinical Development & Potential Approval



Adult & Pediatric

RP — *NR2E3*

RP — *RHO*

LCA — *CEP290*

- Both FDA & EMA granted broad orphan drug designation for RP & LCA
- Received RMAT designation from FDA

Indication

Treatment of retinitis pigmentosa (RP)

Expect to pursue LCA indication in 2H 2024

BLA/MAA

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

- **Currently dosing patients in Phase 1/2 ArMaDa clinical trial of OCU410**
  - Initiation of enrollment in Cohort 2 (medium dose) in the dose-escalation phase of the study

- **Limited options for dAMD, presenting significant unmet medical need**
  - U.S.: 10M (GA: 1M)
  - Worldwide: 266M

- **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*

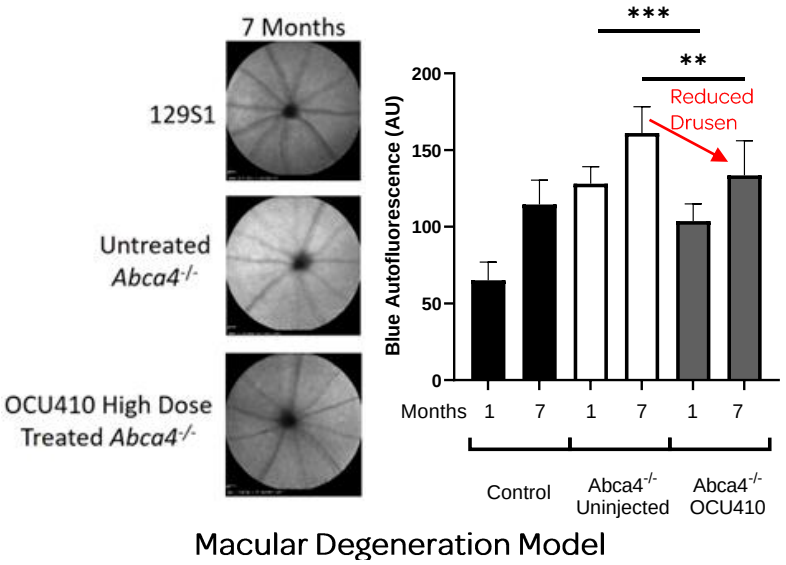
- **Advancement from recently approved therapies for GA:** Potential to address limitations of recently approved therapies for GA focused only on the complement system, including:
  - Patient Compliance
    - Frequent intravitreal injections (~6-12 doses per year)
  - Observed Structural Impact
    - Limited effect of GA lesion growth rate
  - Safety Considerations
    - 12% of patients experienced nAMD when therapy is administered every month for two years (Syfovre®)

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

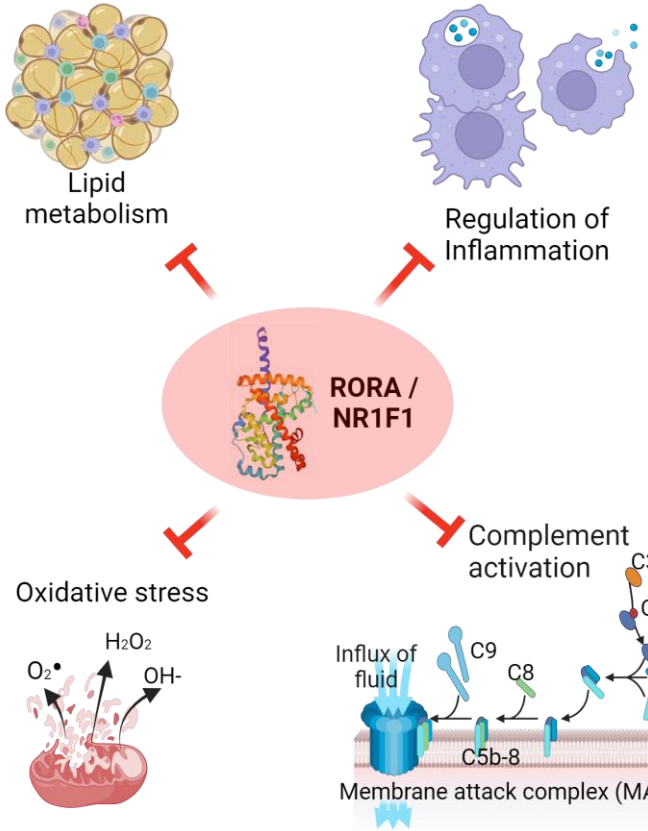
** As demonstrated in Peak scotopic B-wave amplitudes. N≥5 biological replicates*

OCU410 (RORA): A Potential Modifier Therapeutic for Dry-AMD and STGD

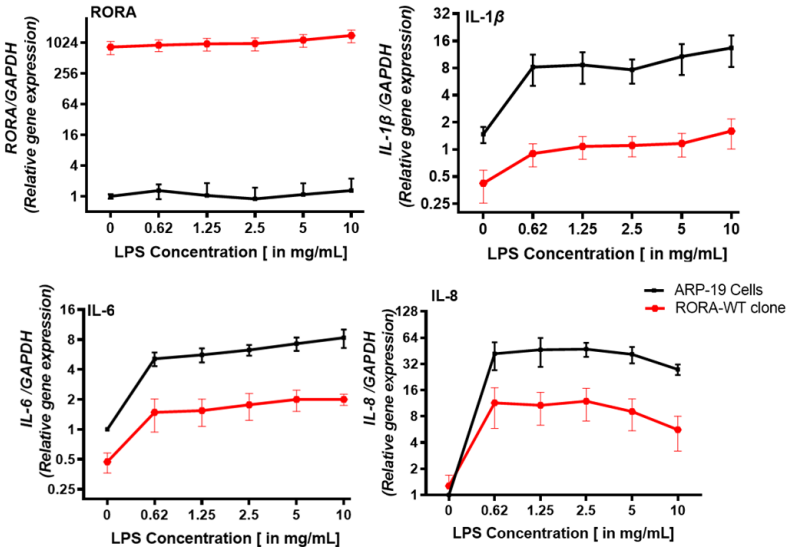
Anti-drusen activity: Improves retinal function



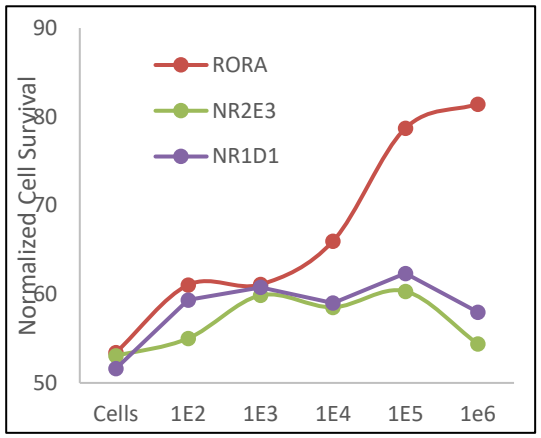
Macular Degeneration Model



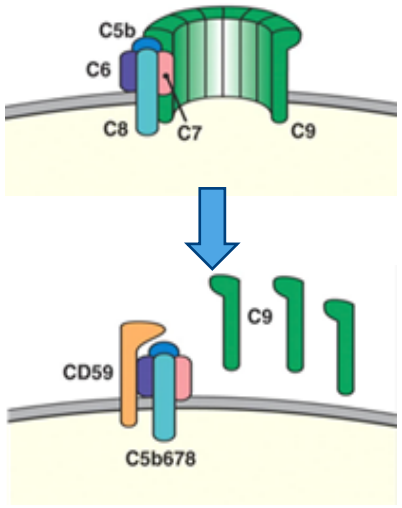
Anti-inflammatory: Suppresses inflammation in HMC3 cells

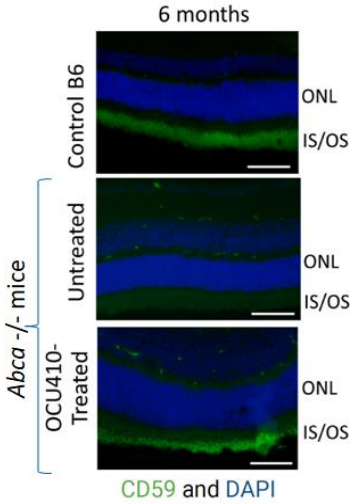


Anti-oxidative: Improves ARPE19 cell survival



Anti-complement: Increases anti-complement (Cd59) protein





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

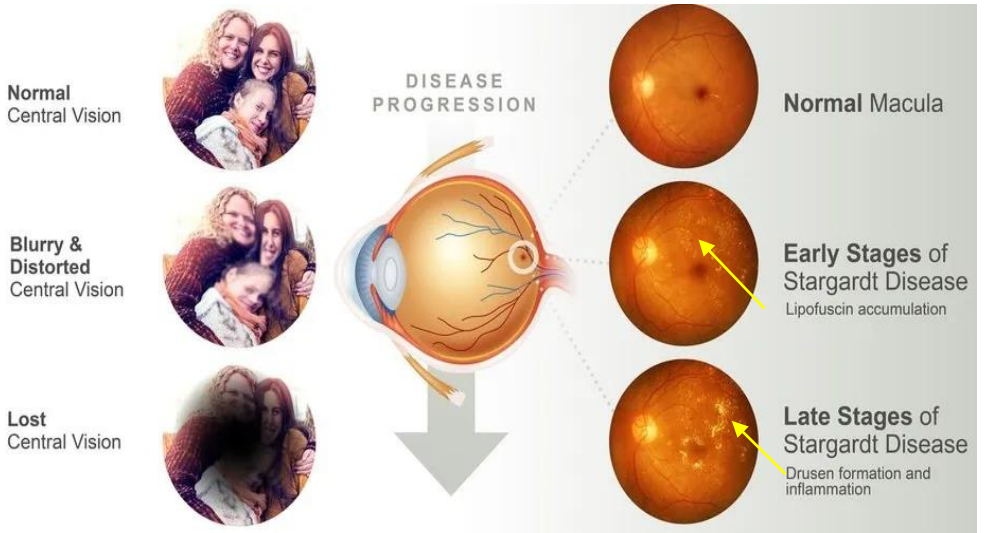
- U.S.: 44,000 patients

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

Currently dosing patients in Phase 1/2 GARDian clinical trial of OCU410ST

- Initiation of enrollment in Cohort 2 (medium dose) in the dose-escalation phase of the study



NeoCart®

(Autologous chondrocyte-derived neocartilage)

NeoCart®: U.S. FDA Agreed to Proposed Control and Overall Design for Phase 3 Trial

NeoCart is a regenerative cell therapy

- Received RMAT designation
- Combines bioengineering and cell processing to enhance autologous cartilage repair
- Potential to accelerate healing and reduce pain through reconstructing damaged knee cartilage

High prevalence of knee cartilage damage, with progression to osteoarthritis (OA)

- Arthroscopic knee procedures: over 1M annually*
- OA: 528M diagnosed worldwide
- Cell therapy global revenue forecast: $45B+, with North America expected to hold largest share**

Current therapies to treat cartilage damage in the knee suboptimal

- Varying outcomes due to variable cellular responses
- Current standard of care suffers from one or more of the following: pain, reduced knee function, failure to address cartilage damage, donor tissue availability, open surgery

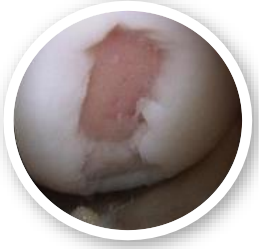
NeoCart potentially addresses shortcomings of current treatments

- Treat pain, improve function, and prevent progression to OA
- Potential for improved efficacy, long-term benefits

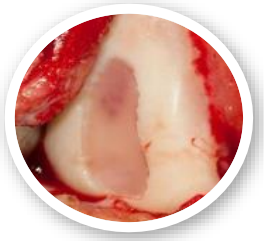
Program advancing on several fronts

- Received FDA concurrence on confirmatory trial design of Phase 3 (plan to initiate in 2H 2024, contingent upon available funding)
- Construction of cGMP manufacturing facility complete

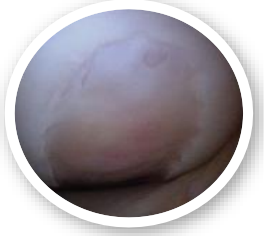
Follow-up Arthroscopy Demonstrates NeoCart® Progression and Integration**



Initial Lesion



Time Zero Implantation



8 Weeks

6 Months

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*The Journal of Bone & Joint Surgery: June 1, 2011 - Volume 93 - Issue 11 - p 994-1000

**https://www.biospace.com/article/cell-therapy-market-size-cagr-trends-forecast-report-2022-2030/

OCU500 Series:

OCU500: COVID-19 Mucosal Vaccine

OCU510: Flu

OCU520: COVID-19/Flu

OCU500 Series: Next-Generation Vaccine Technology

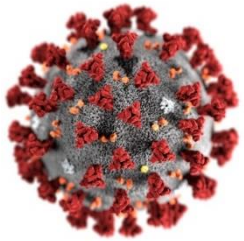
Inhaled mucosal vaccine platform based on ChAd vector

Inhalation technology as a differentiator

- Multiple preclinical studies using Ocugen's vector demonstrated vaccine-induced high neutralizing and effector responses
- Clinical studies using a similar vector administered via the inhalation platform showed mucosal antibodies, systemic antibodies, and durable immune response up to 1 year with 1/5 of the dose compared to the same vaccine given via intramuscular administration
- The inhaled method offers the potential for broad, durable protection from severe disease and reduction in transmission

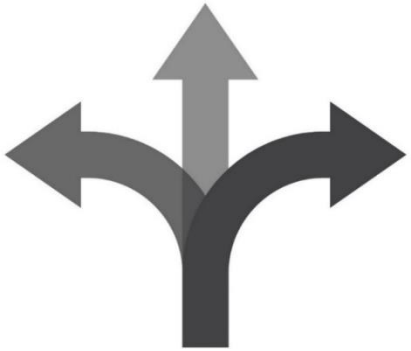
Alignment with American Pandemic Preparedness Plan to transform U.S. capabilities to rapidly and effectively respond to existing and emerging infectious diseases via:

- Legislative advocacy for next-generation mucosal vaccine development
- OCU500 was selected by the NIH/NIAID Project NextGen for inclusion in clinical trials. NIAID is planning to initiate the Phase 1 clinical trials in mid-2024.

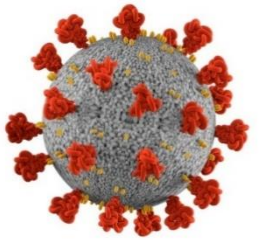


OCU500
COVID-19 vaccine

OCU520
A combination quadrivalent flu and COVID-19 vaccine



OCU510
A seasonal quadrivalent flu vaccine



2024 Near-Term Targeted Milestones

- Initiate OCU400 Ph3 clinical trial and recruit efficiently – in line with 2026 BLA/MAA approval target
- OCU400 Ph3 clinical updates
- Preliminary safety/efficacy updates – OCU410 Ph1/2 clinical trial (GA)
- Preliminary safety/efficacy updates – OCU410ST Ph1/2 clinical trial (Stargardt)
- Finalize partner for OCU400 – to maximize value for patients and shareholders

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**

Patient Video