



Courageous  
Innovation

February 2023  
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 based on the beliefs and assumptions of Ocugen, Inc. and on information currently available to management. All statements contained in this presentation other than statements of historical fact are forward-looking statements. 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. 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. Forward-looking statements that we make in this presentation are based on a combination of facts and factors currently known to us and speak only as of the date of this presentation. Except as required by law, we assume no obligation to update forward-looking statements contained in this presentation whether as a result of new information, future events, or otherwise, after the date of this presentation.*

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

**Mission:** Developing cutting-edge innovations for people facing serious disease and conditions with a commitment to ensuring global market access

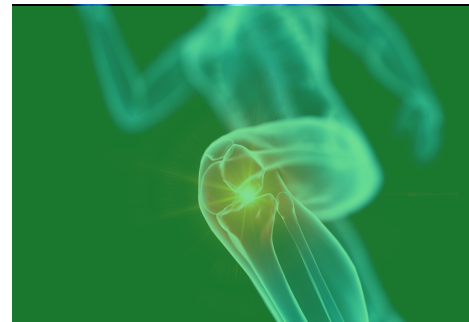
Pioneering modifier gene therapy for inherited retinal diseases, as well as larger blindness diseases with unmet need



Innovating a novel biologic to treat eye diseases that can lead to vision loss for millions of people



Developing vaccines to provide choice in the fight against COVID-19



Pursuing Regenerative Cell Therapy to treat serious conditions like articular cartilage lesions



# Pipeline Overview

	Asset/Program	Indication	Current Status
Vaccines	COVAXIN™ (BBV152) SARS-CoV-2 virus	COVID-19	<ul style="list-style-type: none"> <li>EUA for adults in Mexico; EUA for 5 to 18-year-olds submitted</li> <li>Top line results show that the U.S. Phase 2/3 immuno-bridging and broadening clinical trial met both primary endpoints. Awaiting final data.</li> </ul>
	OCU500 series Inhaled mucosal vaccine	COVID-19 & Flu	<ul style="list-style-type: none"> <li>COVID-19 technology licensed from Washington University</li> <li>Phase 1/2 pending FDA discussions</li> <li>Partnering with CanSino Bio – novel delivery device</li> </ul>
Cell therapies (Regenerative Medicine)	NeoCart® (Autologous chondrocyte-derived neocartilage)	Treatment of Articular Cartilage Defects in the Knee	<ul style="list-style-type: none"> <li>U.S. Regenerative Medicine Advanced Therapy (RMAT) designation</li> <li>Received concurrence from the FDA on Phase 3 clinical trial strategy</li> <li>Phase 3 clinical trial is planned to begin in early 2024</li> </ul>
Gene therapies	OCU400 ** AAV-hNR2E3	Gene mutation-associated retinal degeneration*	<ul style="list-style-type: none"> <li>Completed dose escalation and established maximum tolerable dose (MTD)</li> <li>Encouraging safety profile to date</li> </ul>
		<i>NR2E3 Mutation (RP)</i>	Phase 1/2
		<i>RHO Mutation (RP)</i>	Phase 1/2
		<i>CEP290 Mutation (LCA)</i>	Phase 1/2
	OCU410 AAV-hRORA	Dry Age-Related Macular Degeneration (Dry AMD)*	IND planned for Q2 2023
Biologicals	OCU410ST AAV-hRORA	Stargardt disease (orphan disease)	IND planned for Q2 2023
	OCU200 Transferrin – Tumstatin	Diabetic Macular Edema	IND planned for Q1 2023
		Diabetic Retinopathy	IND-enabling
		Wet Age-Related Macular Degeneration (Wet AMD)	IND-enabling



\*No approved therapies exist  
<https://www.aaopt.org/eye-health/diseases/retinitis-pigmentosa-treatment> | <https://www.aaopt.org/eye-health/diseases/amd-treatment>

\*\*Broad ORPHAN DRUG DESIGNATIONS in the U.S.; Broad ORPHAN MEDICINAL PRODUCT DESIGNATION by the EC for the treatment of RP and LCA

# OCU500

## COVID-19 Mucosal Vaccine

Exclusive license agreement with Washington University to develop, manufacture and commercialize its proprietary vaccine in the United States, Europe, Japan and other major markets

# OCU500 Inhaled Mucosal Vaccine for COVID-19 & Seasonal Flu

- Potential to generate rapid local immunity in the upper airways, and lungs - where SARS-CoV-2 and seasonal flu enters and infects the body
- Shown to generate neutralizing IgG, mucosal IgA, and T cell responses to help reduce transmission rate of COVID-19
- Mucosal immunity has been demonstrated as a potential way to prevent infection and spread of COVID-19, which contributes to the evolution of new variants
- This approach represents a potential universal booster, regardless of previous COVID-19 vaccination
- Quadrivalent flu formulation intended to cover multiple seasonal flu strains

## Other features include:

- 1<sup>st</sup> Inhaled vaccine
- Non-invasive
- Needle-free administration
- Potential for increased compliance
- Scalable manufacturing
- Stored and shipped at standard refrigerated conditions
- Potential to develop multi-strain and variant specific versions



# Licensed Mucosal COVID-19 Vaccines Have Been Well-Tolerated and Demonstrated Efficacy as a Heterologous Booster in Phase 3 Trials

## *Studies demonstrating the benefit of AAV*

### Bharat Biotech: ChAd-Nasal Dropper

#### Ph 3 (N=2160): Superior Immune Response

- iNCOVACC® (N=3000) vs.
- COVAXIN™ (N=160)

#### Improved Immunogenicity in Ph3: iNCOVACC vs. COVAXIN

- Superior GMT ratio of nAb for Wuhan (1.45)
- Superior GMT ratio of nAb for Omicron BA.5 (2.1)
- GMT ratio for secretory IgA in saliva (1.3)

#### Improved Safety in Ph3: iNCOVACC vs. COVAXIN

- Systemic AEs 2.7% (iNCOVACC) vs. 6.2% (COVAXIN)
- Nasal reactions 4.9% (iNCOVACC)
- Injection reactions 23% (COVAXIN)

### CanSino Bio: Ad5-Nebulizer/Inhaled

#### Five booster studies

Ph 3 (SeiHOPE trial): N=13000

Dose: 1/5 of IM dose

#### Improved Immunogenicity:

- Cross protection against Omicron with heterologous booster
- Produced T-cell responses higher than IM route
- Significantly higher neutralizing antibody responses to WT and Omicron BA.1 compared with inactivated vaccine
- Improved serum IgA antibody titers vs. inactivated and subunit vaccines for BA.4/5

#### Improved Safety: iNCOVACC vs. Inactivated Vaccine

- Significantly lower number of injection site reactions vs. inactivated vaccine

#### References:

- [https://papers.ssrn.com/sol3/papers.cfm?abstract\\_id=4342771](https://papers.ssrn.com/sol3/papers.cfm?abstract_id=4342771)
- <https://doi.org/10.1101/2022.03.08.22271816>
- <https://doi.org/10.1080/22221751.2022.2132881>
- [https://www.thelancet.com/journals/lanres/article/PIIS2213-2600\(22\)00087-X/fulltext](https://www.thelancet.com/journals/lanres/article/PIIS2213-2600(22)00087-X/fulltext)
- NCT05517642
- NCT05124561

# COVAXIN™ (BBV152)

A whole-virion inactivated COVID-19 vaccine candidate licensed from Bharat Biotech (BBIL) for North American Markets



# Why COVAXIN™ (BBV152)?

Designed to augment our North American arsenal of vaccines against COVID-19

## DESIGNED FOR BROAD SPECTRUM IMMUNE RESPONSE

- Adult and pediatric phase 2/3 data suggest both humoral & cellular responses generated against multiple viral proteins
- Data support that the vaccine induces a Th1 response (cell-mediated immunity), which can be vital for durable protection

## RESULTS SHOW PREVENTION OF SEVERE COVID-19 DISEASE

- Phase 3 data suggest prevention of hospitalizations caused by COVID-19
- Booster dose provides robust neutralizing antibody responses against Omicron and Delta variants

## KNOWN SAFETY PROFILE USING VERO CELL PLATFORM

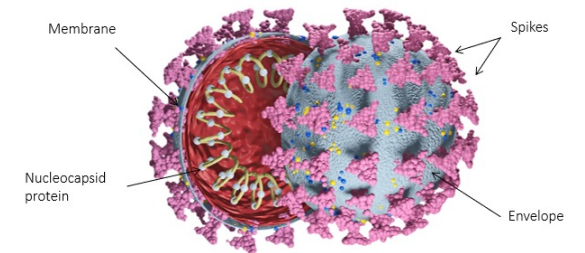
- Data demonstrate strong safety profile within adult and pediatric populations
- Similar technology platform used to produce Polio, Influenza and Rabies vaccines

## TRANSPORTATION AND STORAGE EASE

- 10 dose vial that can be stored and shipped at 2°- 8° C with an expected 2-year shelf life and 6-month stability at room temperature



Image for illustrative purposes only



# Pathway for COVAXIN™ (BBV152) Development

NCT: 05258669

## Phase 2/3 Trial

*A Phase 2/3, Observer-Blind, Immuno-bridging, and Broadening Study of a Whole, Inactivated Severe Acute Respiratory Syndrome Coronavirus (SARS-CoV-2) Vaccine (BBV152) in Healthy Adults*

Study Type:	Interventional (Clinical Trial)
Enrollment:	419 participants
Allocation:	Randomized
Intervention Model:	Parallel assignment
Intervention Model Description:	1:1 randomization ratio
Primary Purpose:	Prevention

Immuno-bridging and broadening

Top line results



Safety

BLA Submission Window

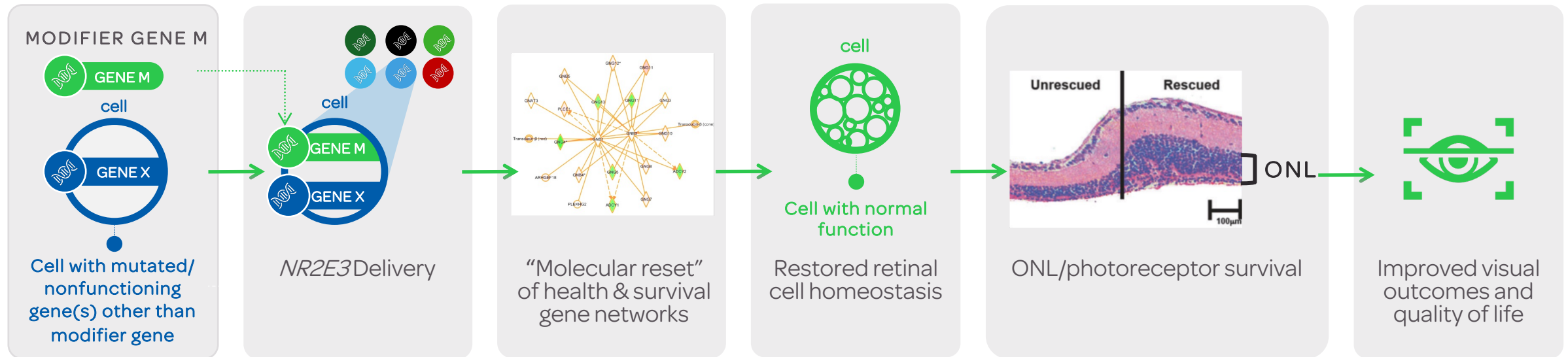
# MODIFIER GENE THERAPY PLATFORM

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

# Modifier Gene Therapy: A Broader Reach

Gene modifier therapy can potentially address multiple genetic defects with a single product utilizing a gene agnostic approach.

In patients with IRDs, this could mean:



# Proof of Principle: Published in Nature Gene Therapy

- Efficacy results shown in five unique mouse models of RP
- Technology developed at Harvard Medical School, Dr. Neena Haider's Lab
- Study suggests potency of modifier gene therapy to elicit broad-spectrum therapeutic benefits in early and advanced stages of RP
- Results suggest evidence of vision rescue in early & advanced stages of disease



Important milestone for development of therapy; demonstrated proof of principle



Protection elicited in multiple animal models of degeneration caused by different mutations



Potential to represent first broad-spectrum gene agnostic therapy and provide rescue even after disease onset

**nature**research

<https://www.nature.com/articles/s41434-020-0134-z>

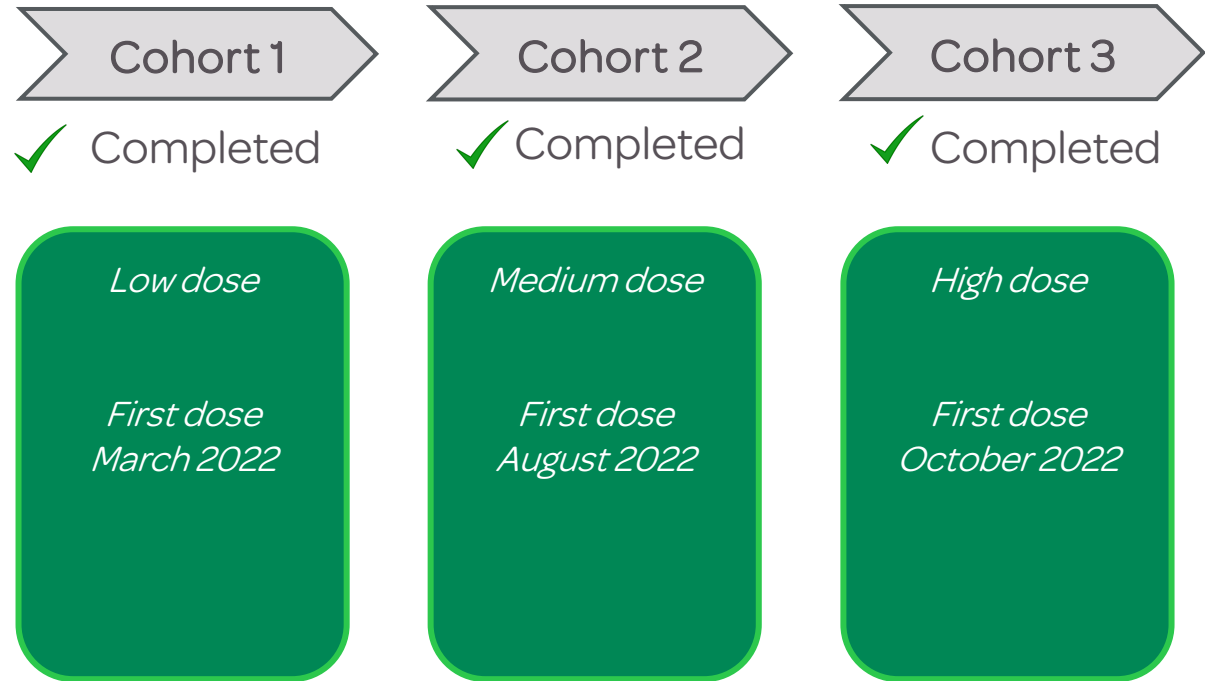


# OCU400 Phase 1/2 U.S. Clinical Trial Progress

## OCU400

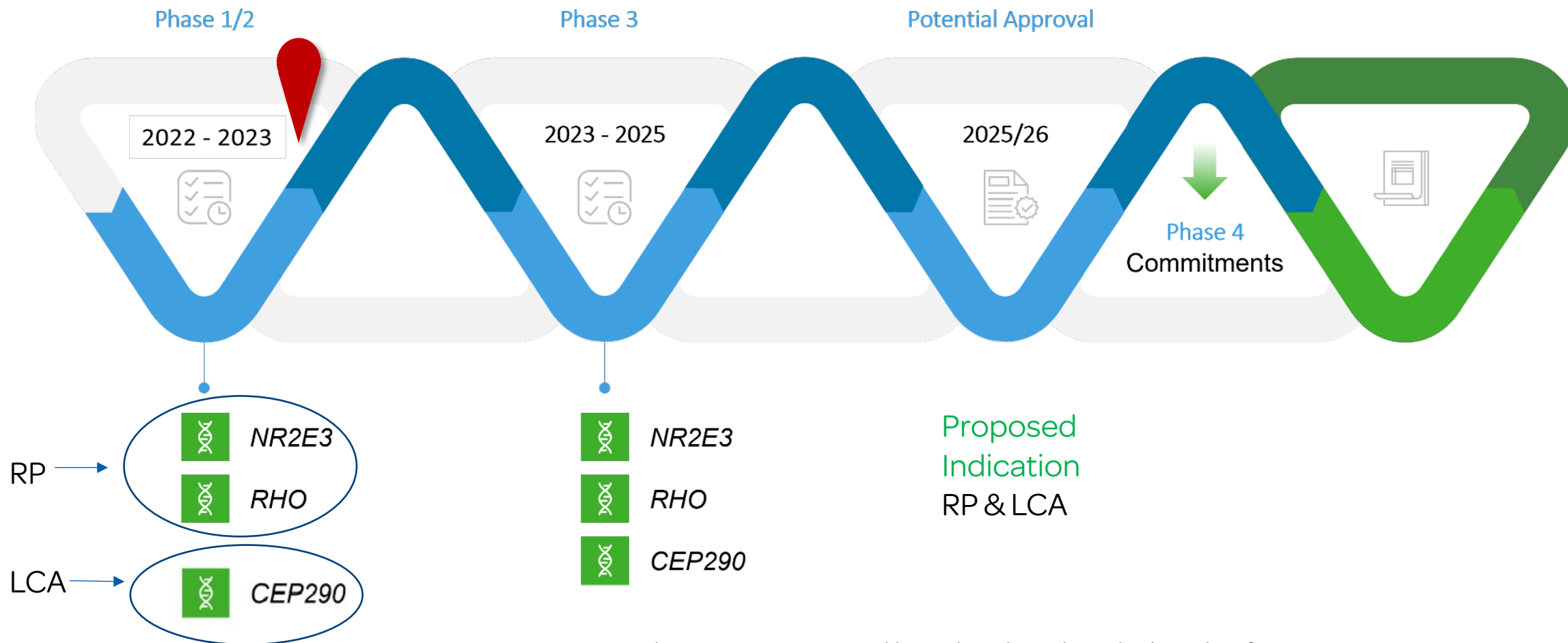
*PHASE 1/2 Study to Assess the Safety and Efficacy of OCU400 for Retinitis Pigmentosa Associated with NR2E3 and RHO Mutations and Leber Congenital Amaurosis with Mutations(s) in CEP290 Gene*

NCT:	05203939
Study Type:	Interventional (Clinical Trial)
Estimated Enrollment:	21 participants
Clinical Trial Sites:	Seven
Allocation:	Non-randomized
Intervention Model:	Sequential assignment
Masking:	None (Open Label)
Primary Endpoint:	Safety
Observational endpoint	Efficacy (structural, functional, BCVA, mobility)
Dosing:	Escalation study involving low, medium, high doses



- More than 70% enrolled
- Dose escalation completed & MTD established (high dose)
- Encouraging safety profile to date
- Expected efficacy signal mid-2023

# OCU400 Expected Pathway to Clinical Development & Potential Approval

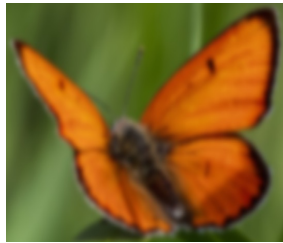


# Age-related Macular Degeneration(AMD)

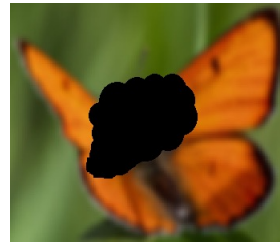
## Stargardt Disease (STGD)



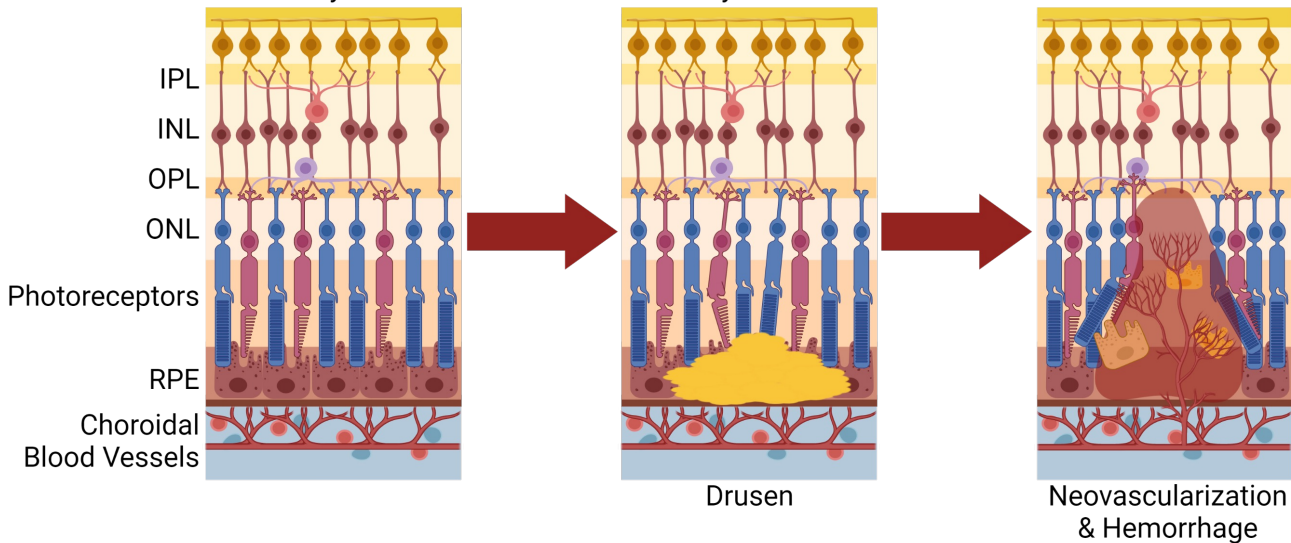
Healthy Retina



Dry AMD



Wet AMD



### Macular Degeneration

Acquired  
Age-related

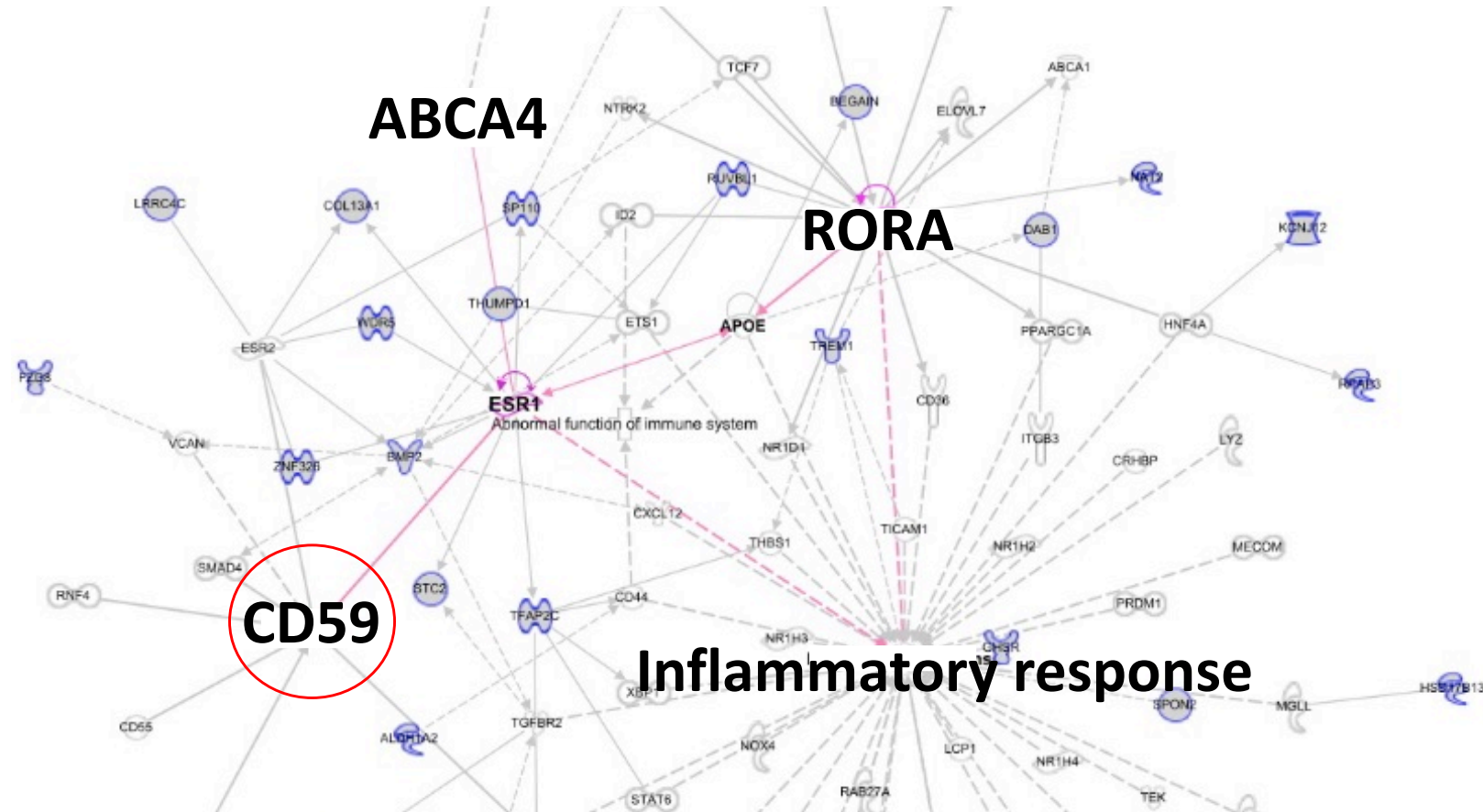
Inherited

- ❖ AMD starts as a “dry,” non-neovascular form
- ❖ Progresses to geographic atrophy
- ❖ Most prevalent (≈90%) form
- ❖ Can progress to a more rapid “wet,” neovascular form
- ❖ Wet AMD may cause vascular leakage, hemorrhage, and fibrovascular growth

- ❖ Stargardt Disease (STGD1)\*
  - Dysfunction of ABCA4,
  - >1,200 ABCA4 variants are associated with STGD
  - Important for detoxifying oxidized retinol compounds in visual cycle

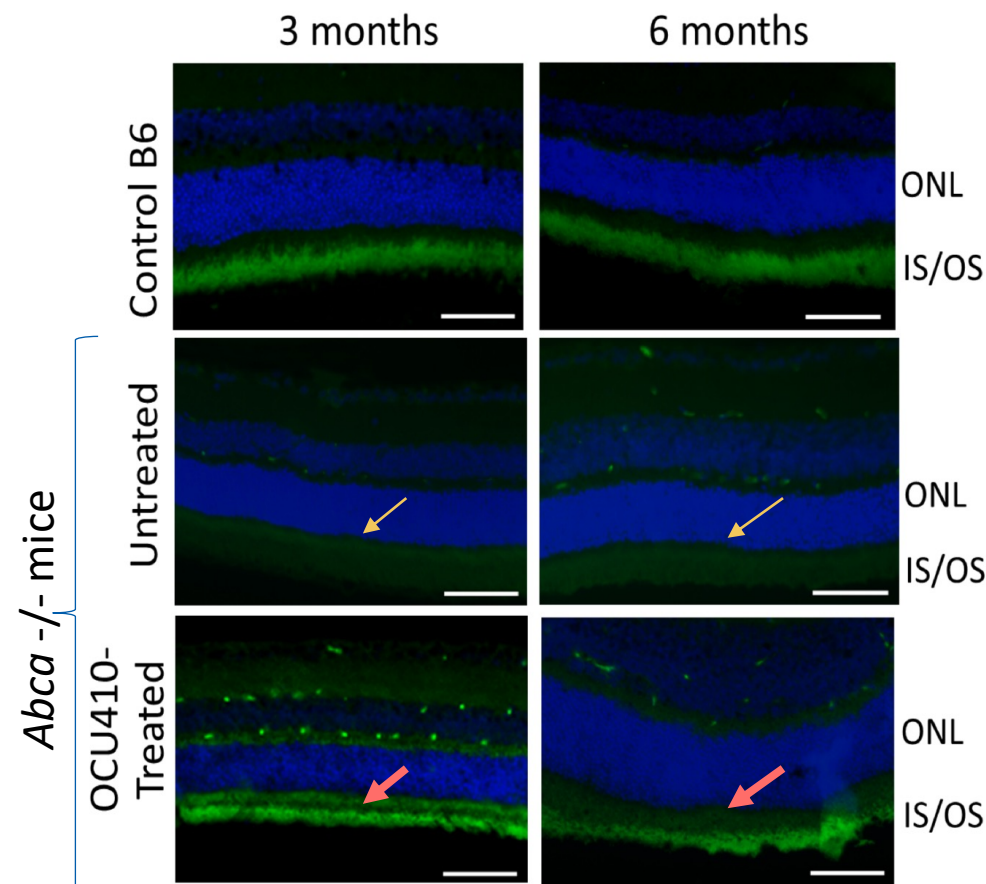
# RORA Regulated Gene Networks are Relevant in AMD & Stargardt Disease

RORA regulated gene networks are relevant in AMD and Stargardt disease



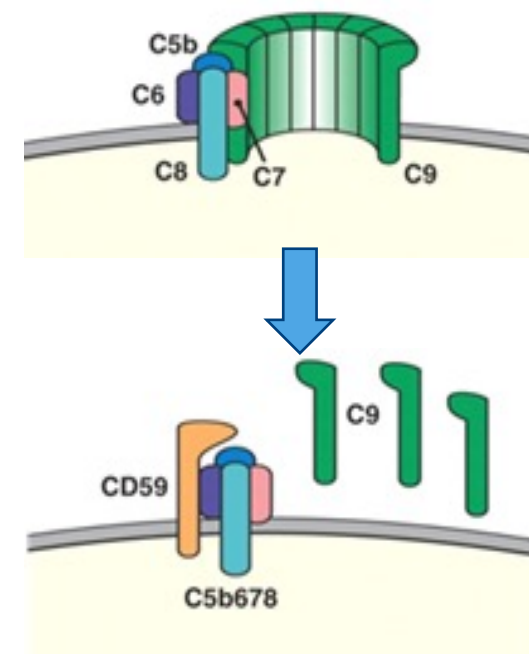
# OCU410 Restores Anti-Complement (Cd59) Expression in *Abca4*<sup>-/-</sup> mice

- Gene variants of ABCA4 are associated with both Stargardt disease and AMD
- Very low CD59 (anti-complement) expression in *ABCA4*<sup>-/-</sup> mice retinas
- OCU410 restored CD59 expression in the RPE cells
- CD59 prevents the formation of the complement membrane attack complex (MAC) and subsequent retina damage



Immunohistochemistry of retina showing CD59 and DAPI

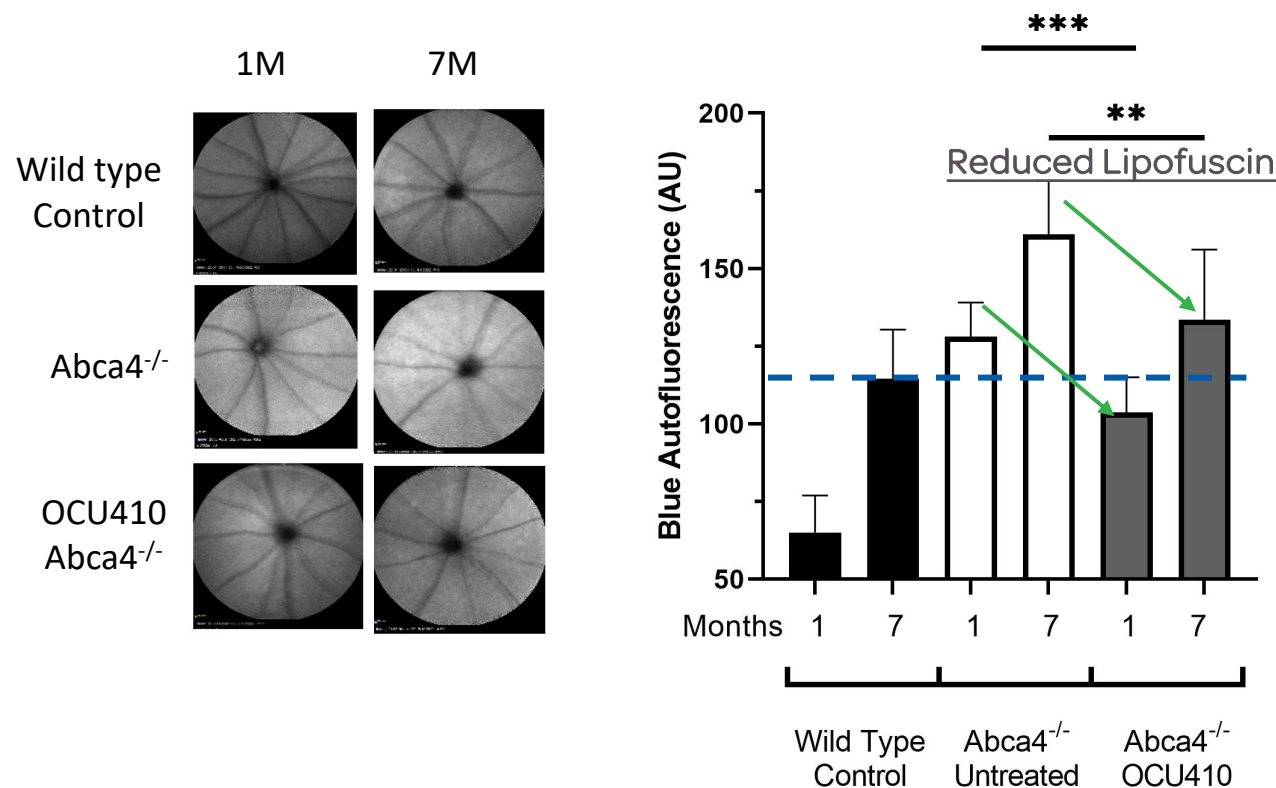
CD59 prevents the formation of the complement membrane attack complex



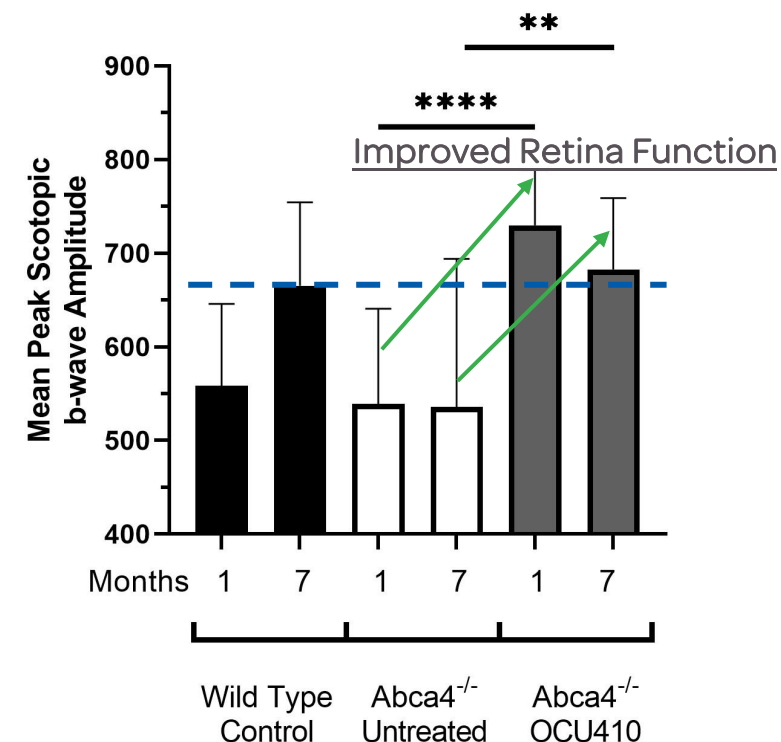


# OCU410 Restoring Retinal Function in *ABCA4* <sup>-/-</sup> Mice

## Blue Autofluorescence (Lipofuscin)



## Electroretinogram (Retina Function)



- OCU410 treatment reduces the age-dependent increase in lipofuscin and improves retina function
- Targeting IND for Geographic Atrophy & Stargardt disease in Q2 2023

# OCU200

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

# OCU200 Potential to Treat DME, DR & Wet AMD

OCU200 Provides hope to ALL patients with DME, DR, or Wet AMD

✓ DME	✓ DR	✓ Wet AMD
~0.7m	~7.7m	~1.1m
patients in the U.S.*	patients in the U.S.*	patients in the U.S.*

~50% of Patients DO NOT Respond to Anti-VEGF/Corticosteroids Therapies

- ✓ OCU200 is a Transferrin-Tumstatin Fusion Protein
  - Tumstatin: Multiple Mechanisms of Action (MOAs) for treatment and prevention of macular edema and neovascularization
  - Transferrin: Targets the site of action and improves uptake (better target engagement)
- ✓ Integrin Targeting provides hope to these patients who are non-responders to current therapies
- ✓ Distinct MOA through targeting Integrin pathways can potentially also help reduce number of injections for patients who do respond to Anti-VEGF & corticosteroids therapies
- ✓ We are executing IND-enabling studies and plan to submit an IND application in the first quarter of 2023

# NeoCart®

(Autologous chondrocyte-derived neocartilage)

# NeoCart® Regenerative Cell Therapy

*Knee injury increases risk of developing OA by more than 5x*

*1MM+ annual arthroscopic procedures of the knee\**

Phase 3 Clinical Trial Planned to Begin Early 2024

## Attributes:

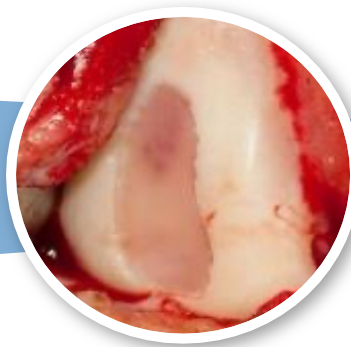
- Designated by FDA as “Regenerative Medicine Advanced Therapy”
- Combines breakthroughs in bio-engineering and cell processing to enhance the autologous cartilage repair process
- Merges a patient’s own cells with a fortified 3-D scaffold designed to accelerate healing and reduce pain
- Patients receive functional cartilage at the time of treatment



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



Initial Lesion



Time Zero Implantation



8 Weeks



6 Months



# 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**





February 2023  
NASDAQ: OCGN