



Courageous  
Innovation

August 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 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 and larger blindness diseases with unmet need
- **Differentiator:** “master gene regulator”; gene-agnostic approach
- **Pipeline:**
  - OCU400 (Ph1/2): RP & LCA; orphan drug designation from FDA/EMA
    - Ph3 target: late 2023/early 2024
  - OCU410 (IND cleared for Ph1/2): dry AMD
  - OCU410ST (IND cleared for Ph1/2): Stargardt

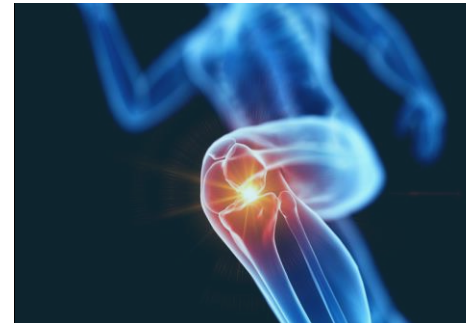


## Inhalation Vaccines Platform *First-in-Class*

- **Therapeutic Focus:** flu and COVID-19
- **Differentiator:** inhalation for improved durability and transmission control
- **Pipeline:**
  - OCU500 (Preclin): COVID-19 bivalent
  - OCU510 (Preclin): flu quadrivalent
  - OCU520 (Preclin): COVID-19 + flu combo

## 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





# Pipeline Overview

	Asset/Program	Indication	Current Status
Gene therapies	OCU400 * AAV-hNR2E3 Gene mutation-associated retinal degeneration	Retinitis pigmentosa (RP)--NR2E3 Mutation	<ul style="list-style-type: none"> <li>Phase 1/2</li> <li>Plan to initiate Phase 3 trial late 2023/early 2024</li> <li>Favorable safety and tolerability profile</li> <li>Initial clinical data from low- and medium-dose cohorts indicates positive trend in Multi-luminance mobility testing and Best-Corrected Visual Acuity scores for OCU400 treated eyes</li> </ul>
		RP--RHO Mutation	
		Leber congenital amaurosis (LCA)--CEP290 Mutation	
	OCU410 AAV-hRORA	Dry Age-Related Macular Degeneration (Dry AMD)	<ul style="list-style-type: none"> <li>INDs cleared 3Q 2023</li> </ul>
	OCU410ST AAV-hRORA	Stargardt disease (orphan disease)	
Biologics	OCU200 Transferrin – Tumstatin	Diabetic Macular Edema	<ul style="list-style-type: none"> <li>IND submitted. Waiting for FDA clearance before initiating Phase 1 trial.</li> </ul>
		Diabetic Retinopathy	<ul style="list-style-type: none"> <li>IND-ready</li> </ul>
		Wet Age-Related Macular Degeneration (Wet AMD)	<ul style="list-style-type: none"> <li>IND-ready</li> </ul>
Cell therapies (Regenerative Medicine)	NeoCart® (Autologous chondrocyte-derived neocartilage)RMAT**	Treatment of Articular Cartilage Defects in the Knee	<ul style="list-style-type: none"> <li>Phase 3 clinical trial is planned for 2H 2024</li> </ul>
Vaccines	OCU500 Series		<ul style="list-style-type: none"> <li>IND planned for 1Q 2024 (pending government funding)</li> </ul>
	OCU500: COVID-19 (Bivalent)	For Prevention of Disease Caused by COVID-19	
	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

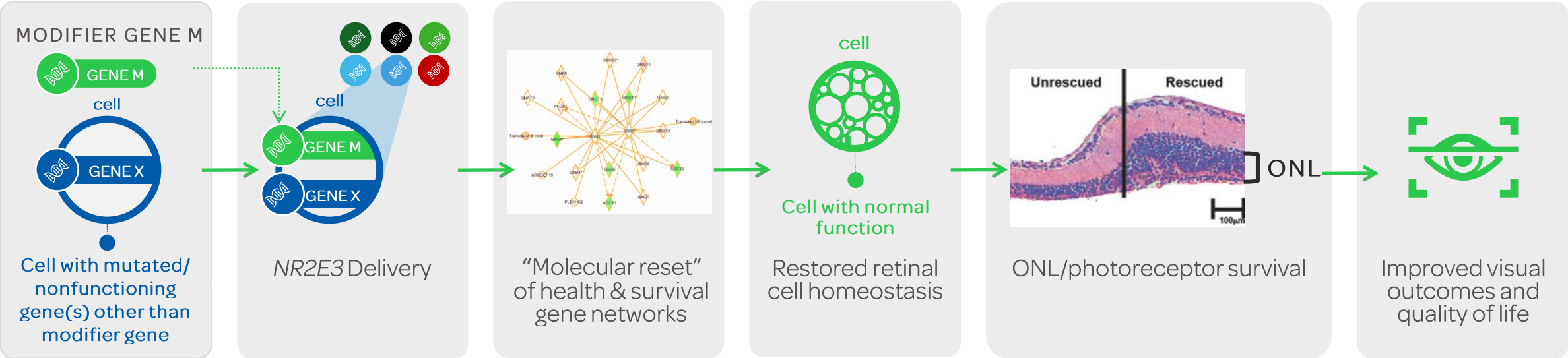
# Modifier Gene Therapy Platform

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

# 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:



# OCU400: Phase 1/2 Clinical Trial Progressing as Planned, Developing a Novel Gene Therapy in Ophthalmic Areas of High Unmet Need

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

Despite its prevalence, RP and LCA patients have 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

Dose escalation and recruitment of RP patients completed

- High dose established as Maximum Tolerable Dose (MTD)
- Continue to enroll patients with LCA
- Intend to initiate a Phase 3 trial late 2023/early 2024



# Study Overview

Primary Endpoint: Safety

Safety of subretinal administration of  
OCU400

Exploratory Endpoint: Efficacy

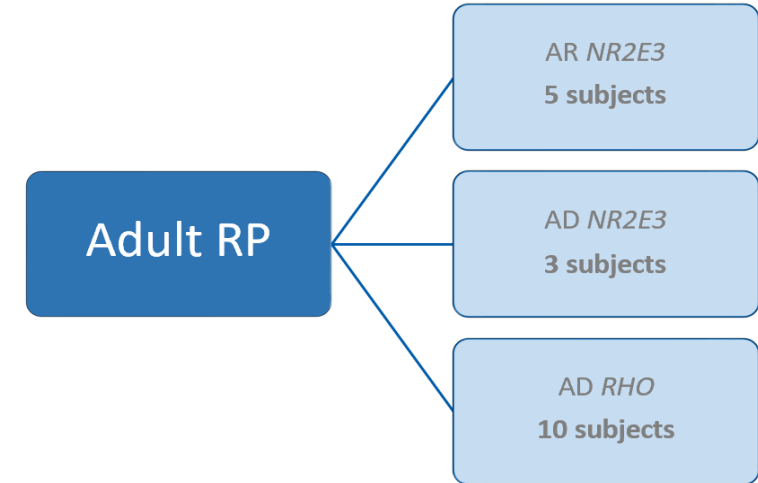
Multi-Luminance Mobility Test (MLMT)

Best Corrected Visual Acuity (BCVA)

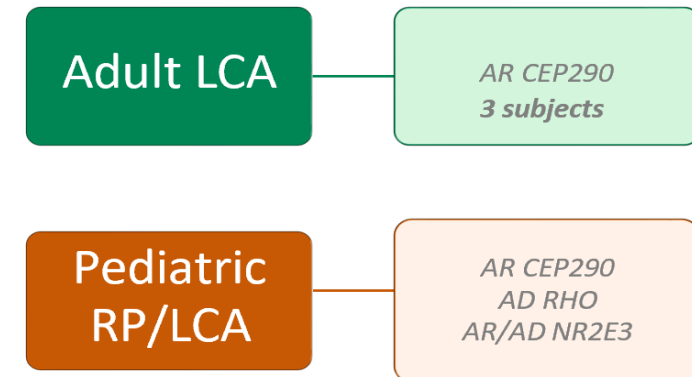
Clinical Trials.gov Identifier: NCT05203939

# Enrollment Status

## COMPLETED



## ENROLLING





Multi-Luminescence Mobility Test			
	Total Subjects for analyses (N=7); pooled analyses Subjects with 9-months follow-up : Cohort 1, N=3 Subjects with 6 months follow-up: N=1 from Cohort 1 and N=3 from Cohort 2		Total Subject for analyses (N=3) Cohort 1 with 9 month follow-up
	Improvement ≥ 1 Lux	Improvement ≥ 2 Lux	Improvement ≥ 2 Lux
Treated Eye	71.4%	28.6%	66.7%
Untreated Eye	28.6%	0.0%	0.0%

- *100% of treated eyes showed stability or improved MLMT scores*
- *71% of treated eyes improved by at least 1 Lux Level in pooled analyses vs ONLY 29% of untreated eyes*
- *29 % of treated eyes improved by at least 2 Lux Level in pooled analyses vs 0 % of untreated eyes*
- *67 % treated eyes improved by at least 2 Lux Level in cohort 1 subjects with 9 months follow up vs 0 % of untreated eyes*

MLMT is used as efficacy measure to assess visual function

LUX LEVEL 400	LUX LEVEL 250	LUX LEVEL 130	LUX LEVEL 50	LUX LEVEL 10	LUX LEVEL 5	LUX LEVEL 1
0	1	2	3	4	5	6
Traditional work office	School classroom	Warehouse aisle	Family living room	Nighttime urban street	Parking lot at night	Full moon night

## Best Corrected Visual Acuity (BCVA) Score

	Total Subjects for analyses (N=7) Subjects with 9-months follow-up : Cohort 1, N=3 Subjects with 6 months follow-up: N=1 from Cohort 1 and N=3 from Cohort 2
	Improvement ≥ 8 Letters
Treated Eye	42.9%
Untreated Eye	0.0%

# OCU400: Expected Pathway to Clinical Development & Potential Approval

- Ocugen plans to meet with regulatory agencies in 3Q to potentially finalize Phase 3 clinical program and overall package
- Continuing to enroll LCA and pediatric patients in Phase 1/2 trial



Both FDA & EMA granted broad orphan drug designation for RP & LCA

# OCU410ST: Received ODD for *ABCA4*-Associated Retinopathies: Stargardt, Retinitis Pigmentosa 19(RP19) & Cone-rod Dystrophy 3(CORD3)

## *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, 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)

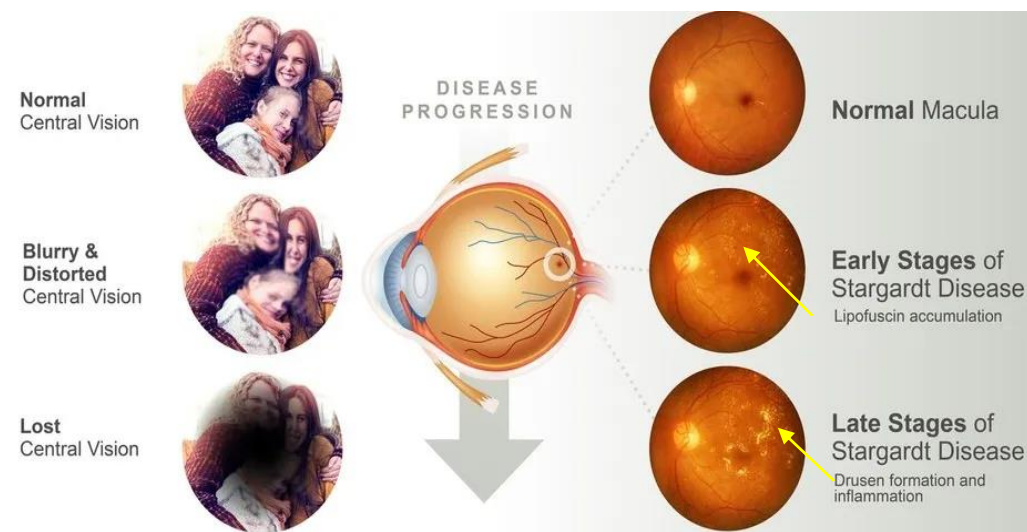
## No treatment options exist

- US: 44,000 patients

## Modifier gene therapy platform addresses shortcomings of current approaches

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

IND application to initiate a Phase 1/2 trial was cleared by the FDA and the Company plans to initiate the Phase 1/2 trial by the end of 2023



# OCU410 for the Treatment of Dry Age-related Macular Degeneration (dAMD)

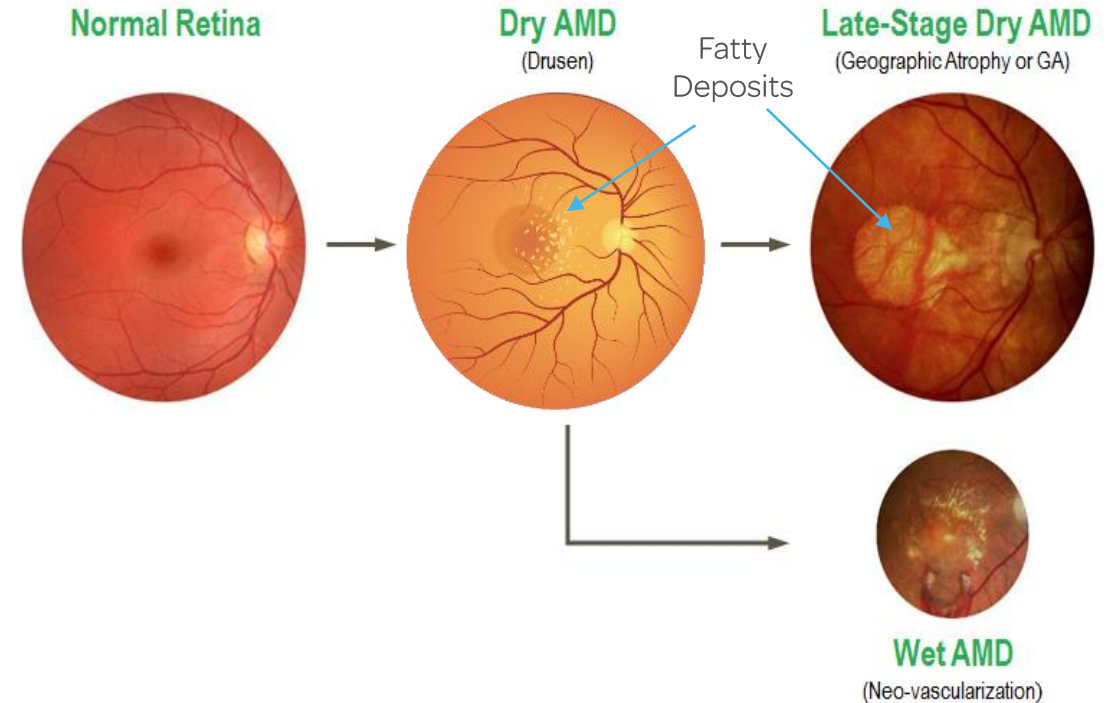
Limited options for AMD, presenting significant unmet medical need

- US: 10M
- Worldwide: condition affects more than 266M people

Recently approved therapy for geographic atrophy (GA)—advanced form of dAMD—has limitations

- Frequent intravitreal injections (N ~6-12 doses per year); Patient compliance
- Limited effect of GA lesion growth rate
- Approximately 12% of patients experience neovascular AMD when the drug is administered every month for two years

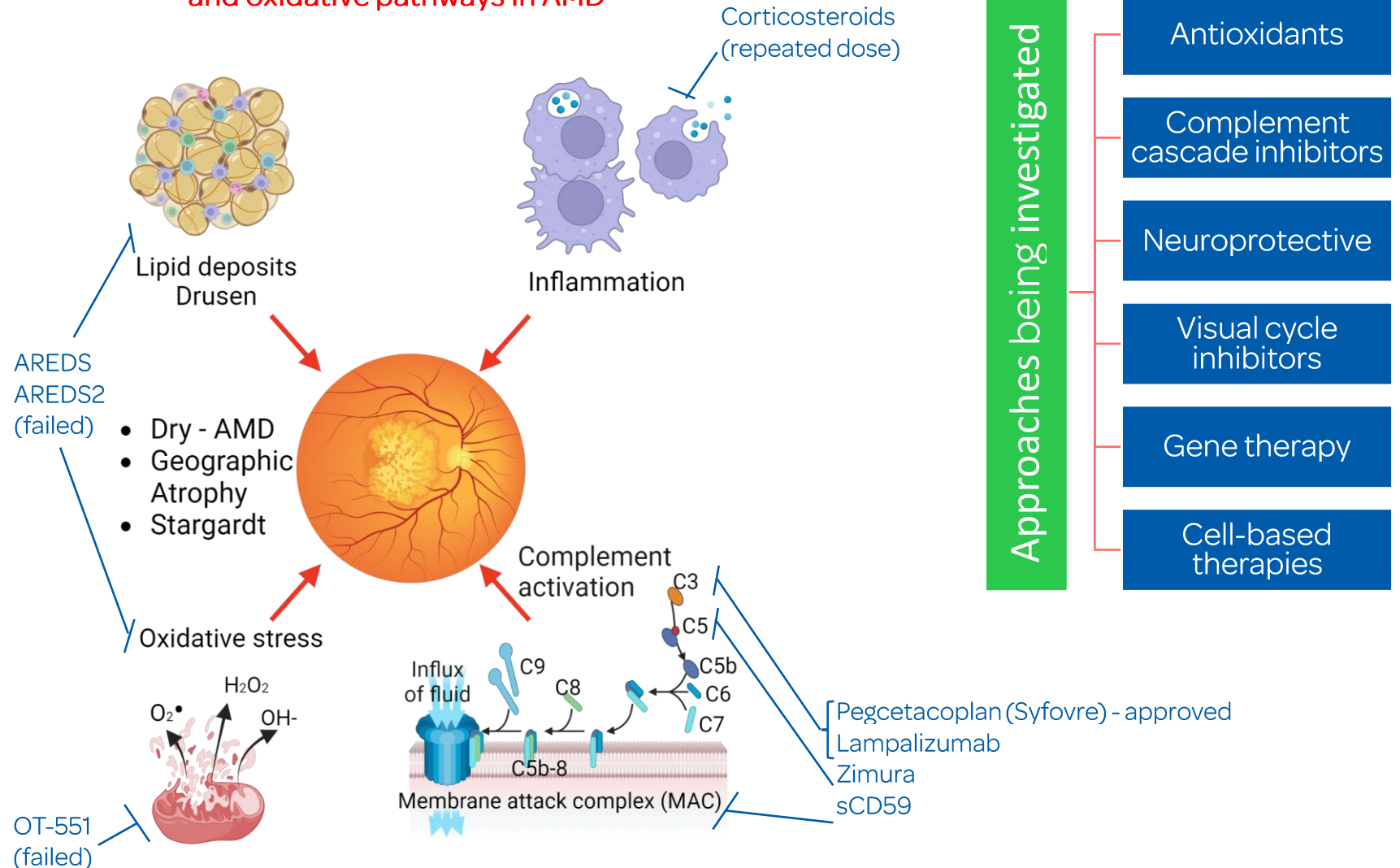
IND application to initiate a Phase 1/2 trial was cleared by the FDA and the Company plans to initiate the Phase 1/2 trial by the end of 2023





# AMD: Risk Factors, Treatment Options and Unmet Needs

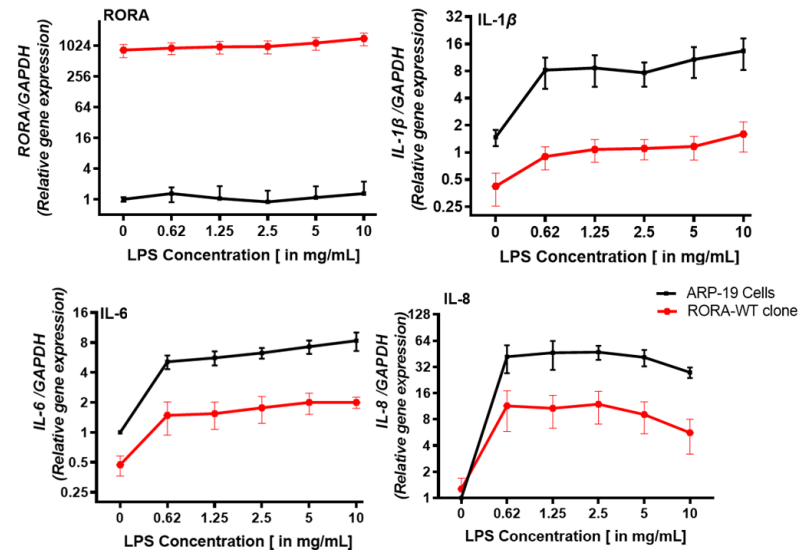
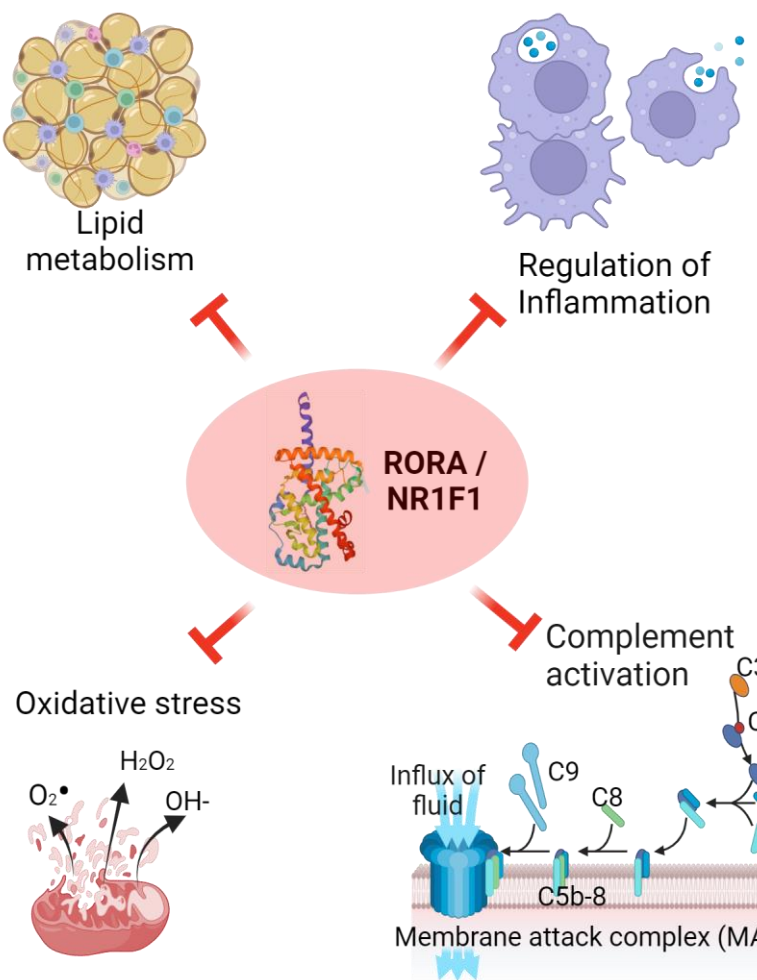
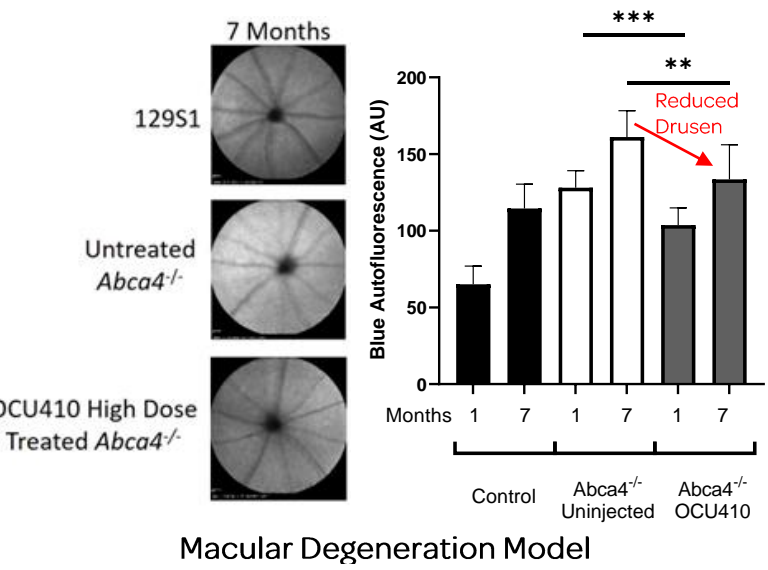
A strong role of inflammation, complement, and oxidative pathways in AMD



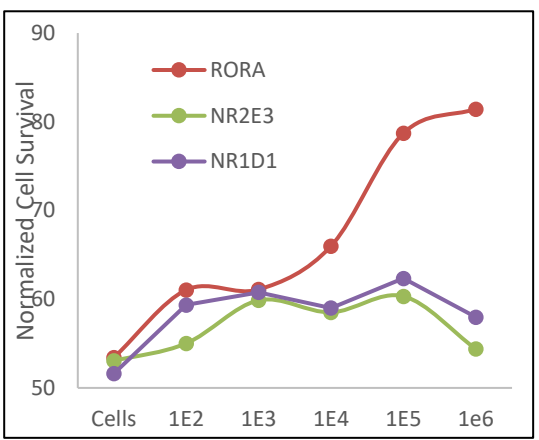
# OCU410 (RORA): A Potential Modifier Therapeutic for Dry-AMD and STGD

Anti-drusen activity and improves retinal function

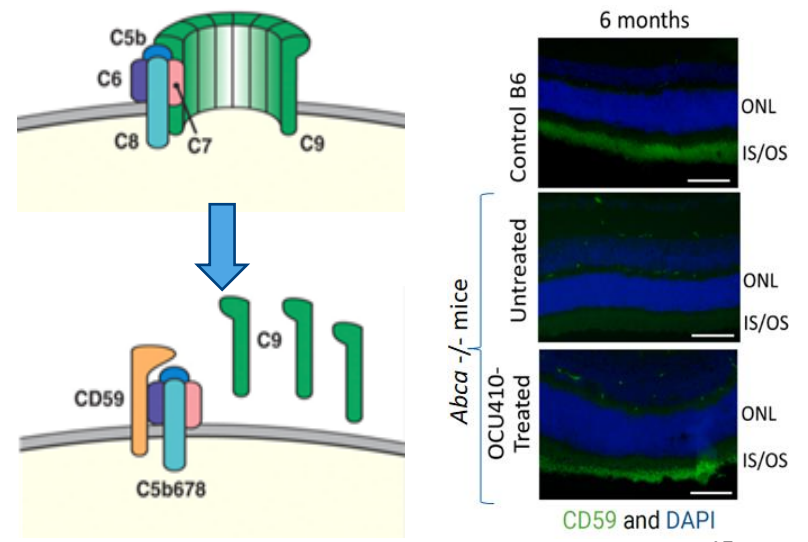
Anti-inflammatory: Suppresses inflammation in HMC3 cells



Anti-oxidative: Improves ARPE19 cells survival



Anti-complement: Increased anti-complement (*Cd59*) protein



# OCU200

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

# OCU200: Submitted an IND with the U.S. FDA to Initiate a Phase 1 Clinical Trial Targeting Diabetic Macular Edema (DME)

OCU200 is our novel biologics candidate for sight-threatening conditions

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

High prevalence of DME, DR and wet AMD patients

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

Limited treatment options available for the above patients

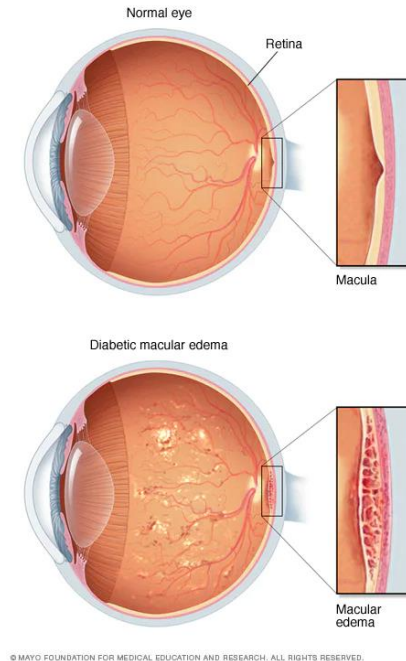
- Current therapies target only one pathway, either angiogenesis or inflammation
- Up to 50% of patient population are not responsive to current treatments

OCU200 potentially addresses shortcomings of current treatments

- Intended to target multiple causative pathways such as angiogenesis, oxidation, inflammation
- Potential to offer better treatment options for *all* patients

Company submitted an IND application on February 27, 2023\*

- Initially targeting DME



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

# 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 technology

- 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 (initiate in 2H 2024)
- Renovating facility to accommodate cGMP manufacturing

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



Initial Lesion



Time Zero Implantation



8 Weeks



6 Months

# OCU500 Series:

OCU500: COVID-19 Mucosal Vaccine  
OCU510: Flu  
OCU520: COVID-19/Flu

# OCU500 Series: Next-Generation Vaccine Technology

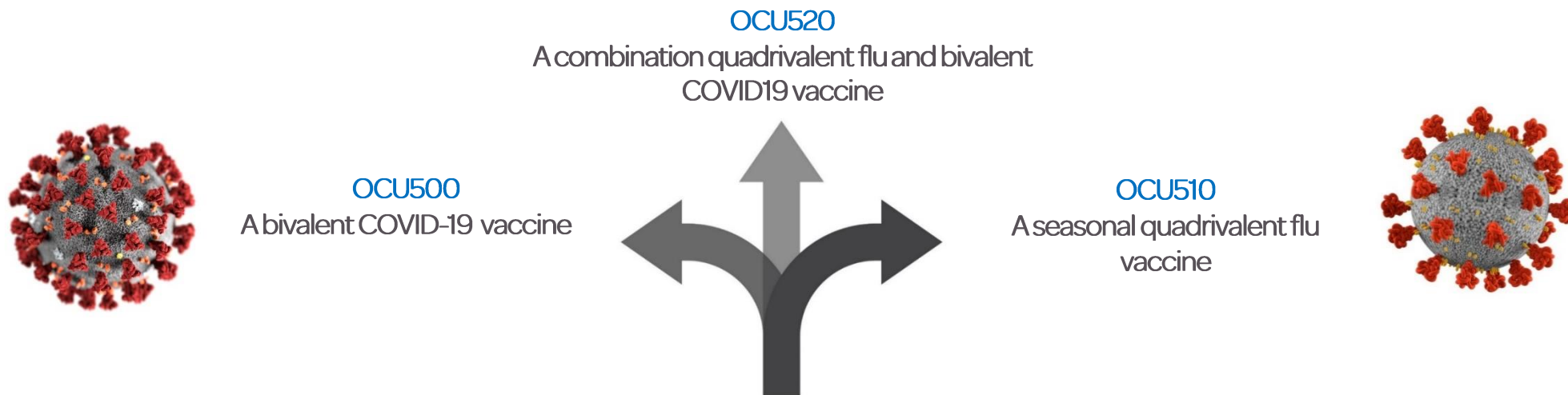
## *Inhaled mucosal vaccine platform based on ChAd vector*

### 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
- Multiple proposal submissions for federal funding of Ocugen's inhaled vaccines platform for COVID-19 and flu
- Ongoing dialogue with several government agencies regarding the development of the inhaled vaccines platform



# 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**







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