



Courageous
Innovation

October 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.

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

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 (Ph1/2): dry AMD
 - OCU410ST (Ph1/2): Stargardt

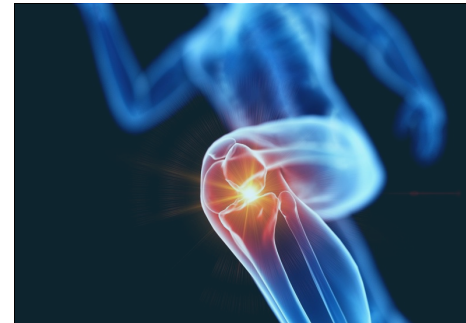


Inhalation Vaccines Platform *First-in-Class*

- **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	<i>Retinitis pigmentosa (RP)--NR2E3 Mutation</i>	<ul style="list-style-type: none"> Phase 1/2 Completed recruitment of RP patients Plan to initiate Phase 3 trial late 2023/early 2024 subject to FDA concurrence
		<i>RP--RHO Mutation</i>	
		<i>Leber congenital amaurosis (LCA)--CEP290 Mutation</i>	
	OCU410 AAV-hRORA	Dry Age-Related Macular Degeneration (Dry AMD)	<ul style="list-style-type: none"> Phase 1/2
	OCU410ST AAV-hRORA	Stargardt disease (orphan disease)	
Biologics	OCU200 Transferrin – Tumstatin	Diabetic Macular Edema	<ul style="list-style-type: none"> IND submitted. Waiting for FDA clearance before initiating Phase 1 trial.
		Diabetic Retinopathy	<ul style="list-style-type: none"> IND-ready
		Wet Age-Related Macular Degeneration (Wet AMD)	<ul style="list-style-type: none"> IND-ready
Cell therapies (Regenerative Medicine)	NeoCart® (Autologous chondrocyte-derived neocartilage) RMAT***	Treatment of Articular Cartilage Defects in the Knee	<ul style="list-style-type: none"> Phase 3 clinical trial is planned for 2H 2024
Vaccines	OCU500 Series		
	OCU500: COVID-19 (Bivalent)	For Prevention of Disease Caused by COVID-19	<ul style="list-style-type: none"> OCU500 IND planned for 1Q 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

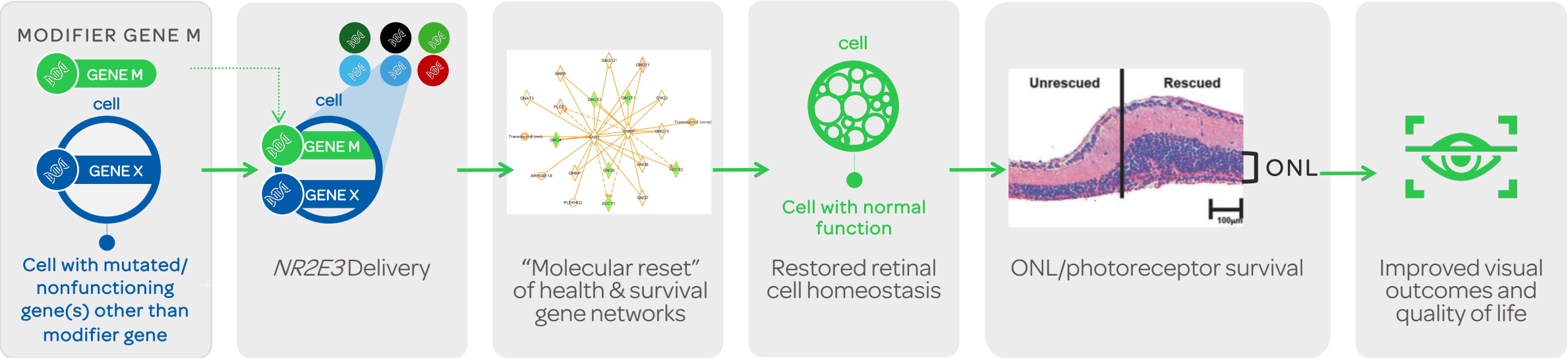
Modifier Gene Therapy Platform

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

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 subject to FDA concurrence



OCU400 Clinical Program

A 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 mutation(s) in *CEP290* gene

Primary: Safety

Safety of subretinal administration of OCU400

Immune responses

Systemic Distribution

Exploratory: Efficacy

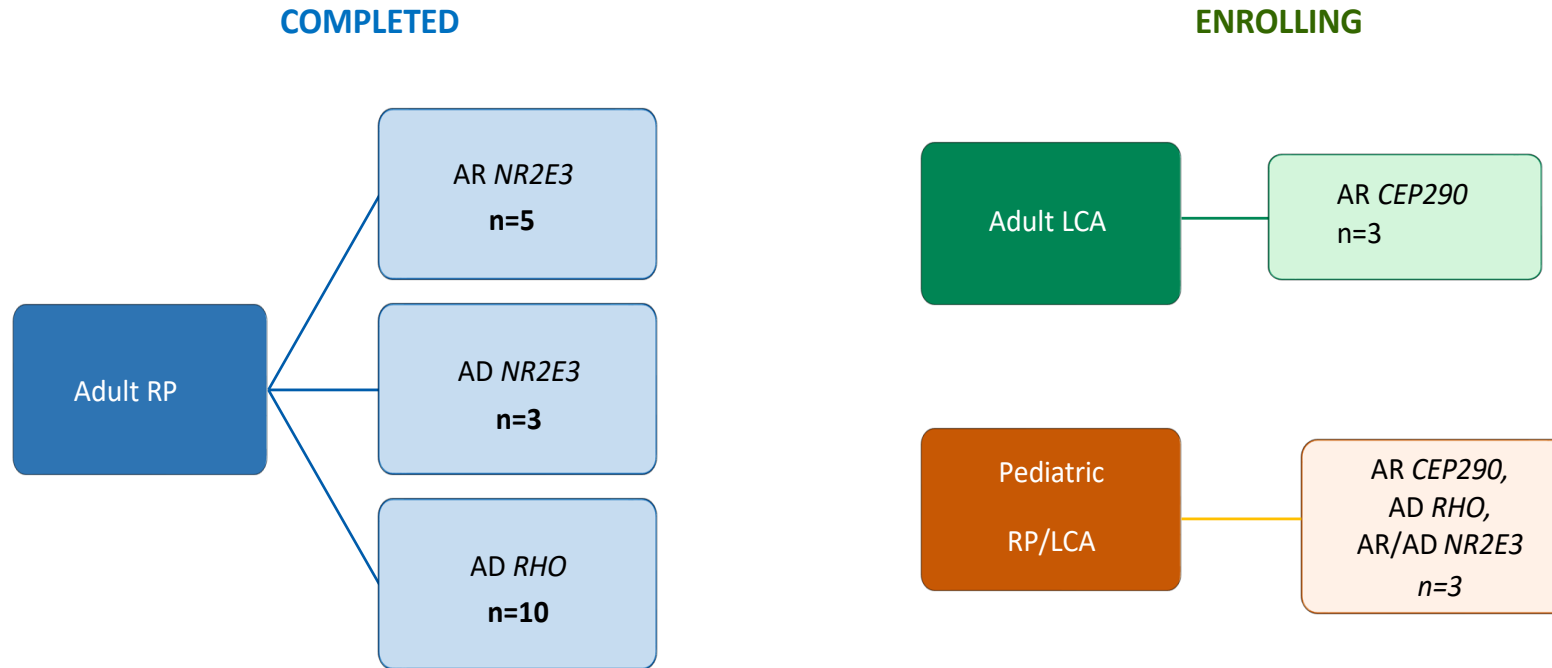
Best Corrected Visual Acuity (BCVA)

Low Luminance Visual Acuity (LLVA)

Multi-Luminance Mobility Test (MLMT)

Clinical Trials.gov Identifier: [NCT05203939](#)

Enrollment Status



- Subjects (N=18) with ages ranging 18-77 years enrolled in this study to date
- Consists of 10 *RHO* subjects, 5 Autosomal Recessive *NR2E3* and 3 Autosomal Dominant *NR2E3* subjects
- Sub-retinal injection was mostly around the central region (and few subjects with central temporal, superior-temporal regions)

Safety Summary for OCU400–Clinical Study Update

- The Phase 1/2 clinical trial demonstrated that OCU400 continued to be generally safe and well-tolerated in subjects across different mutations and dose levels
- There were no serious adverse events (SAEs) related to the investigational product in the low and medium dose cohorts
- In the high dose and open-enrollment cohorts, SAEs were reported for two subjects. None of them were related to the study drug.
- Adverse events were mostly deemed related to the surgical procedure and resolved within a few days to weeks

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
- *Stabilization 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 could ultimately lead to vision restoration.
- Comprehensive care, early diagnosis, and access to emerging therapies are essential components of a *strategy to stabilize vision in RP and LCA patients*

Responder Analyses

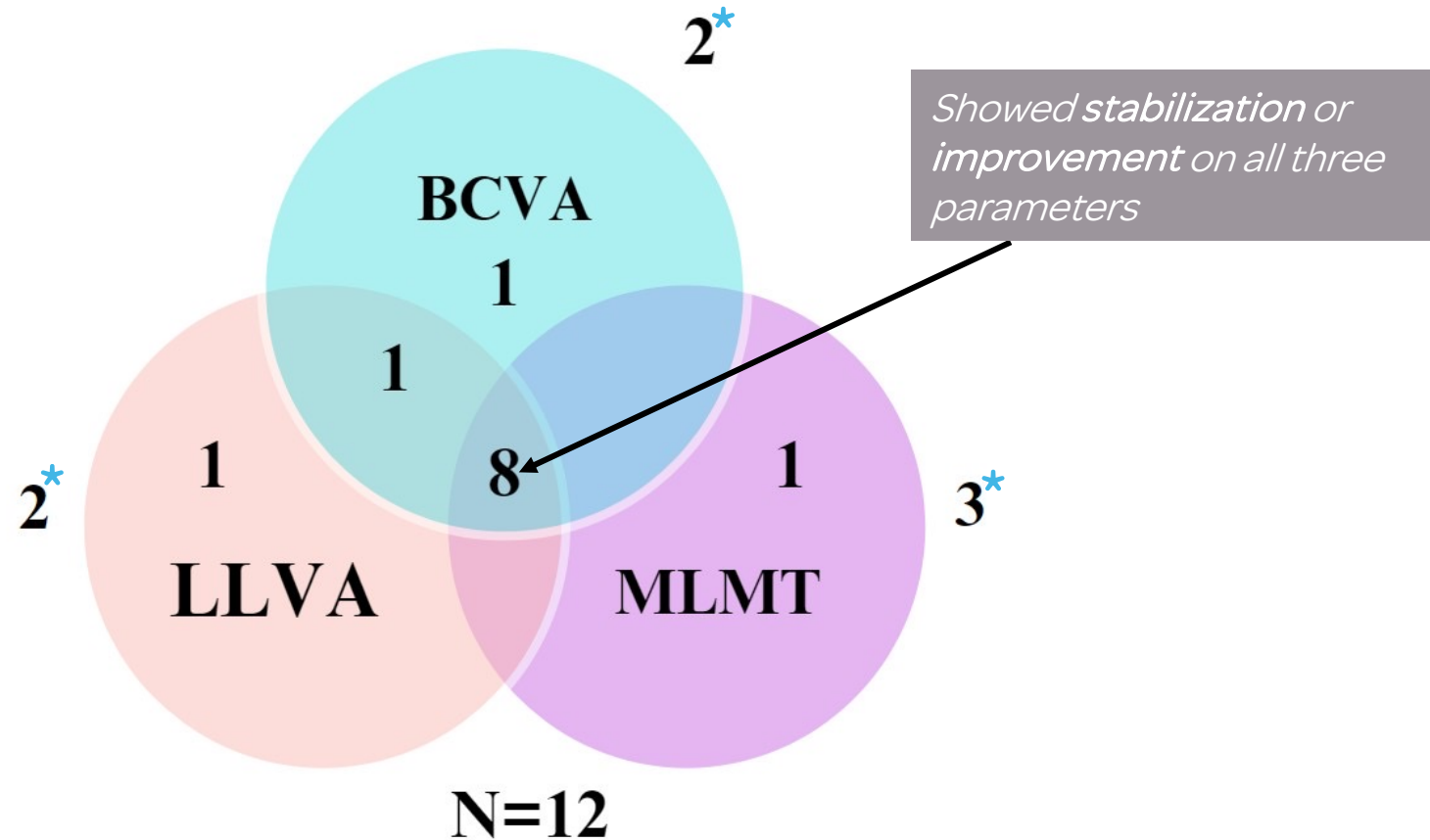
Assessed for subjects who have completed a minimum of 6-months follow-up post-OCU400 dosing

- Improvement in Best-Corrected Visual Acuity (BCVA) from Baseline
 ≥ 4 Letters, and ≥ 7 Letters
- Improvement in Low Luminance Visual Acuity (LLVA) from Baseline
 ≥ 5 Letters and ≥ 10 Letters
- Improvement in Multi-Luminance Mobility Test (MLMT) from
 ≥ 3 Lux Levels, ≥ 1 Lux Level, ≥ 0 Lux Level/Ceiling Effect

LUX LEVEL 400	LUX LEVEL 250	LUX LEVEL 130	LUX LEVEL 50	LUX LEVEL 10	LUX LEVEL 5	LUX LEVEL 1	LUX LEVEL .1
0	1	2	3	4	5	6	7
Traditional work office	School classroom	Warehouse aisle	Family living room	Nighttime urban street	Parking lot at night	Full moon night	New moon night

Responder Analysis

Stabilization/Improvement from Baseline [Treated Eyes]



* Non responder

Conclusions from Latest Clinical Study Results

- OCU400 continues to demonstrate a favorable safety and tolerability profile
- Clinical study update suggests *continued positive trends* in Best-Corrected Visual Acuity (BCVA) and Multi-Luminance Mobility Testing (MLMT), as well as positive trends in **Low-Luminance** Visual Acuity (LLVA) among treated eyes
- 83% (10/12) of subjects demonstrated *stabilization or improvement* in the treated eye either on *BCVA or LLVA or MLMT* scores from baseline
- 75% (9/12) of subjects *demonstrated stabilization or improvement* in treated eyes in MLMT scores from baseline
- 86% (6/7) of *RHO* mutation subjects experienced *either stabilization or improvement in MLMT scores* from baseline, among which 29% (2/7) demonstrated **3 Lux** luminance level improvement
- Treatment effect in *RHO* patients supports the *gene-agnostic* mechanism of action of OCU400

OCU400: Expected Pathway to Clinical Development & Potential Approval

- Ocugen plans to meet with regulatory agencies in 4Q 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

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

- OCU410 Phase 1/2 study currently underway
- 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*

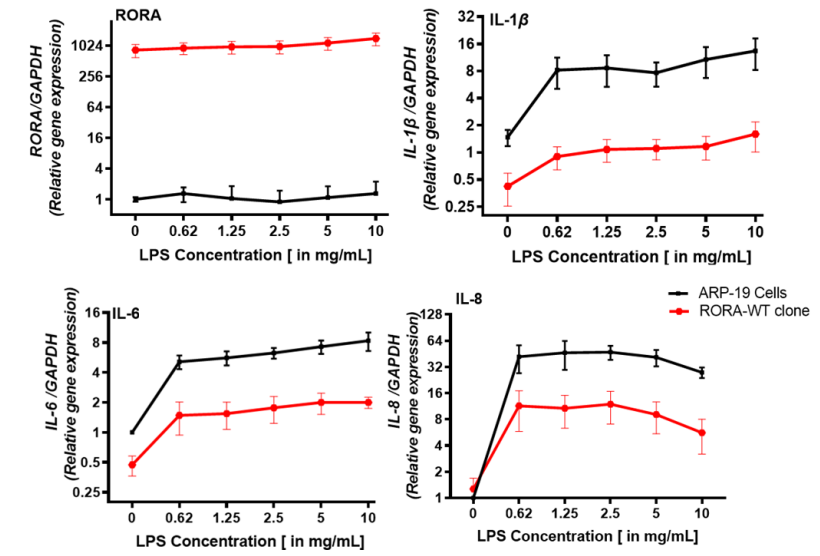
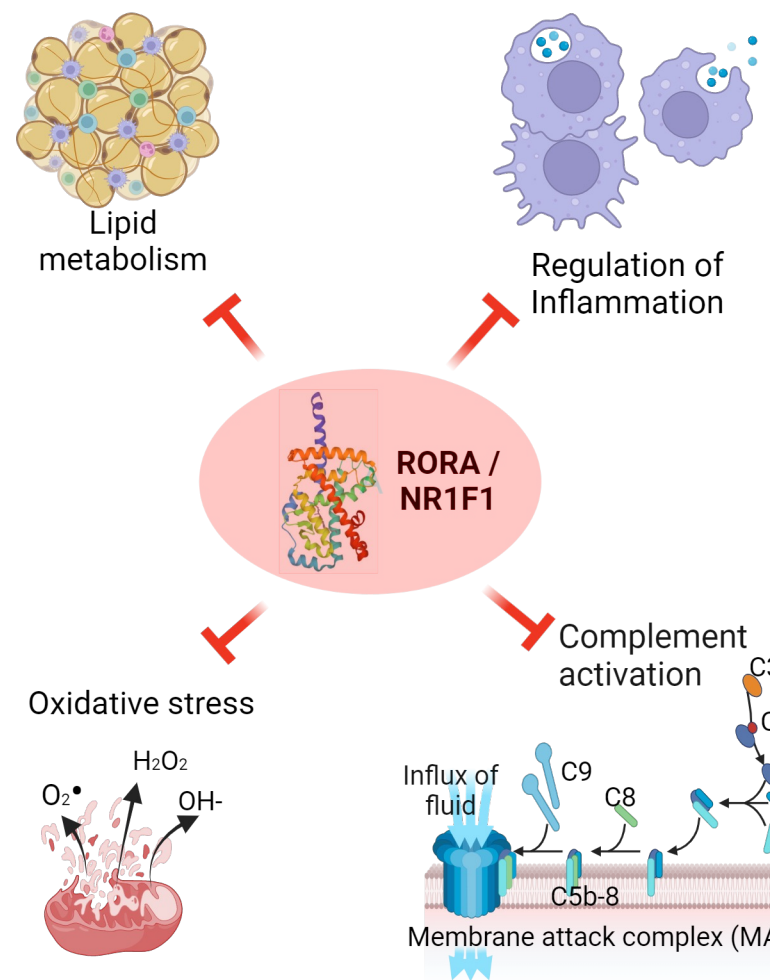
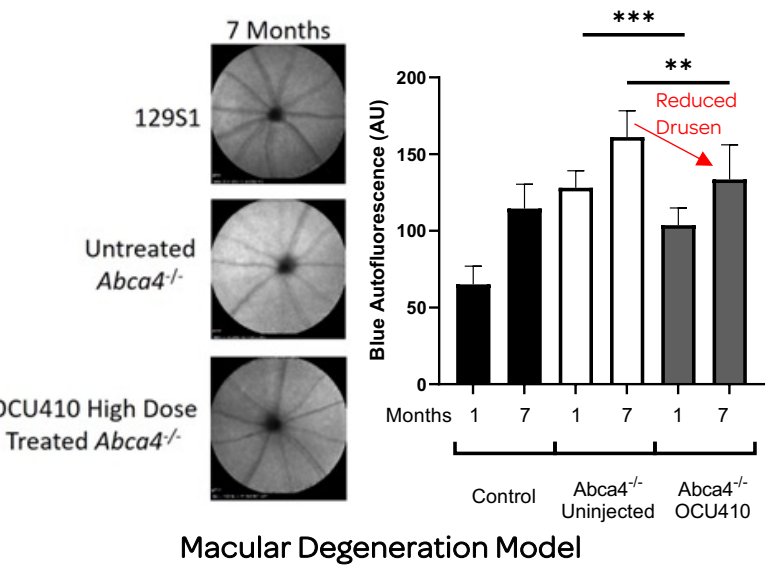
- Advancement from recently approved therapies for GA: Potential to address limitations of recently approved therapies for GA focused only on the complement system, including:
 - Patient Compliance
 - Frequent intravitreal injections (~6-12 doses per year)
 - Observed Structural Impact
 - Limited effect 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

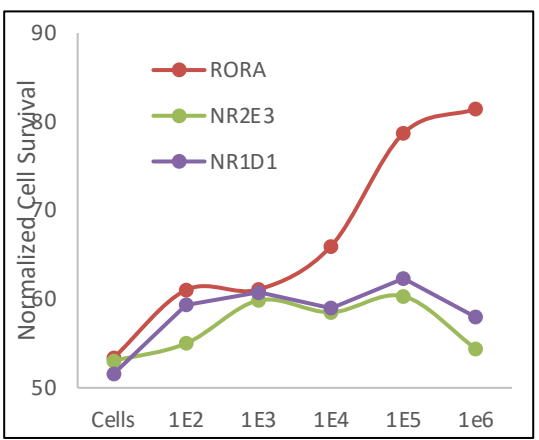
OCU410 (RORA): A Potential Modifier Therapeutic for Dry-AMD and STGD

Anti-drusen activity and improves retinal function

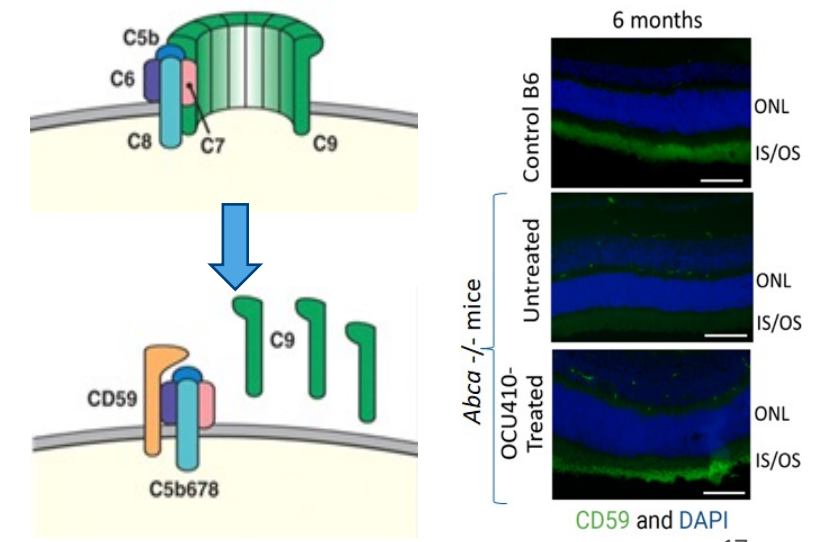
Anti-inflammatory: Suppresses inflammation in HMC3 cells



Anti-oxidative: Improves ARPE19 cells survival



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



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, 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)

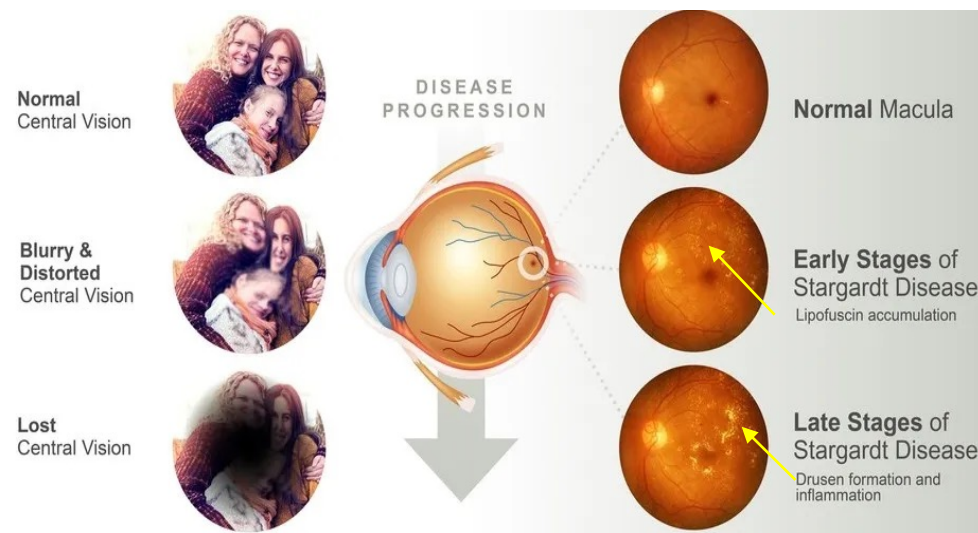
No treatment options exist

- U.S.: 44,000 patients

Modifier gene therapy platform addresses shortcomings of current approaches

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

Phase 1/2 underway



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

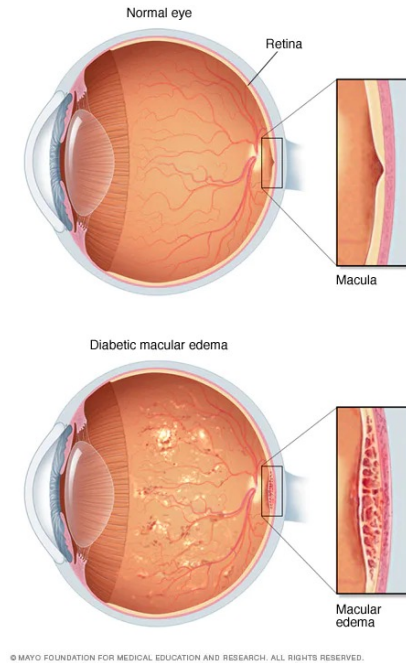
- Current therapies target only one pathway, either angiogenesis or inflammation
- Up to 50% of patient population are not responsive to current treatments

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

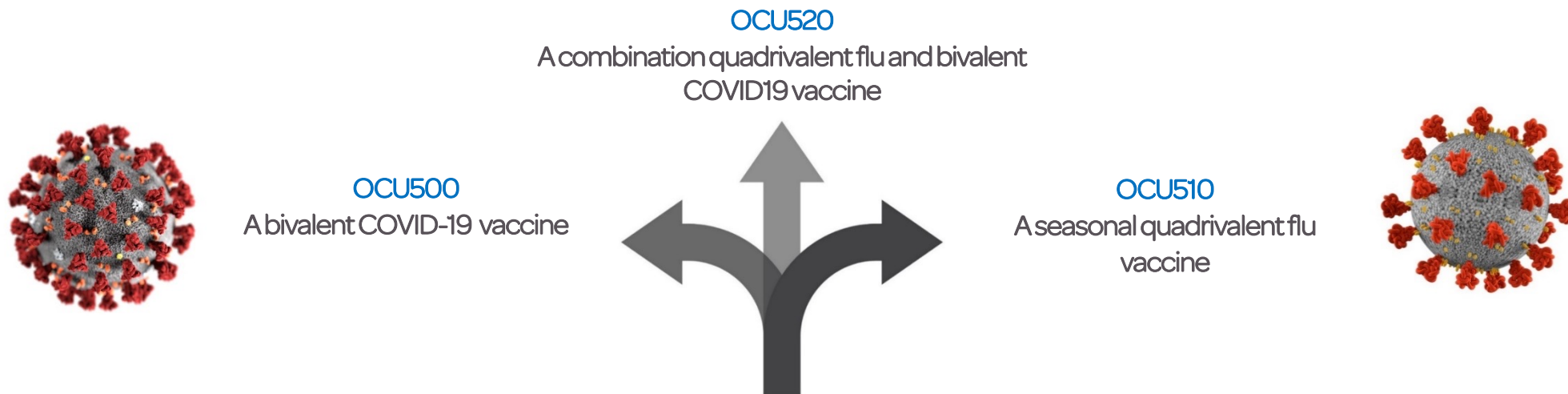
Inhaled mucosal vaccine platform based on ChAd vector

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
- Announced on October 10, 2023, that OCU500 was selected by the NIH/NIAID Project NextGen for inclusion in clinical trials



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**





October 2023
NASDAQ: OCGN