

## Our Mission is to

# Develop Gene Therapies to Cure Blindness Diseases

### and

# Develop a Vaccine to Save Lives from COVID-19



Corporate Deck: July 2021

### **Forward Looking Statement**

This presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including statements regarding our business strategy, future results of operations and financial position, prospective products, product approvals, research and development costs, timing and likelihood of success, estimated market size or growth, and plans and objectives of management for future operations, are forward-looking statements. When used in this presentation, the words "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

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# Ocugen Overview

	<ul> <li>COVAXIN<sup>™</sup>: Whole-virion inactivated COVID-19 vaccine candidate (with adjuvant). Licensed rights from Bharat Biotech for the US and Canadian markets (currently received EUA in India). Standard vaccine storage condition (2-8°C)</li> </ul>
	<ul> <li>Demonstrated 77.8% overall efficacy, 93.4% in severe COVID-19 disease (including hospitalization) and 65.2% efficacy against Delta variant in Phase 3 trial by Bharat Biotech</li> </ul>
VACCINE	<ul> <li>Phase 3 clinical trial enrolled 25,800 participants between 18-98 years of age, including 2,760 over the age of 60 and 7,058 with at least one pre-existing condition. Phase 1/2 enrolled 755 participants</li> </ul>
	<ul> <li>Potential coverage against multiple protein antigens of the virus and potentially applicable to broader population, including 12– 17-year-olds (as seen in Phase 2 study)</li> </ul>
	• Effectively neutralizes additional Kappa, Zeta, and Alpha variants of SARS-Cov-2, reducing the possibility of mutant virus escape
	• Potential for one product to treat many diseases & multi-factor approach (POC study results published in Nature)
	<ul> <li>OCU400 (AAV-hNR2E3): Orphan medicinal product designation for the treatment of both retinitis pigmentosa (RP) and Leber Congenital amaurosis (LCA) covering diseases caused by mutations in over 175 genes. Initiation of Phase 1/2a this year</li> </ul>
GENE THERAPY PLATFORM	<ul> <li>OCU410 (AAV-hRORA): Potential to treat dry age-related macular degeneration (Dry AMD) through multi-factor treatment approach – initiation of Phase 1/2 in 2022</li> </ul>
	<ul> <li>Strategic manufacturing partnership with CanSinoBio (~\$13B market cap) – sets clear path for critical manufacturing</li> </ul>
NOVEL	<ul> <li>OCU200: Targeting major retinal diseases: Diabetic Macular Edema (DME), Diabetic Retinopathy (DR), and Wet Age-Related Macular Degeneration (Wet AMD) (estimated global market size over \$10B) – initiation of Phase 1/2 in 2022</li> </ul>
BIOLOGIC	<ul> <li>Novel MoA: Potential to initially treat non-responders to anti-VEGF/ therapies (~50% of patients)</li> </ul>



### Leadership Team



Shankar Musunuri, PhD, MBA Chairman, CEO and Co-Founder





Sanjay Subramanian, MBA CFO and Head of Corporate Development BAUSCH- Health



Bruce D. Forrest, MB, BS, MD, MBA Acting CMO Wyeth Pfizer



J.P. Gabriel SVP, Manufacturing & Supply Chain

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Vijay Tammara, PhD SVP, Regulatory & Quality



Michael Shine, MBA SVP, Commercial





Arun Upadhyay, PhD VP, Head of Research & Development





Jessica Crespo, CPA Corporate Controller and Treasurer





Zara Gaudioso, SHRM-CP Head of Human Resources



### **Scientific Advisory Boards**

#### **Retina Scientific Advisory Board**

**Vaccine Scientific Advisory Board** 





Carl D. Regillo, MD, FACS

WillsEye Hospital



Mark Pennesi, MD, PhD

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Satish Chandran, PhD





David Fajgenbaum, MD, MBA, MSc, FCPP





Bruce D. Forrest, MB, BS, MD, MBA

Wyeth **Pfizer** 



**Catherine Pachuk, PhD** 





Harvey Rubin, MD, PhD





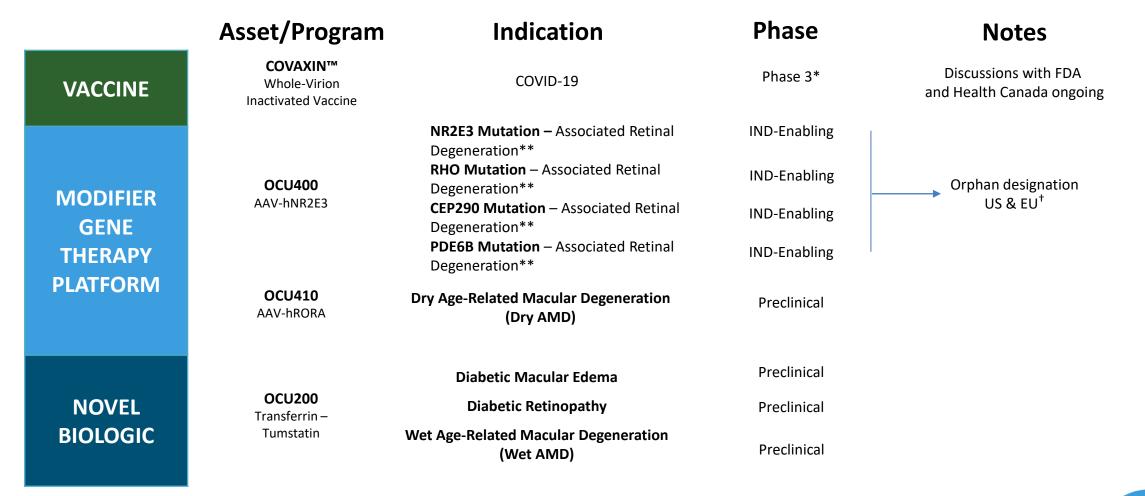
Susan Weiss, PhD

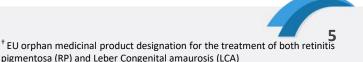






# **Pipeline and Regulatory overview**







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

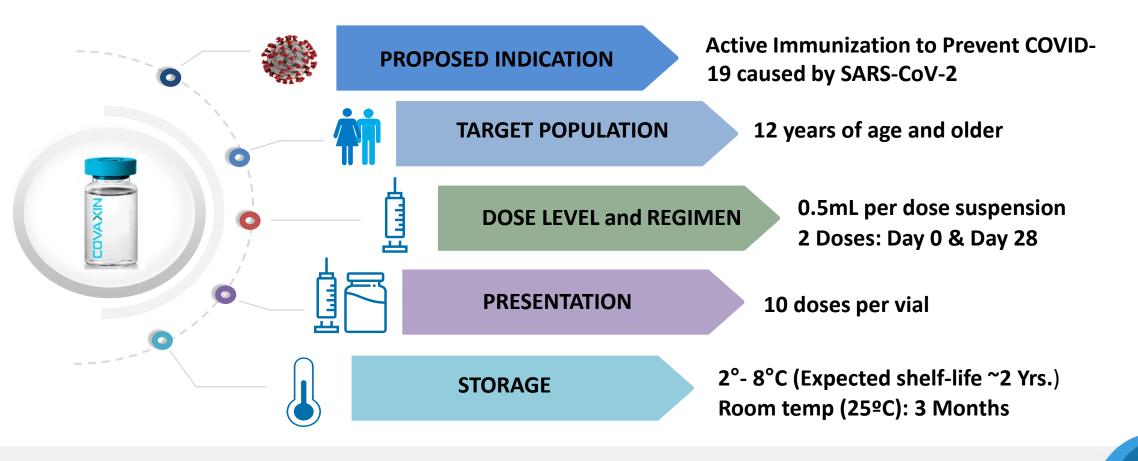
pigmentosa (RP) and Leber Congenital amaurosis (LCA)

# **COVAXIN**<sup>TM</sup>

Whole-Virion Inactivated COVID-19 Vaccine Licensed from Bharat Biotech (BBIL) for the US and Canadian Markets

### **COVAXIN™** - **Product Profile**

### Whole virion inactivated SARS-CoV-2 (NIV-2020-770) Antigen concentration & Adjuvant: 6µg + Algel–IMDG(TLR7/8)





# Why COVAXIN™

Designed to fill a significant unmet need in our North American arsenal of vaccines against COVID-19

В	

### **Broad Spectrum Immune Response**

Both humoral & cellular responses generated against multiple viral proteins Induces a Th1 response (cell-mediated immunity)



#### Efficacy $\rightarrow$ 77.8% Efficacy Demonstrated in Phase 3 Trial (93.4% against severe)

Effective in neutralizing multiple variants, including rapidly-spreading Delta variant (65.2% efficacy) Potentially serve as a universal booster to minimize/eliminate viral escape and control the Pandemic



### Safe in 12+ (Demonstrated in Phase 2 clinical trial)

Proven technology platform and supply chain currently used for several licensed vaccines (Influenza, Polio, Rabies, JEV etc.).

Historically demonstrated acceptable safety, tolerability and efficacy consistent with adults

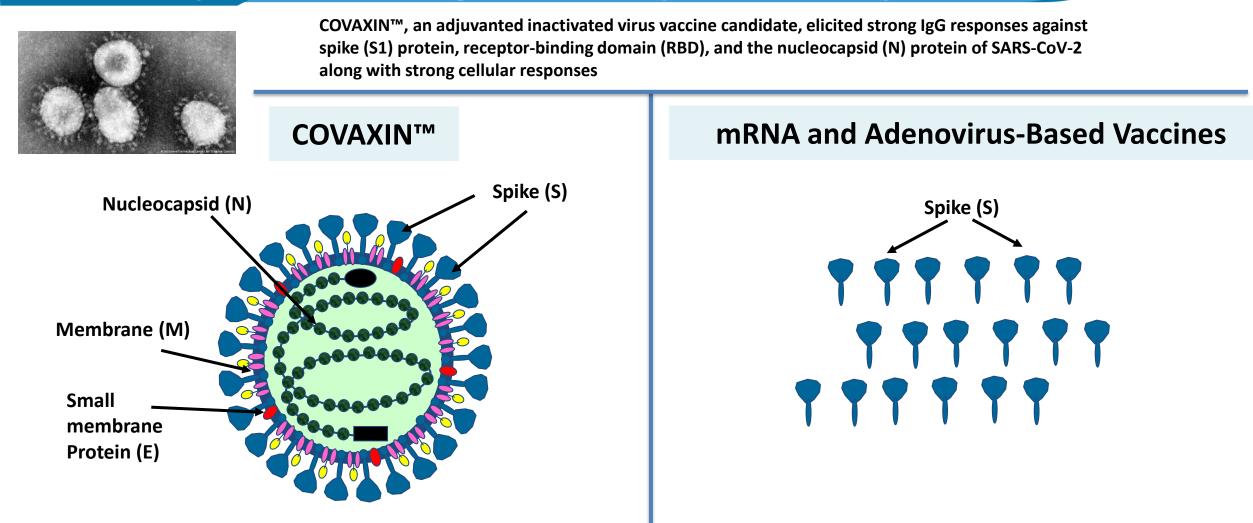


### **Transportation and Storage Ease**

Stable for 3 months at room temperature Can be stored in standard conditions (2°- 8°C) for several years. Can be stockpiled.



# COVAXIN<sup>™</sup> Presents Multiple Protein Targets to the Immune System Resulting in Broad Spectrum Response



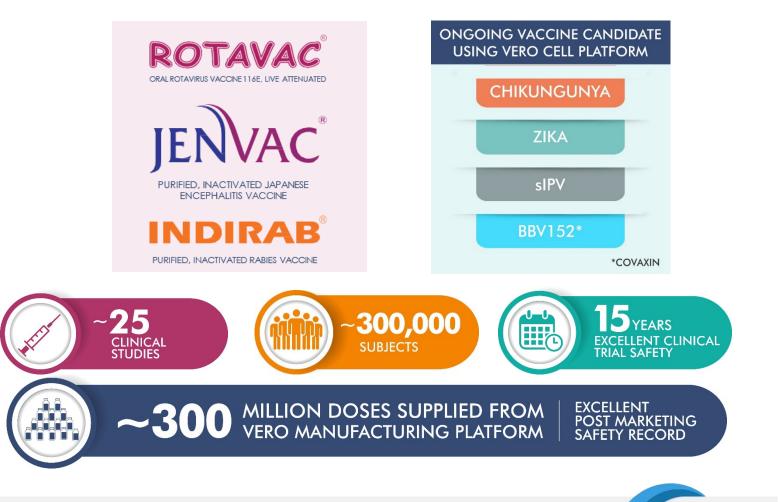


### **COVAXIN™** Developed and Manufactured by Bharat Biotech

#### Established Robust Manufacturing Process for COVAXIN

Ocugen licensed COVAXIN<sup>™</sup> on the back of Bharat's strong track record of developing & commercializing vaccines globally

Inactivated Vero cell derived vaccines are proven, time-tested and longlasting. A few include:



# COVAXIN<sup>™</sup> is Distinct Amongst Leading COVID-19 Vaccines and Select Vaccine Candidates in the United States and Canada

Company	Technology	Antigen	Status in US & Canada
COVAXIN™	Inactivated SARS CoV-2 Virus, Aluminum hydroxide, TLR agonist	Whole virus (Including S & N Proteins)	BLA submission to be pursued in US; regulatory pathway in process in Canada
Pfizer/ BioNTech	Lipoplex of SARS CoV-2 S protein mRNA	S protein	EUA in US; Authorized by Interim Order in Canada
Moderna	Lipoplex of SARS CoV-2 S protein mRNA	S protein	EUA in US; Authorized by Interim Order in Canada
AstraZeneca	Non-replicating infectious Adenovirus	S protein	Authorized by Interim Order in Canada
Johnson & Johnson	Non-replicating infectious Adenovirus	S protein	EUA in US; Authorized by Interim Order in Canada

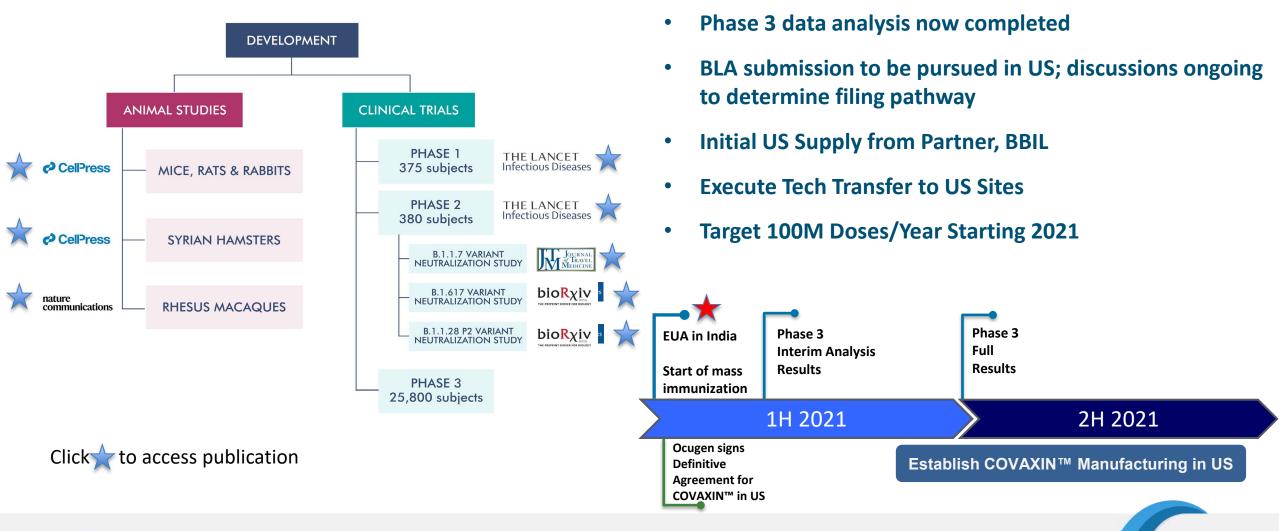


# **Technology Comparisons: Target Product Profile**

Characteristic	mRNA	Adeno- Based	COVAXIN™
Acceptable Safety	$\checkmark$	$\checkmark$	$\checkmark$
Neutralizing antibody response	$\checkmark$	$\checkmark$	√+
Cellular responses against multiple viral antigens	$\checkmark$	$\checkmark$	√+
Efficacy	$\checkmark$	$\checkmark$	√+
Stability at 2-8°C	X	$\checkmark$	$\checkmark$
Multiple Viral Antigens	X	X	$\checkmark$

"+" : B and T cell immune responses to multiple proteins, Safety and Efficacy in Phase 3 clinical trial by Bharat Biotech

# **COVAXIN™** Progress and Planned Milestones for U.S. Dev.



# FINAL Phase 3 Clinical Trial Results Demonstrate Protective Effect of COVAXIN™

Fast facts of a double-blind, randomized, multicenter, Phase 3 clinical trial

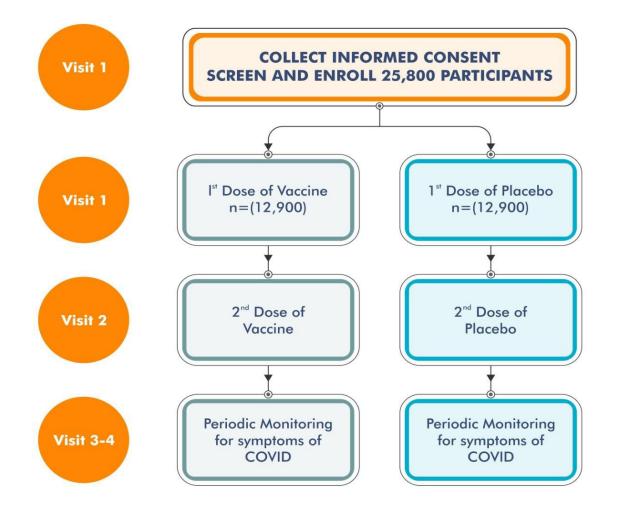
- Participants recruited between November 2020 and January 2021 across 25 sites
- 1:1 randomization among healthy adults (age 18-98 years)
- n = 25,798
- Primary endpoint: Preventing symptomatic COVID-19 occurring at least 14 days after second dose
- Secondary endpoint: Efficacy in subgroups based on age (18 59 years; ≥60 years)
- Evaluated safety, reactogenicity and consistency of immune responses

- ✓ **Overall vaccine efficacy: 77.8%** (95% CI: 65.2 86.4)
- ✓ Efficacy against severe disease: 93.4% (95% CI: 57.1 − 99.8)
- Efficacy against asymptomatic disease: 63.6% (95% Cl: 29.0 82.4)
- Safety outcomes: 12.4% reported adverse events
   (AE) in both vaccine and placebo arms (p<0.05)</li>
  - Most frequently reported systemic AEs included headache, followed by pyrexia, fatigue and myalgia
  - Serious AEs were reported by <0.5% of clinical trial participants
- Demonstrated efficacy against B.1.617.2 (Delta):
   65.2% (95% CI: 33.1 83.0)
  - First Phase 3 clinical trial to include Delta variant data

Source: Efficacy, safety, and lot to lot immunogenicity of an inactivated SARS-CoV-2 vaccine (BBV152): a, double-blind, randomised, controlled phase 3 trial; Ella, Reddy, Blackwelder, Potdar, et al.; medRxiv 2021.06.30.21259439; accessed July 7, 2021



# **Phase 3: Study Outline**

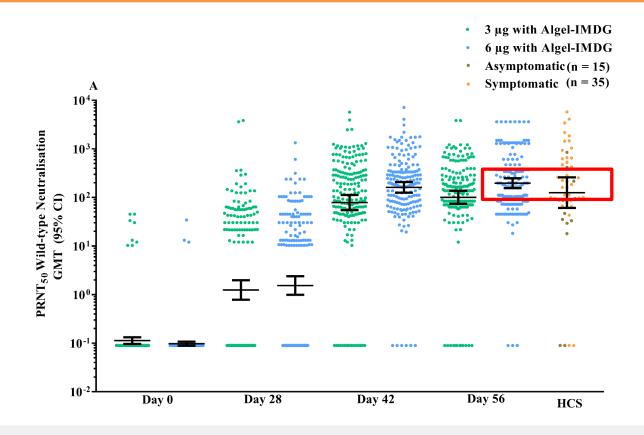


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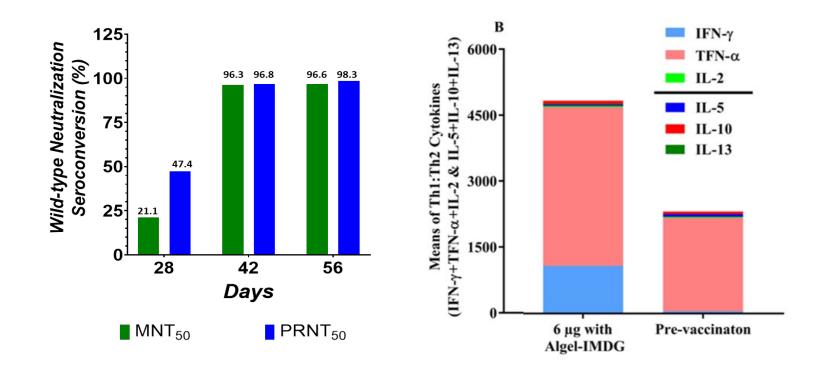
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# Phase 2: Study Results

- 6µg +Algel-IMDG demonstrated high neutralizing Abs responses compared to 3µg + Algel-IMDG group
- Mean GMT (95% CI) higher than human convalescent serum (HCS)
- 6µg +Algel-IMDG (**Covaxin**<sup>™</sup>) selected for Phase 3 study



# **Phase 2: Study Results**



### <u>Safety</u>

Events	Rate (%)	CI
Local	4.2% (1.8, 8.1)	95%
Systemic	7.4% (4.1, 12.1)	95%
Serious	0%	
Combined	10.3% (7.4, 13.8)	95%

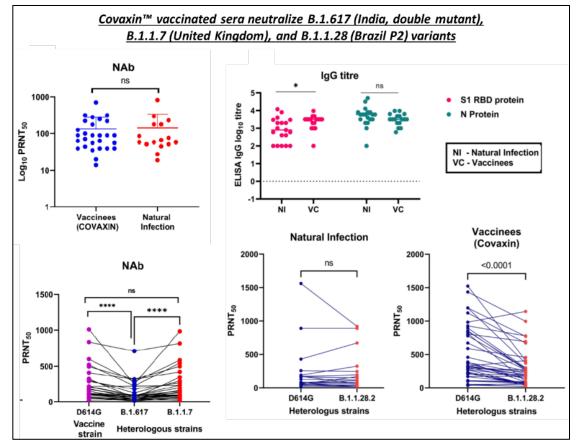
- High Seroconversion rates (>96%) in both MNT50 and PRNT50 measured up to day 56
- Induction of Th1 cell mediated immunity as measured by IFN-y, IL-2, TNF- $\alpha$

 No vaccine-related severe or lifethreatening adverse events reported to date



# Additional Research Demonstrating Effect Against Multiple Variants

• COVAXIN-vaccinated sera effectively neutralized several SARS-CoV-2 variants in an in-vitro plaque reduction neutralization assay



- ✓ <u>B.1.617 (India Kappa)</u>
- ✓ B.1.1.7 (United Kingdom Alpha)
- <u>B.1.1.28 (Brazil P2 Zeta)</u>
- The study was conducted by Indian Council of Medical Research (ICMR)-National Institute of Virology
- These studies suggest that COVAXIN vaccination may be effective against multiple SARS-CoV-2 variants.



# **Ocugen's Modifier Gene Therapy Platform**

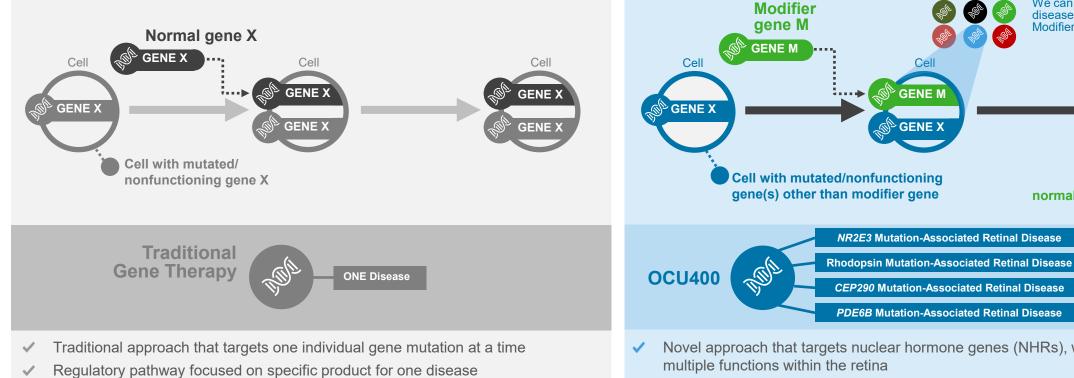
# **Breakthrough Technology Designed to**

Address Multiple Diseases with One Product Approach Complex Diseases Through Multiple Factors

# **Traditional Approach vs. Ocugen's Novel Platform**

Gene Augmentation: Transfer functional version of a non-functional gene into the target cells.

**Modifier Gene Therapy**: Introduce a functional gene to modify the expression of many genes, gene-networks and regulate basic biological processes in retina



Longer time to recoup development costs

Novel approach that targets nuclear hormone genes (NHRs), which regulate

- Smoother regulatory pathway due to ability to target multiple diseases with one product
- Ability to recoup development costs over multiple therapeutic indications

We can address a number of

Cell

Broad

Spectrum

**Therapy for RP** 

diseases using the same

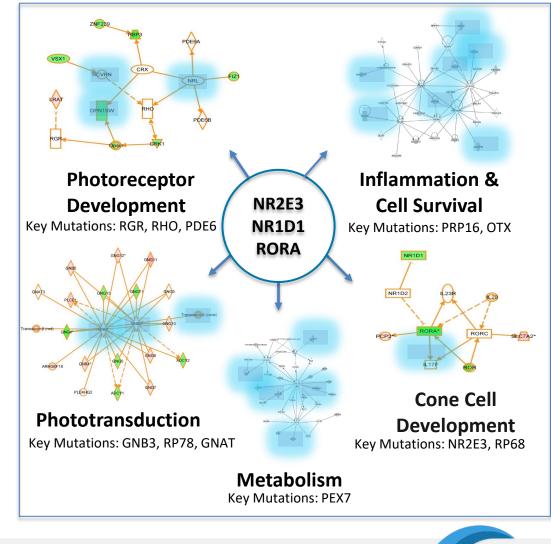
Cell with

normal function

Modifier Gene product.

# Why Target Nuclear Hormone Receptor Genes (NHRs)?

- Modulators of retinal development & function
- > Act as "master genes" in the retina
- Molecular reset of key transcription factors and associated gene networks retinal homeostasis
- Gene modifier concept including impact on clinical phenotypes is well known in other disease areas, CF and SMA \*

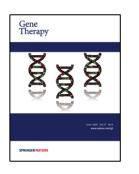


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## **Nature Gene Therapy Publication**

### Preclinical POC Data for *Nr2e3* Published in *Nature Gene Therapy*

- Efficacy results shown in 5 unique mouse models of RP
- > Technology developed at Harvard Medical School, Dr. Neena Haider's Lab
- Study demonstrates potency of modifier gene therapy to elicit broad-spectrum therapeutic benefits in early and advanced stages of RP
- Results show evidence of vision rescue in Early & Advanced Stages of disease



> Important milestone for development of therapy; demonstrated proof of principle

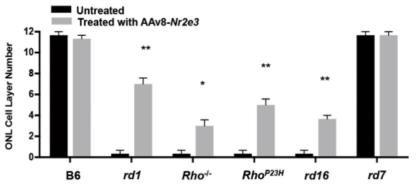
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- Protection elicited in multiple animal models of degeneration caused by different mutations
- > Potential to represent first broad-spectrum therapy and to provide rescue even after disease onset



# **OCU400 – Rescue in Early & Advanced Stage of Disease**

### Early Stage Rescue

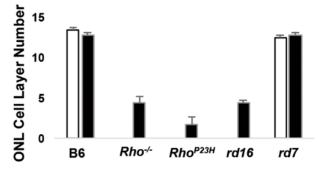


- PO single subretinal injection, evaluation 3-4 months post injection
- rd1 evaluated one-month post injection

#### **ONL: Outer Nuclear Layer**

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#### **Advanced Stage Rescue**

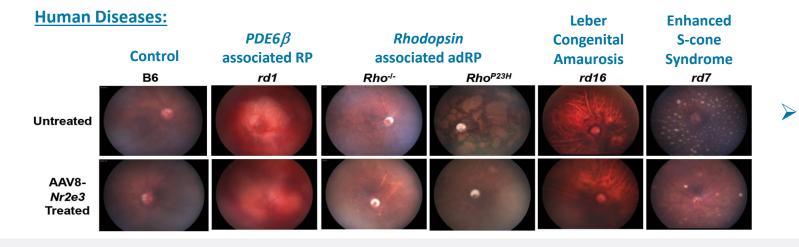


□ Uninjected ■ AAV8-Nr2e3 Injected

• P21 subretinal injection, evaluation 2–3 months post injection

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

- Restored ONL photoreceptors morphology in rd7
- ONL cell layer change in *rd7* model doesn't progress until 4-5 mos. of age



natureresearch

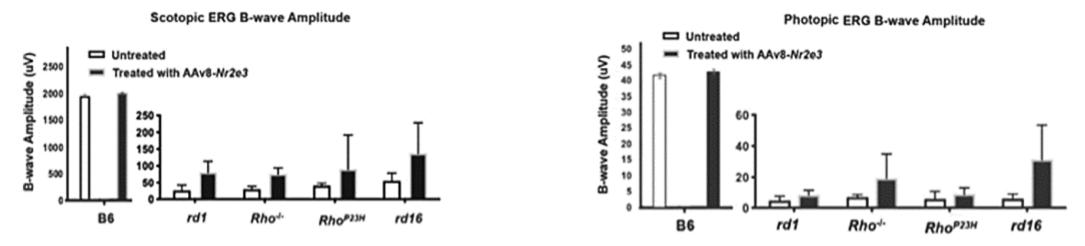
Fundus images and ONL count show how single product recuses vision in multiple mutations



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# **OCU400 – Demonstrates Improved Vision Signals in Retina**

### Electroretinogram (ERG) Response Reveals Rescue under Both Scotopic (dim-lit) as well as Photopic (well-lit) Conditions



ERG response: P0 single subretinal injection, evaluation 3-4 months post injection

Human vision is enabled by three primary modes:

- Photopic vision: Vision under well-lit conditions, which provides for color perception and functions primarily due to cone cells in the eye
- Mesopic vision: A combination of photopic vision and scotopic vision in low lighting, which functions due to a combination of rod and cone cells in the eye
- Scotopic vision: Monochromatic vision in very low light, which functions primarily due to rod cells in the eye

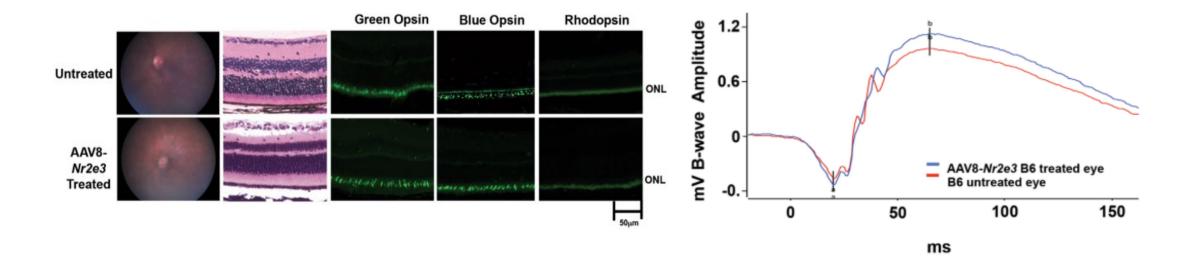
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### OCU400 – Demonstrated Safety in Mouse Model

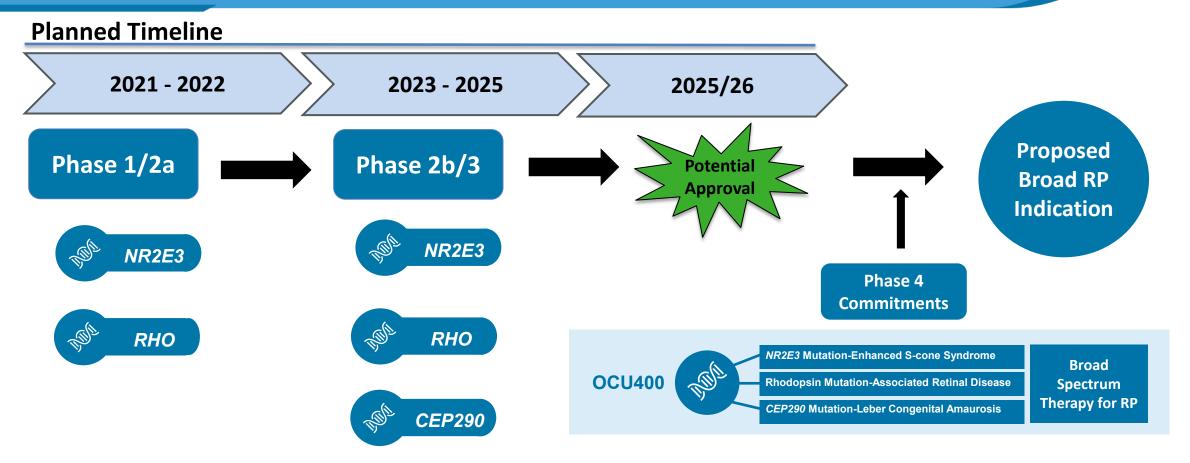
### Study Results Confirm Overexpression of *Nr2e3* by subretinal AAV8-*Nr2e3* Injection Is Not Detrimental to Retina – No Off-Target Effects



natureresearch



# **OCU400 – Clinical and Regulatory Strategy**



- > Successfully completed manufacturing at commercial scale (200L) at CanSinoBio to support clinical studies
- Preclinical tox studies in-progress
- > On target to file IND in 2H21

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# **OCU400 – Competitive Overview**

	OCU400	Traditional Gene Therapy	Cell Therapy
Features	The second secon	Roche HORAMA Biogen MEIRAGT Biogen SMEIRAGT NOVARTIS Allergan SANOFI	≫astellas jCyte ReNeuron
One product for many IRDs (including broad RP indication)			Limited
Technology established in the ocular disease space			×
POC data in RP models with different genetic mutations		$\bigotimes$	$\mathbf{X}$
Expected long-term outcome	Potentially longer benefit due to promotion of homeostasis	Potentially limited due to loss of retinal cells over time	Not established
Target Patient Population	Large	Small (specific to mutation)	Variable
Developmental cost	Low (economies of scale)	High (No economies of scale)	High



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Potential Competitors pursuing treatment of RP with Traditional Gene Therapy



Potential Competitors pursuing treatment of RP with Cell Therapy

# OCU410 (AAV-RORA) – Dry Age-Related Macular Degeneration

#### We Believe OCU410 Has the Potential to Address this Disease through its Multi-Factor Approach



### Dry AMD

- Leads to irreversible blindness due to degeneration of the retina
- ~9-10M patients in the U.S.
- Currently no approved treatment for Dry AMD

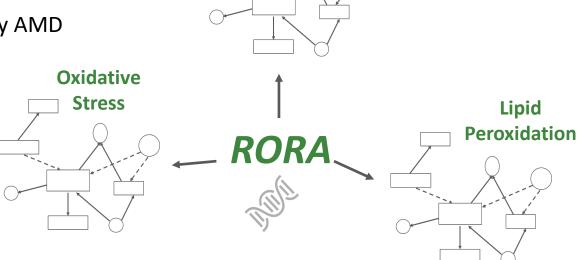




### **Contributing Factors**

- Aging
- Genetics
- Environmental Factors





Inflammation



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# **OCU200:**

Diabetic Macular Edema (DME) Diabetic Retinopathy (DR) Wet Age-Related Macular Degeneration (Wet AMD)

Novel Biologic Offering Benefits Beyond Anti-VEGF

# OCU200 – Potential to Treat DME, DR & Wet AMD

#### OCU200 Provides Hope to All patients with DME, DR or Wet AMD

- DME  $\rightarrow$  ~0.7M patients in the US\*
- DR  $\rightarrow$  ~7.7M patients in the US\*
- Wet AMD  $\rightarrow$  ~1.1M patients in the US\*

#### OCU200 is a Transferrin-Tumstatin Fusion Protein

- Tumstatin: Multiple MOAs for treatment and prevention of macular degeneration 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
- Significant global market potential



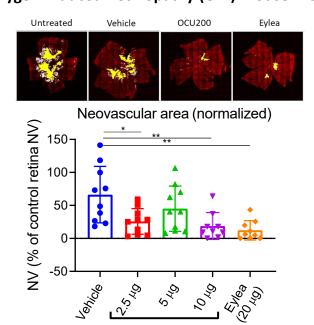
~50% of Patients <u>DO NOT</u> Respond to Anti-VEGF/Corticosteroids Therapies

# **OCU200 – Transferrin-Tumstatin Fusion Protein**

#### **OCU200** Demonstrated Superior Efficacy Compared to Existing Anti-VEGF Therapies

Inhibits new blood vessel formation
 Anti-inflammatory
 Anti-oxidative

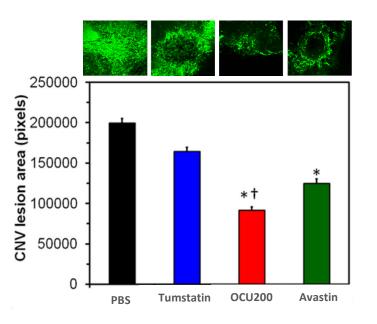
#### DME/DR Oxygen-Induced Retinopathy (OIR) Mouse Model



Effect of OCU200 intravitreal treatments on Neovascularization (NV). Data are presented as mean $\pm$  SD. Filled circles represent data points from individual eyes \* P < 0.05, \*\* P < 0.01 (n = 9-10 eyes per group)

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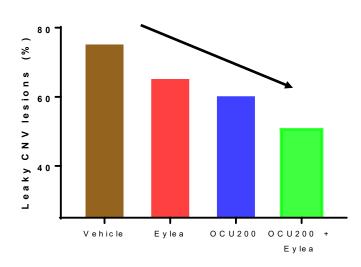
Wet AMD In-Vivo Laser-Induced Rat CNV Model



\* indicates p<0.05 when compared to PBS and/or tumstatin treatment

+ indicates p<0.05 when compared to Avastin; CNV lesions measured on day 14 after treatment





Data expressed as percentage of CNV lesions on Day 10 after treatment. Laser induction & treatment start on Day 0



## **OCU200 – Distinct Mechanism of Action**

	OCU200	Anti-VEGF	Anti-Integrin
Features	💿 ocugen	Genentech <sup>(1)</sup> U NOVARTIS <sup>(1)</sup> REGENERON <sup>(1)</sup> KODIAK	CASCLEPIX Allegro
Reduces VEGF level/Fluid			
Selectively works on active endothelial cells (Neovascular)		$\mathbf{X}$	
Activates native anti-angiogenic response		$\mathbf{X}$	
Enhanced effective delivery through Transferrin		$\bigotimes$	$\bigotimes$
Pro-apoptotic and anti-oxidative		$\bigotimes$	
Dosing Frequency	Expected once in 3 months	1-3 months	1-3 months

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- COVAXIN<sup>M</sup> Vaccine candidate for the US and Canadian markets with potential for revenues this year
- > Ophthalmology
  - Modifier Gene Therapy Platform has the potential for one product to treat many diseases
  - Novel biologic has the potential to treat anti-VEGF /corticosteroids non-responders (~50% of the patients)
  - Multiple near and mid-term milestones with plan to initiate four Phase 1/2 trials over next 18 months



**A Bold Vision to Cure Blindness Diseases** and **Offer a Differentiated** Vaccine to Save Lives from **COVID-19** 

> For more information, contact: IR@ocugen.com

