# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, DC 20549**

## FORM 8–K

**CURRENT REPORT**
**Pursuant to Section 13 OR 15 (d)**
**of the Securities Exchange Act of 1934**

Date of Report (Date of Earliest Event Reported): **April 13, 2020**

## OCUGEN, INC.

(Exact Name of Registrant as Specified in its Charter)

| **Delaware** | **001-36751** | **04-3522315** |
|---|---|---|
| (State or Other Jurisdiction of Incorporation) | (Commission File Number) | (I.R.S. Employer Identification Number) |

**5 Great Valley Parkway, Suite 160**
**Malvern, Pennsylvania 19355**
**(484) 328-4701**
(Addresses, including zip code, and telephone numbers, including area code, of principal executive offices)

**N/A**
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8–K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a–12 under the Exchange Act (17 CFR 240.14a–12)
- o Pre–commencement communications pursuant to Rule 14d–2(b) under the Exchange Act (17 CFR 240.14d–2(b))
- o Pre–commencement communications pursuant to Rule 13e–4(c) under the Exchange Act (17 CFR 240.13e–4(c))

**Securities registered pursuant to Section 12(b) of the Act:**

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common Stock, $0.01 par value per share | OCGN | The Nasdaq Stock Market LLC<br>(The Nasdaq Capital Market) |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

**Item 7.01 Regulation FD Disclosure**

Attached as Exhibit 99.1 and furnished for purposes of Regulation FD is a presentation that Ocugen, Inc. ("Ocugen") will post on its website on April 13, 2020 and may use from time to time in presentations or discussions with investors, analysts and other parties.

The information in this Item 7.01 (including Exhibit 99.1) is being furnished solely to satisfy the requirements of Regulation FD and shall not be deemed to be "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934 (the "Exchange Act") or otherwise subject to the liabilities of that Section, nor shall it be deemed to be incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act.

**Item 9.01 Financial Statements and Exhibits**

The following exhibit is being filed herewith:

**(d) Exhibits**

| Exhibit No. | Document |
|---|---|
| 99.1 | Ocugen, Inc. Presentation |

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: April 13, 2020

OCUGEN, INC.

By: /s/ Shankar Musunuri

Name: Shankar Musunuri

Title: Chief Executive Officer and Chairman

ocugen

# Developing **Transformative Therapies** to Treat the Whole Eye

NASDAQ: OCGN

Corporate Deck

# 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.

Forward-looking statements involve known and unknown risks, uncertainties and other factors, including those risks set forth in the Company's filings with the Securities and Exchange Commission, which are available at www.sec.gov, that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. Forward-looking statements are based on our management's beliefs and assumptions and information available to management as of the date of this presentation. Our actual future results may be materially different from what we expect. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.

This presentation includes estimates by us of statistical data relating to market size and growth and other estimated data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. This presentation also includes statistical and other industry and market data that obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data.

This communication shall not constitute an offer to sell or the solicitation of an offer to sell or the solicitation of an offer to buy any securities, nor shall there be any sale of securi in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offering of securities shall be made except by means of a prospectus meeting the requirements of Section 10 of the Securities Act of 1933, as amended.

# Company Highlights

OCGN (NASDAQ)

Strong Global IP

Diversified Portfolio

## THREE WAVES OF TECHNOLOGICAL INNOVATION

**SMALL MOLECULE PHASE 3 RARE DISEASE ASSET**
**OCU300** for ocular Graft Versus Host Disease (oGVHD)

**MODIFIER GENE THERAPY PLATFORM**
**OCU400** for Inherited Retinal Diseases
**OCU410** for Dry AMD

**NOVEL BIOLOGIC THERAPIES FOR RETINAL DISEASES**
**OCU200** for DME, Diabetic Retinopathy, Wet AMD

**Strategic Gene Therap Manufacturing Partners**

**CanSinoBIO**

ocugen

# Experienced Leadership Team

- Diverse experience in large pharma, signature biotech, and small companies
- Development, Manufacturing and Commercialization expertise provides know-how to take pipeline from preclinical to market

| | | | |
|---|---|---|---|
| **Shankar Musunuri, PhD, MBA**<br>Chairman, CEO and Co-Founder | Nuron Biotech | Pfizer | Amylin |
| **Sanjay Subramanian, MBA**<br>Chief Financial Officer | Aralez Pharmaceuticals | Bausch Health | GM |
| **Daniel Jorgensen, MD, MPH, MBA**<br>Chief Medical Officer | CDC | Pfizer | Aventis |
| **Rasappa Arumugham, PhD**<br>Chief Scientific Officer | Merck | Pfizer | Soligenix |
| **Vijay Tammara, PhD**<br>Senior Vice President, Regulatory & Quality | FDA | Nuron Biotech | Merck |
| **Kelly Beck, MBA**<br>Vice President, Investor Relations & Administration | tengion | DrugDev | PRAHEALTHSCIENCES |

# Three Waves of Technological Innovation

| | Indication | | Prevalence (US) | Preclinical | Phase 1 | Phase 2 | Phase |
|---|---|---|---|---|---|---|---|
| ***OCULAR SURFACE DISEASE (small molecule)*** | | | | | | | |
| OCU300 | oGVHD | Orphan US | 63,000 | | | | |
| ***MODIFIER GENE THERAPY PLATFORM*** | | | | | | | |
| OCU400 *AAV-NR2E3* | *NR2E3* Mutation-Associated Retinal Degeneration | Orphan US | 500-600 | | | | |
| | *CEP290* Mutation-Associated Retinal Degeneration | Orphan US | 2,500-3,000 | | | | |
| | *RHO* Mutation-Associated Re tinal Degeneration | | 10,400-12,700 | | | | |
| OCU410 *AAV-RORA* | Dry AMD | | 9-10M | | | | |
| ***RETINAL DISEASES (novel biologics)*** | | | | | | | |
| OCU200 Tumstatin-Transferrin | Diabetic Macular Edema | | 745,000 | | | | |
| | Diabetic Retinopathy | | 7.7M | | | | |
| | Wet AMD | | 1.1M | | | | |

ocugen

# OCU300: oGVHD

## *Near-term Commercialization Opportunity*
## *Potential to be First FDA Approved Treatment*

# OCU300 for oGVHD:
# Unmet Need for Patients with Rare Ocular Disease

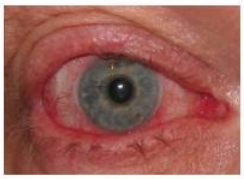
## Ocular Graft vs Host Disease (oGVHD)

- Autoimmune disease that occurs in allogeneic bone marrow transplant (BMT) patients
  - **Donor derived leukocytes attack recipient ocular tissue**
- Patients encounter dry, tearless eyes, vision issues, severe pain, discomfort, and potential ocular scarring
- May lead to significant vision loss and irreparable ocular surface damage

Ocugen is the **first and only company to receive orphan drug designation** from FDA for treatment of oGVHD

**~60%** of allogeneic bone marrow transplan patients will develop oGVHD

**~63,000** patients in the US



**~3-6 months** from transplant is when patients will develop oGVHD

**Top 30 BMT centers** treat majority of patients with oGVHD

ocugen



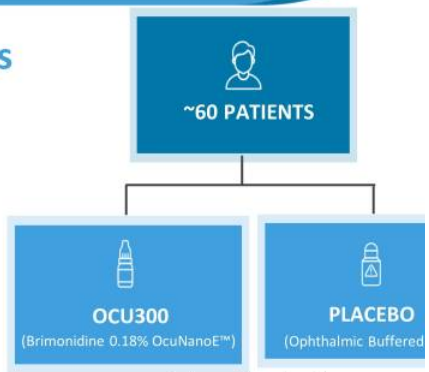
Source: Prevalence of Hematopoietic Cell Transplant Survivors in the United States, Majhail N et al Oct 201
Source: https://bethematch.org/tcdirectory/search/advanced/#-/-/-/false/-/TotalTransplants-

# Phase 3 Study With Topline Results Expected 2H2020

***Over 95% planned enrollment completed***

## Indication: Treatment of ocular discomfort and ocular redness in patients with oGVHD

- 84-day, Randomized, Double-Masked, Placebo-Controlled Study
- Key inclusion criteria: diagnosis of 'definite' oGVHD using the International Chronic Ocular GVHD Consensus Group revised diagnostic criteria (Ogawa, 2013)
- Patients referred to specialty BMT centers; 10+ centers are active in this study



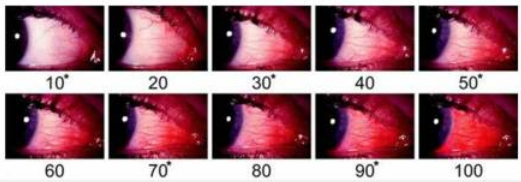

**2:1 randomization**
(OCU300 n=40; Placebo n=20)

### *Co-primary endpoints include:*

- **Symptom:** Ocular discomfort based on Visual Analog Scale (VAS)

**On a scale from 0-10, what was the intensity of your Ocular Discomfort, at its worst, over the past 24 hours?**


- **Sign:** Ocular redness based on Validated Bulbar Redness (VBR) Score




ocugen



*Enrollment as of March 20, 2020*

# Active Ingredient Proven Safe and Effective

## Safety

**Existing Molecule (Brimonidine)**

- 505(b)(2) regulatory pathway allows use of safety data already available for brimonidine
- Brimonidine approved for chronic treatment in glaucoma

## Efficacy

**Early stage clinical studies led to Phase 3 design**

**OcuNanoE™ drug delivery system improves overall effic**

**OCU300 is preservative-free (no BAK)**

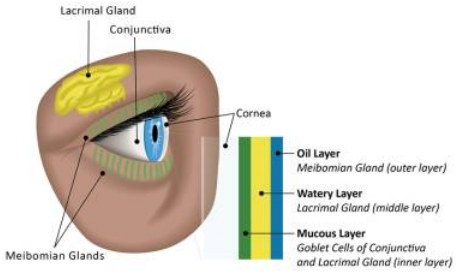
- BAK (benzalkonium chloride) is damaging to the cornea

## Generic substitution prohibited

- Generic brimonidine (0.2%) not approved for oGVHD
- Concentration/formulation different from OCU300
- Contains BAK preservative
- OCU300 completing controlled studies in oGVHD patients
- AB criteria not applicable

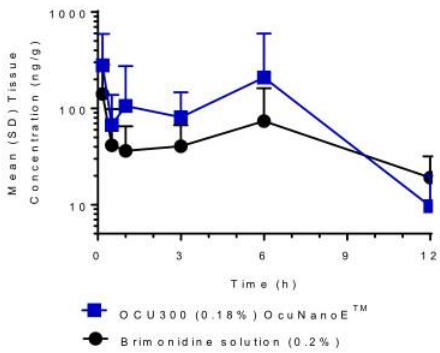
ocugen

# OcuNanoE™ Drug Delivery System Improves Overall Efficacy

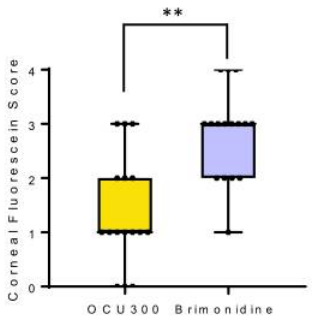



**Drug distribution to lacrimal gland from traditional eye drops is low relative to other target tissues**

**Brimonidine Level in Lacrimal Gland (Preclinical)**




**Mouse DED Model (Preclinical)**




- OCU300 = Brimonidine (0.18%) OcuNanoE™
- Brimonidine = Commercial 0.2% solution
- Figure shows median, interquartile range & mi fluorescein score
- **p<0.01

**OcuNanoE™ increases brimonidine in lacrimal gland and improves overall efficacy of OCU300**

ocugen

# OCU300 has Compelling Value Proposition

## Patients

- Spend **3 months** in hospital after receiving **bone marrow transplant**
- Most **exhibit symptoms** while still **under hematologist/ oncologist care**

## Physicians

- **Hematologists/Oncologists** are **first prescribers**, then referred to **specialized ophthalmologists**
- Hematologists looking for **approved therapy**; no knowledge of off-label options

## Market Access

- **No** approved therapy
- Seek to establish **ICD-10** diagnostic code
- Analysis supports **premium pricing**
- Opportunities to **partner** for **commercialization**

## Market Potential

- Potential to be **first appr product** in US market
- First and only company t **Orphan Drug Designati** for oGVHD
- Advances in hematopoiet transplantation leading to **number** of **transplant su**

| ~63,000 Patients | Targeted BMT Centers | Premium Pricing | Orphan Dr |
|---|---|---|---|

ocugen

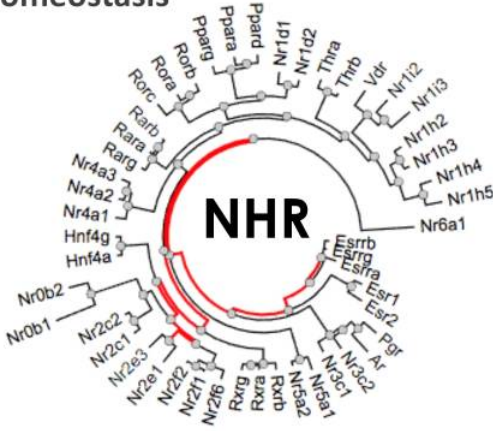
# Breakthrough Modifier Gene Therapy Platform Addressing Multiple Diseases with One Product

# NHRs as Modifier Gene Therapeutics

## Nuclear Hormone Receptors (NHRs)

**NHRs such as *NR2E3* play a critical role in modulating cellular homeostasis by regulating basic biological processes**

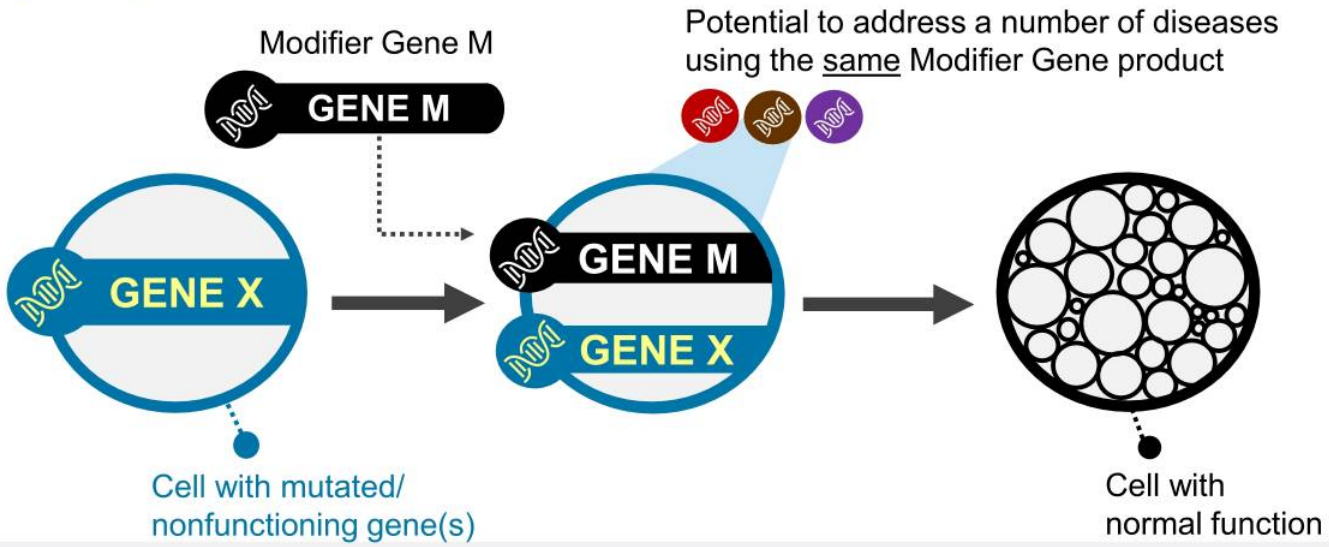
- Development
- Metabolism
- Circadian cycle
- Energy homeostasis
- Steroidogenesis
- Lipid metabolism
- Xenobiotics




**NHRs such as *NR2E3*, *NR1D1*, *RORA* and *NR2C1* are important modulators of retinal development, function and thereby disease**

# Potential to Treat Many Diseases with One Product

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



# Preclinical Data Published in *Nature Gene Therapy*

## Efficacy results in 5 unique mouse models of retinitis pigmentosa (RP)

**Underwent administration of AAV-*NR2E3* by subretinal injection**

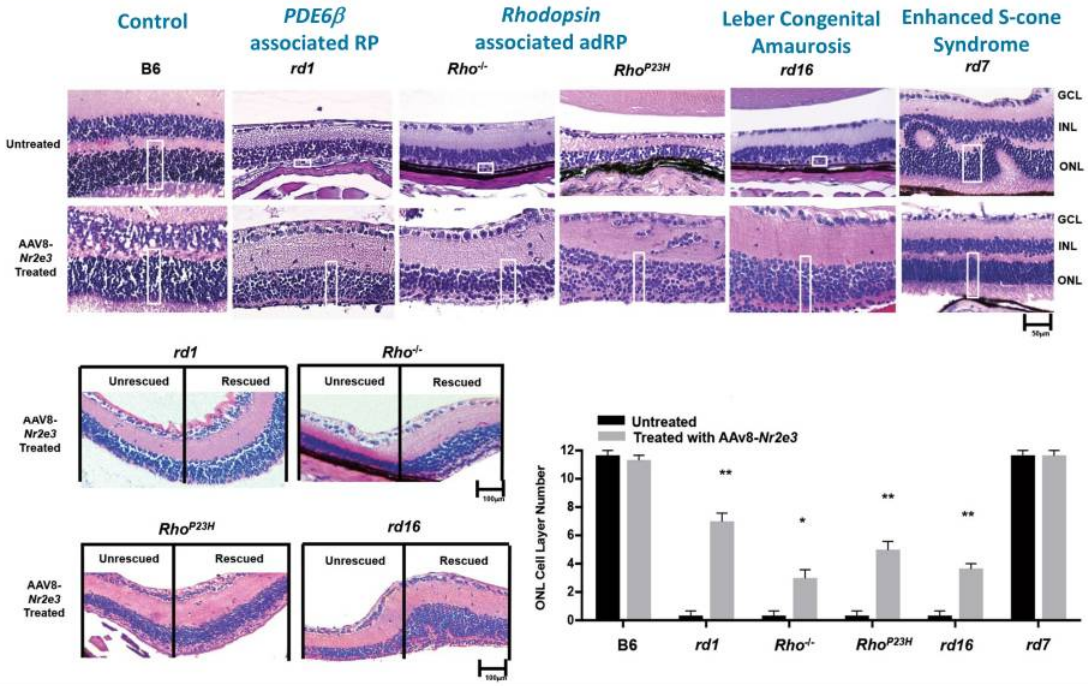
## Study demonstrates potency of novel modifier gene therapy to elicit broad-spectrum therapeutic benefits in early and intermediate stages of RP

- 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 therapy and to provide rescue even after disease on



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

# OCU400 Preserves Photoreceptors in Early Stage of Disease



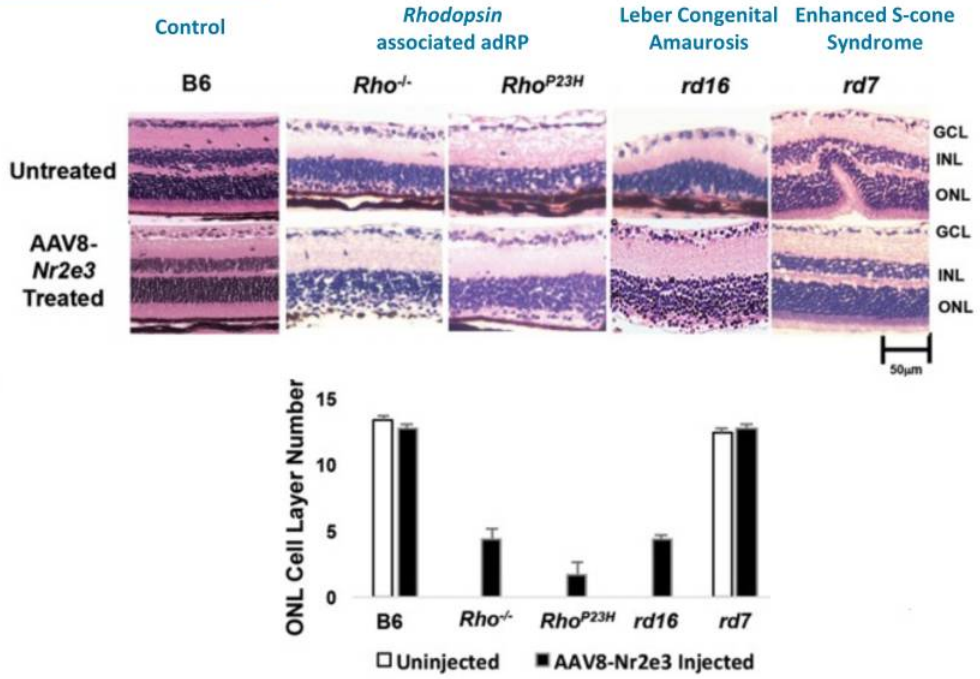

## Retina histology

- P0 single subretinal inje evaluation 3-4 months injection
- *rd1* evaluated 1-month injection
- Restored Outer Nuclea (ONL) photoreceptors morphology in rd7



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

# OCU400 Preserves Photoreceptors in Advanced Stage of Disease







## Retina histology

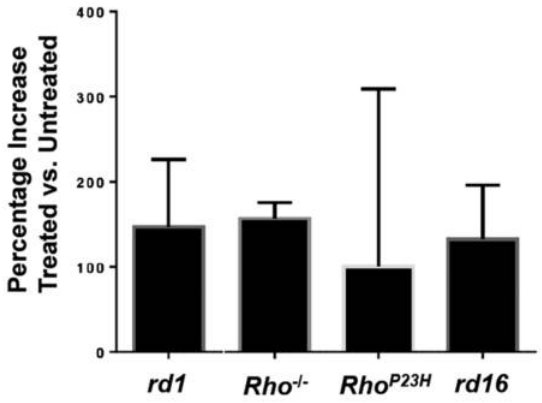
- Animals injected at P21 a evaluated 2–3 months po injection
- Restored ONL photorecep morphology in rd7
- ONL cell layer change in *r* model doesn't progress u 4-5 mos. of age



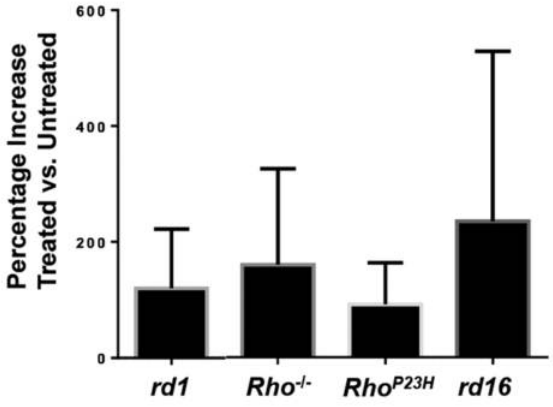
nature research https://www.nature.com/articles/s41434-020-0134-z

# OCU400 Demonstrates Improved Vision Signals in Retina

**Scotopic ERG B-wave Percentage Increase**



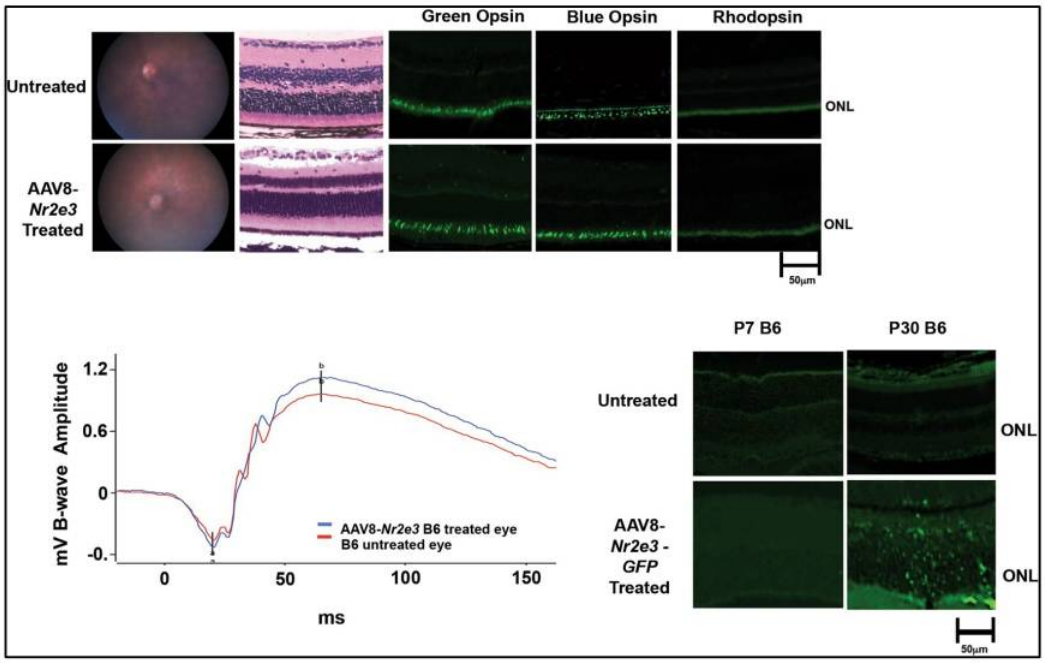

**Photopic ERG B-wave Percentage Increase**




- **ERG response**: P0 single subretinal injection, evaluation 3-4 months post injection
- *rd1* evaluated 1- month post injection

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

# OCU400 Demonstrated Safety in Mouse Model

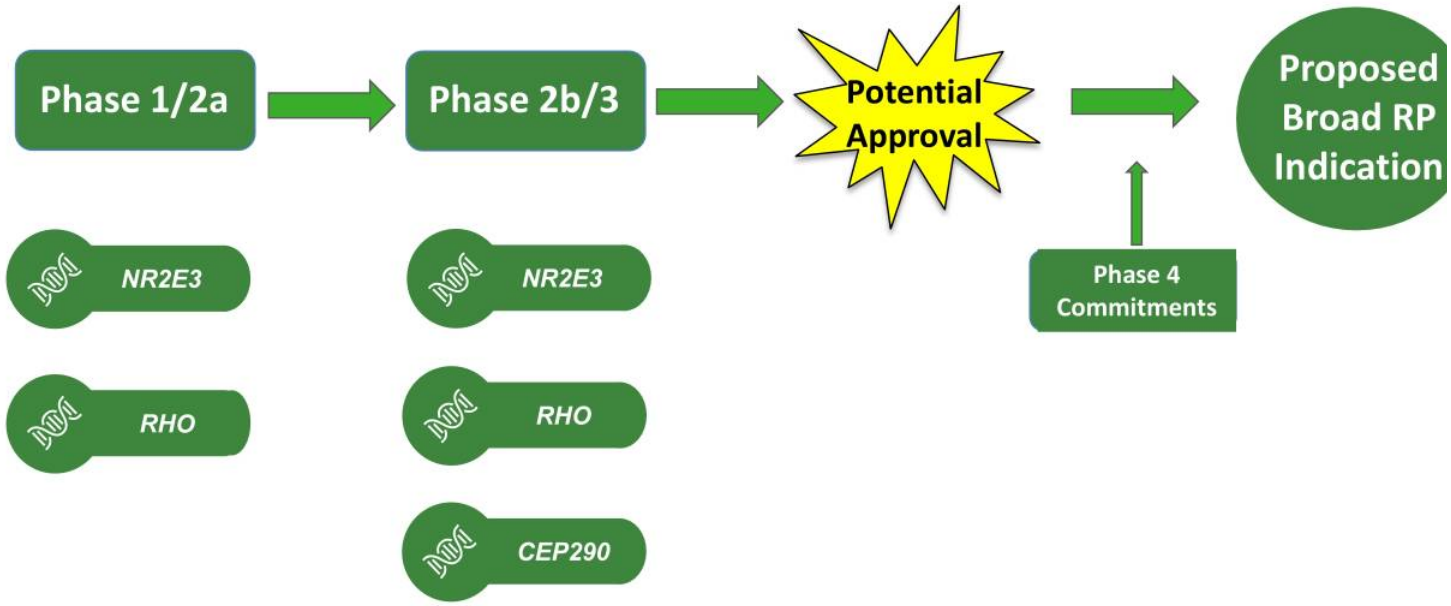



**Study results confi**
**overexpression o**
***Nr2e3* by subretin**
**AAV8-*Nr2e3* injecti**
**is not detrimental**
**retina**



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

# OCU400 Regulatory Strategy



# Gene Therapy Manufacturing: Plagued by Backlog and Timing Delays

## Cell & gene therapy manufacturing demand continues to increase

- 1,060 clinical trials globally; 80 cell and gene therapy trials in Phase 3
- Large pharma acquiring companies to support internal programs
  - eg: Roche acquired Spark; Pfizer acquired Bamboo; Celgene acquired Juno
- Others being acquired by major CMOs to establish their presence in the gene therapy
  - eg: Thermo Fisher acquired Brammer Bio; Catalent acquired Paragon

## Gene therapy companies facing manufacturing bottleneck & costs

- Long wait in the queue for CMO while large pharma can bypass (due to scope and financial power)
- Traditional CMO model not appropriate for implementing specialized process optimization steps
- High cost for the CMC development and clinical supplies; approximately:
  - $7M - $10M for Phase 1
  - $8M - $10M for late stage
  - $10M - $15M for scale-up development for commercialization/BLA filing

**Critical to find a Strategic and Reliable Partner that also shares costs**

ocugen



Source: Manufacturing Cures: Infrastructure Challenges Facing Cell And Gene Therapy Developers In Vivo June 2019 invivo.pharmaintelligence.informa.com

# OCU400 Gene Therapy Manufacturing: Strategic Partnership with CanSinoBIO

## CanSinoBIO

- Biotech company publicly-listed on Hong Kong exchange (6185.HK) with market cap of ~$2 Billion USD
- State-of-the-art facilities with world class team
- Provides scalable GMP cell lines (such as HEK293 suspension culture adopted) for commercial manufacturing

## CanSinoBIO to perform CMC development & manufacturing of clinical supplies

- CanSinoBIO responsible for all associated costs
- Option for commercial manufacturing agreement

## CanSinoBIO has rights to develop, manufacture and commercialize OCU400 for Greater China market

ocugen ⟷ CanSinoBIO

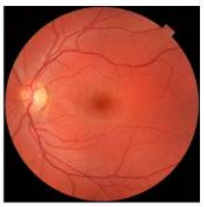
**Partnership paves a path for Ocugen to advance OCU400 into the clinic with significantly reduced capital and resources**

# OCU410 (AAV-*RORA*): Dry AMD

## Dry Age-Related Macular Degeneration (AMD)

- Leads to irreversible blindness due to degeneration of the retina

**~9-10M patients in the US**

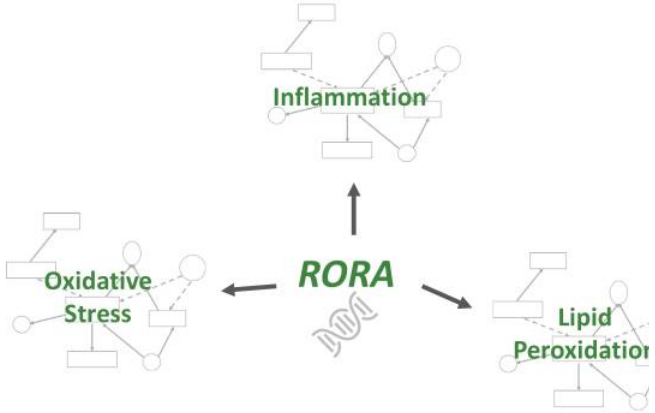


Normal Retina

Dry AMD

Contributing Factors

- Aging
- Genetics
- Environmental Factors




**Currently no approved treatment for Dry AMD**

ocugen



Sources: https://www.brightfocus.org/macular/article/age-related-macular-facts-figures
https://www.uniprot.org/uniprot/P35398#function

# OCU200: Diabetic Macular Edema (DME)
# Diabetic Retinopathy (DR)
# Wet AMD

## *Novel Biologic Offering Benefits Beyond Anti-VEGF*

# DME, DR & Wet AMD are Leading Causes of Blindness

**Diabetic Macular Edema (DME)**
**Diabetic Retinopathy (DR)**
**Wet AMD**

- Most common causes of vision loss in patients with diabetes and aging population
- Anti-VEGF & corticosteroids therapies not effective in approximately 50% of patients
- Leakage and fluid accumulation continues in sub-retinal space even after many months of treatment

**~745,000** DME patients in the US

**~7.7M** DR patients in the US

**~1.1M** Wet AMD patients in the US

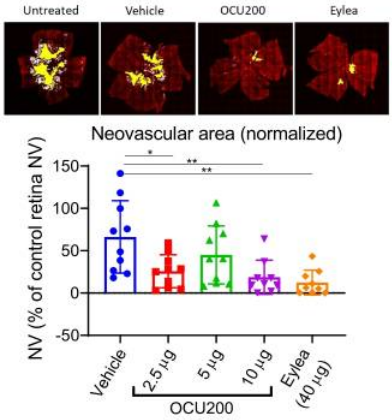
**Targeting Integrin Pathways Offer New Opportunities**

ocugen

# OCU200: Tumstatin-Transferrin Fusion Protein

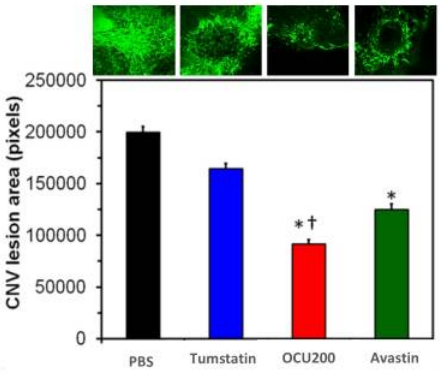
• Inhibits new blood vessel formation • Anti-inflammatory • Anti-oxidative

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



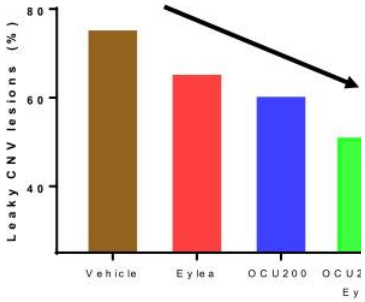

Effect of OCU200 intravitreal treatments on Neovascularization (NV). Data are presented as mean± SD. Filled circles represent data points from individual eyes
* P < 0.05, ** P < 0.01 (n = 9-10 eyes per group)

**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

**Wet AMD**
**In-Vivo Laser-Induced Mouse CNV Mo**




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

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

ocugen

# Summary of Near-Term Milestones

| OCU300<br>ocular GVHD<br>*(Phase 3 small molecule)* | OCU400 (AAV-*NR2E3*)<br>Retinal Degenerative Diseases<br>*(gene therapy)* | OCU200<br>DME, DR, Wet AMD<br>*(novel biologic)* |
|---|---|---|
| ✓ Mar 2020: Over 95% Planned Enrollment Achieved | ✓ Mar 2020: Preclinical Data Published in Nature Gene Therapy | • 2020-2021: Continue IND-E Studies |
| • 2H2020: Topline Results Expected | • 2020: Continue IND-Enabling Studies<br>✓ Initiated Tox Studies | • 2022: Target Phase 1/2a Cli |
| | • 2021: Target Phase 1/2a Clinical Trial | |

Note: Check mark (✓) denotes completed milestone. All other milestones are anticipated future milestones.

# A Bold Vision to Treat the Whole Eye

**For more information, contact:**

**Kelly Beck**

**Vice President, Investor Relations & Administration**

kelly.beck@ocugen.com