Odylia THERAPEUTICS

Bringing life changing treatments to people with genetic diseases regardless of prevalence or commercial interest

Novel Anc80 AAV Platform, and Late-Preclinical Stage Gene Therapy to treat RPGRIP1-Associated Retinal Dystrophy

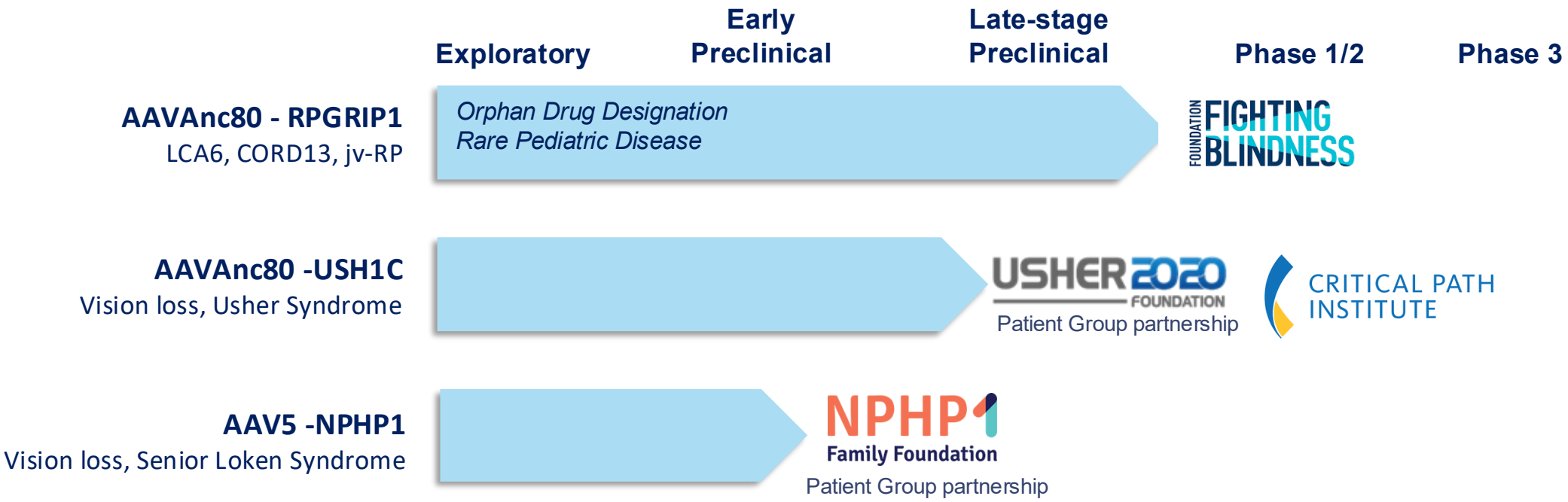
Non-confidential

re*Imagining* drug development for rare disease

Odylia Therapeutics
a nonprofit drug development company

Odylia delivers on the promise of treatments for the vastly underserved rare disease patient community. As a nonprofit drug development company, Odylia increases the chances of success, while accelerating timelines and reducing costs. We do this by focusing on the safety, efficacy, and available technologies necessary to treat each rare disease, rather than being mired in the need to generate a high return on investment.

A Dynamic Portfolio Driving Innovation in Rare Disease



Odylia THERAPEUTICS

Led by Drug Developers & the Rare Disease Community

The Odylia team includes leaders with extensive experience in drug development, rare diseases, and health care innovation

Ashley Winslow, PhD, Chief Executive Officer and Chief Scientific Officer

Over 20 years of drug development experience in the rare disease space. PhD in medical genetics from the University of Cambridge and postdoctoral work at Massachusetts General Hospital and Harvard Medical School. Previously worked for Pfizer, and Jim Wilson's Orphan Disease Center at the University of Pennsylvania.

Scott Dorfman, Co-Founder & Executive Chairman

Founder and former Chairman, & CEO of Innotrac Corporation (NASDAQ: INOC. Operations Partner for Fulcrum Equity Partners and board member of Dropoff Inc., Complemar Corporation, Fulcrum Equity, Odylia Therapeutics and Usher 2020 Foundation. Rare disease father and health innovation investor.

Luk Vandenberghe, PhD, Co-Founder

Assistant Professor, Harvard Medical School and Director, Grousbeck Gene Therapy Center, Massachusetts Eye and Ear. Discovery of novel delivery technologies, and experience seeding multiple venture-backed startups.

Odylia THERAPEUTICS

Depth & Passion: Odylia's BoD and Advisors

Collectively 100s of INDs, clinical programs, pre-launch, and commercial launch programs

Board of Directors

Scott Dorfman
Chairman of the Board
Venture Partner, Fulcrum
Prior: CEO, Innotrac,

Elizabeth Attias, ScD
CEO, Atom Strategic Consulting;
Chief Strategy and Development Officer, Sermonix Pharmaceuticals

Khandan Baradaran, PhD
Prior: SVP Regulatory Affairs, Nanoscope
Regulatory CMC, Ultragenyx

Joy Cavagnaro, Ph.D.
President, Access Bio
Prior: VP of Reg Affairs & Int. Compliance, HGS
Ex-FDA

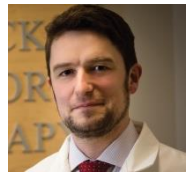
Wendy Erler
Senior VP
Patient Affairs, Sarepta
Prior: Alexion, AstraZeneca, Global Genes

Mat Pletcher, PhD
CSO, Arbor Biotechnologies
Prior: Weaver BioSciences, Roche, Astellas, RDH12 Foundation

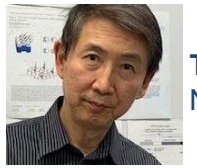
Gregory Robinson, Ph.D.
Prior CSO, Nightstar, Agilis, Tevard
BD, Shire

Odylia's Board of Directors and Advisors have deep expertise in drug development, commercialization, CMC, clinical delivery, startup formation, and alternative business models.

Advisors



Luk Vandenberghe, Ph.D.
Assist. Professor, Harvard Medical School
Director, Grousbeck Gene Therapy Center, Massachusetts Eye and Ear



Tiansen Li, Ph.D.
National Eye Institute, NIH

Jean Bennett, M.D., Ph.D.
Professor, Perelman School of Medicine
University of Pennsylvania

Eric Pierce, M.D., Ph.D.
Director, Ocular Genomics Institute
Harvard Medical School
Massachusetts Eye and Ear

Alan Spiro, B.A., Ph.D., J.D.
Partner, ExSight Ventures

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RPGRIP1 (OT-004) Gene Therapy

RPGRIP1 Associated Retinal Dystrophy: Clinical Overview

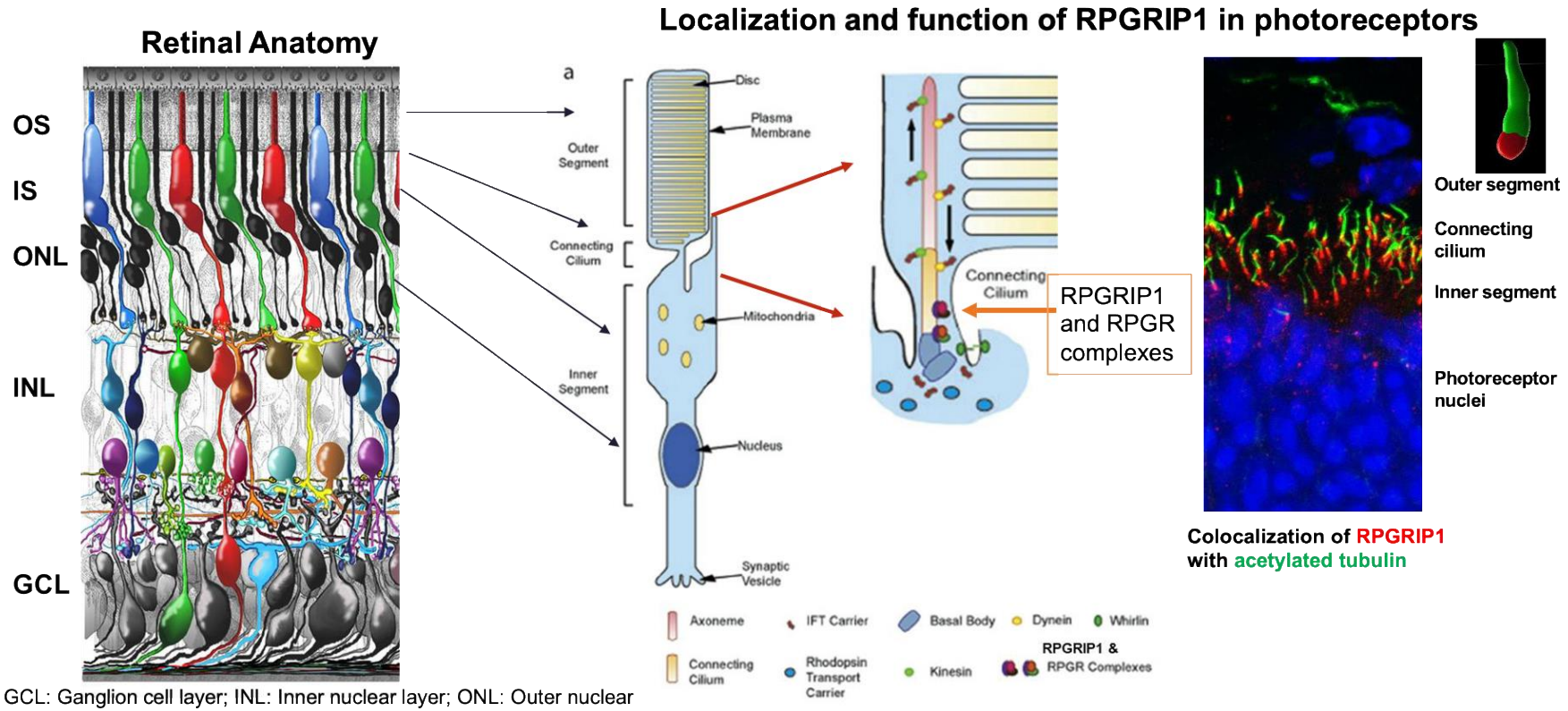
Homozygous and compound heterozygous loss-of-function mutations in RPGRIP1 are associated with the diagnoses of ***LCA6, CORD13, jvRP,*** collectively referred to as ***RPGRIP1- associated retinal dystrophy.***

Patients with LCA6 describe vision loss (photo on the right):

Symptoms reported in clinical publications specific to RPGRIP1 mutations	• Reduced ERG • Nystagmus • Macular Degeneration/blurred vision • Reduced visual acuity • Fundus pigmentary deposits (bone spicule, granularity) • Photophobia • Night blindness • Hyperopia • Vascular attenuation • Reduced visual acuity (limited to light perception) • Eye poking in infants • Drusen-like deposits • Peripheral vision loss • Disc pallor • Chorioretinal atrophy • Keratoconus/keratoglobus
Age at onset	• Usually in early infancy but before 1 year of age • Can be variable
Age at major decline points	• Visual loss onset within first year of life • Potential treatment windows exist in pediatric and young adult populations

RPGRIP1 Gene Therapy Program Overview: OT-004

OT-004 was developed to treat vision loss caused by RPGRIP1 mutations. This gene therapy utilizes the Anc80 AAV capsid to deliver a functional copy of the RPGRIP1 gene to photoreceptors (RK promoter).

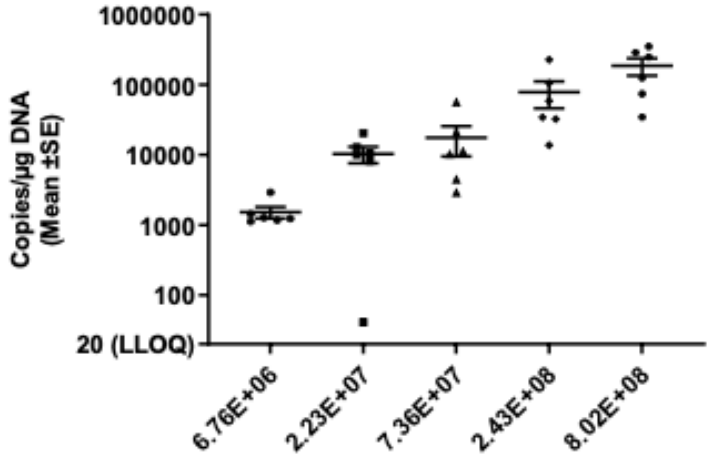


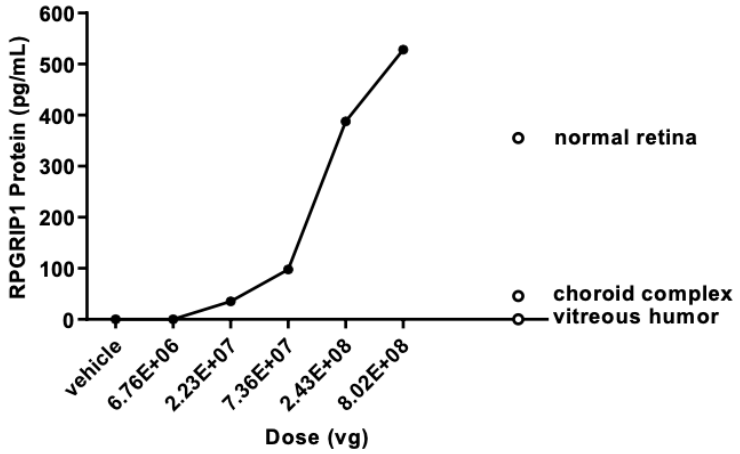
GCL: Ganglion cell layer; INL: Inner nuclear layer; ONL: Outer nuclear layer; IS: Inner segment; CC: Connecting cilium; OS: Outer segment

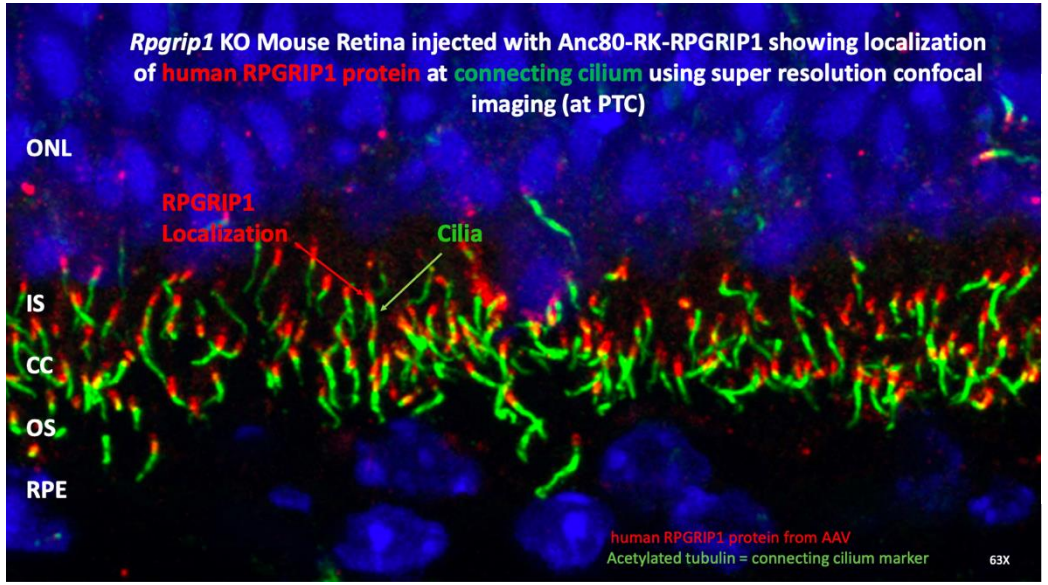
Impact of OT-004

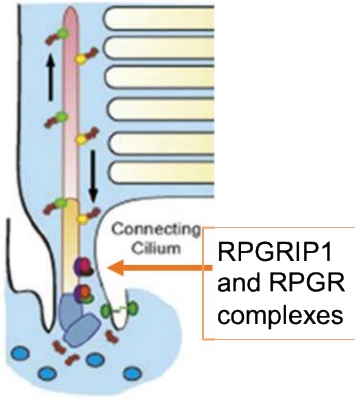
OT-004 Function: Delivery, Expression, Localization

OT-004 drives dose-dependent expression in WT mouse retina





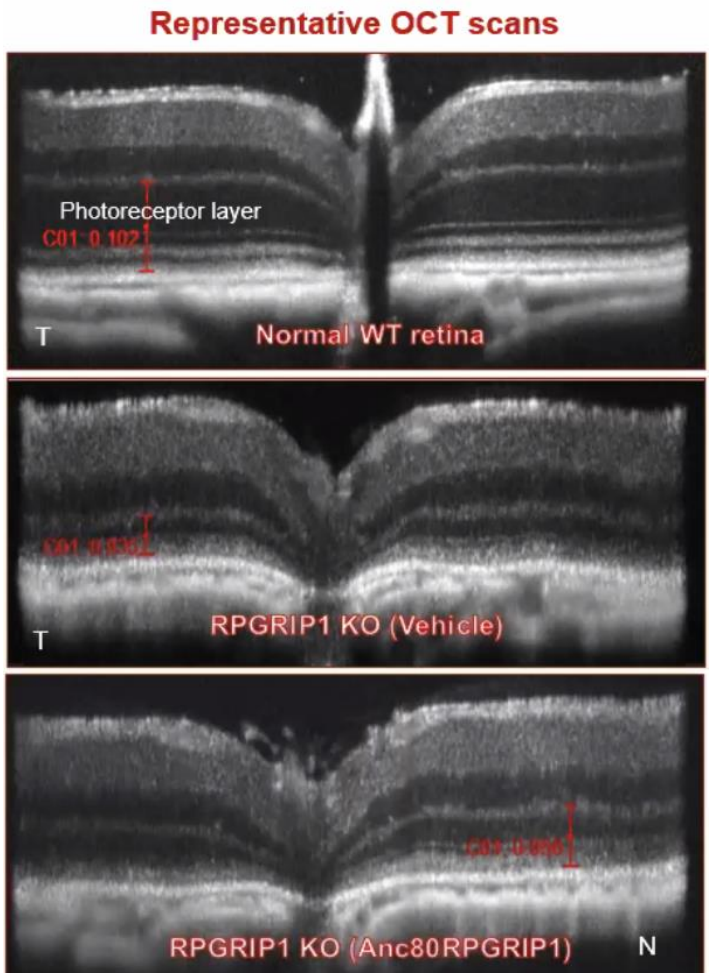




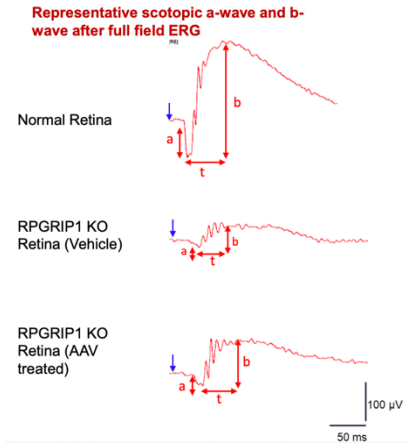
OT-004- RPGRIP1 localization to the connecting cilia after subretinal delivery in the RPGRIP1 KO mouse

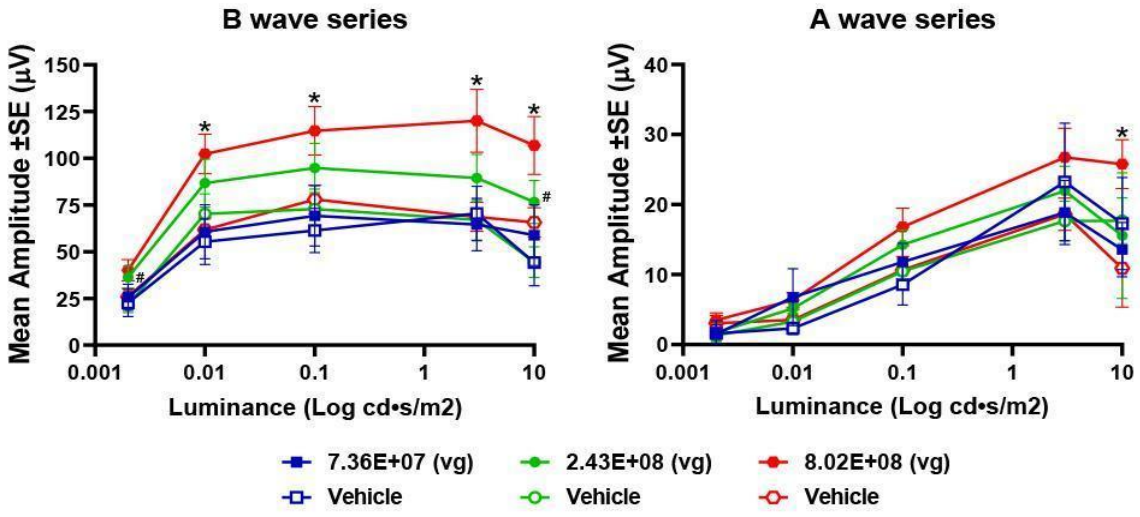
Impact of OT-004 on Retinal Anatomy & Function

Retained retinal thickness and function with OT-004 at the highest dose in RPGRIP1 KO mice



Dose	7.36E+07 (vg)	2.43E+08 (vg)	8.02E+08 (vg)	Sham Controls
12 weeks post injection				
% increase	10%	11%	19%	-57%
p-value	0.0408 *	0.0124 *	0.0141 *	
24 weeks post injection				
% increase	49%	41%	63%	-79%
p-value	0.1771	0.0009 ***	0.1425	





NHP Tolerability Study (non-GLP toxicology)

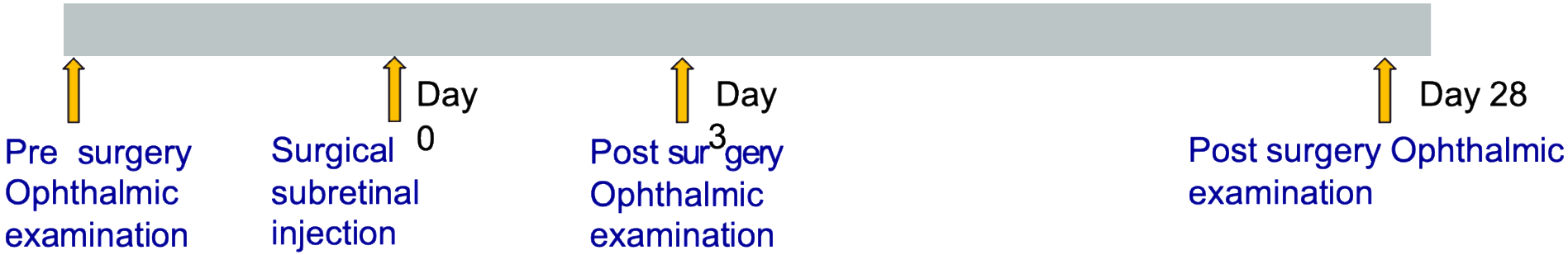
Study Design

Group	No. of Animals Females	Dosing Regimen Left Eye	Dosing Regimen Right Eye	Dose Level (vg/eye)a	Dose Concentration (vg/mL)
1 (Control)	2	Vehicle	Vehicle	0	0
2 (Low)	3	Test Article	Test Article	4.5×10^{10}	3.0×10^{11}
3 (High)	3	Test Article	Test Article	4.5×10^{11}	3.0×10^{12}

a Dose levels were based on a dose volume of 150 µL/eye.

Four weeks post subretinal injections ophthalmic examinations were performed to study tolerability and biodistribution of vector

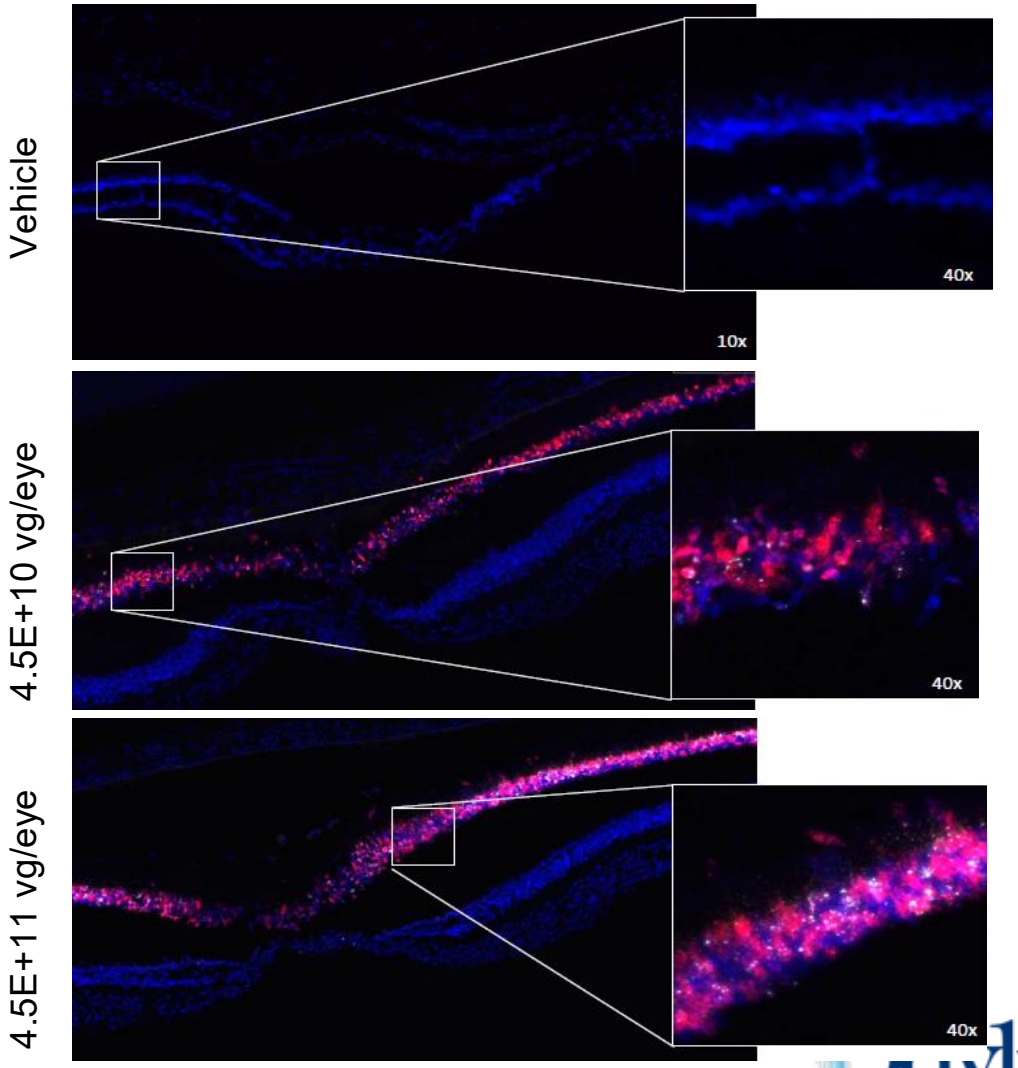
- Fundus imaging
- Slit lamp observation
- IOP measurement
- Optical coherence tomography
- Histopathology (H&E)
- ISH for biodistribution



NHP Tolerability Study (non-GLP toxicology)

In Situ Hybridization of RPGRIP1 mRNA (red) and vector DNA (white)

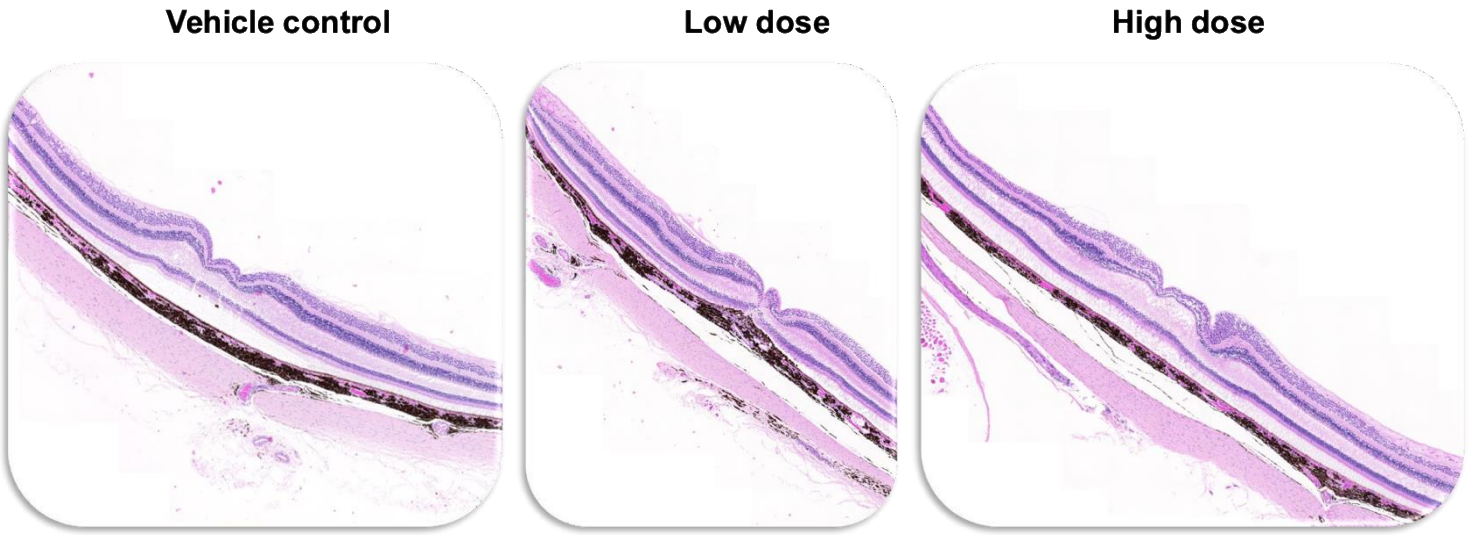
- In situ probes for RPGRIP1 DNA and RNA detected a dose-dependent increase
- Image analysis software scoring indicate that mRNA expression was correlated with the amount of vector DNA in a cell
- No vector DNA or RNA was detected in vehicle injected retinas



Red= mRNA; White= DNA

OT-004 Tolerability Study in NHPs (cynos)

- Well tolerated after 4 weeks
- No test article related clinical findings
- Clinical pathology results showed no abnormal findings in control or treated animals no signs of retinal inflammation or infiltrations in and around macula region
- No effects of drug on hematology, coagulation, clinical chemistry, urinalysis, or organ weight
- OCT findings- Transient and commonly seen changes: reflective material, focal retinal separations (some animals)resolved by week 4
- *Single subretinal injection led to desired biodistribution of the RPGRIP1 DNA/RNA confirmed by ISH in central retina including macula and fovea essential for vision preservation in patients*



Regulatory &
Operational Strategy

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RPGRIP1 Target Product Profile

Product	OT-004 is a gene therapy constructed of the Anc80 AAV vector that delivers the RPGRIP1 transgene, driven by the Rhodopsin Kinase promoter
MOA	Gene replacement therapy using Anc80 viral vector to deliver the human RPGRIP1 gene into the photoreceptors of the retina to preserve or restore vision in patients with RPGRIP1 associated retinal dystrophy
Pursued Indications	Retinal dystrophy caused by biallelic mutations in the RPGRIP1 gene. Diagnoses can include Leber congenital amaurosis 6 (LCA6), cone-rod dystrophy 13 (CORD13), early-onset retinitis pigmentosa (RP), Achromatopsia.
Target population	Patients with retinal dystrophy caused by biallelic mutations of the RPGRIP1 gene.
Efficacy	Improve vision in patients suffering from RPGRIP1 associated retinal dystrophy by preserving or restoring photoreceptor function in subretinal area
Safety	Limited warnings or precautions
USP	First-in-class, highly specific gene therapy restoring vision in patients with RPGRIP1- associated retinal dystrophy with single administration. The vector design utilizes a novel capsid with improved biological qualities for subretinal delivery
Administration and dosing	Single subretinal injection
Costing	Comparable price to Luxturna (voretigene neparvovac-rzyl, Roche) for inherited retinal disease due to mutations of both copies of the RPE65 gene

Program Milestone Summary & Partners

Program Milestones

Preclinical studies

- ✓ Efficacy data & Feasibility- studies complete
- → Tox and biodistribution studies- underway, terminal timepoint May 2026
- → Assay development underway

Regulatory

- ✓ Orphan Drug Designation & Rare Pediatric Disease Designations Granted Nov 2021
- ✓ PreIND FDA –January 2025

Clinical Development

- ✓ Clinical Advisory Group- clinical study design
- ✓ Patient identification- underway
- ✓ Patient Advisory Board- launched Q3 2022, clinical study design

Manufacturing

- ✓ Partnered with Andelyn Biosciences- manufacturing process, tox batch produced

Partners (past & present)

Foundation Fighting Blindness
Virscio
Andelyn Biosciences — Reliably Expert. Purposefully Driven.
Pharmaron
charles river
LabCorp Covance
Grousbeck Gene Therapy Center
PTC Therapeutics
Massachusetts Eye and Ear — Bold Science. Life-Changing Cures.

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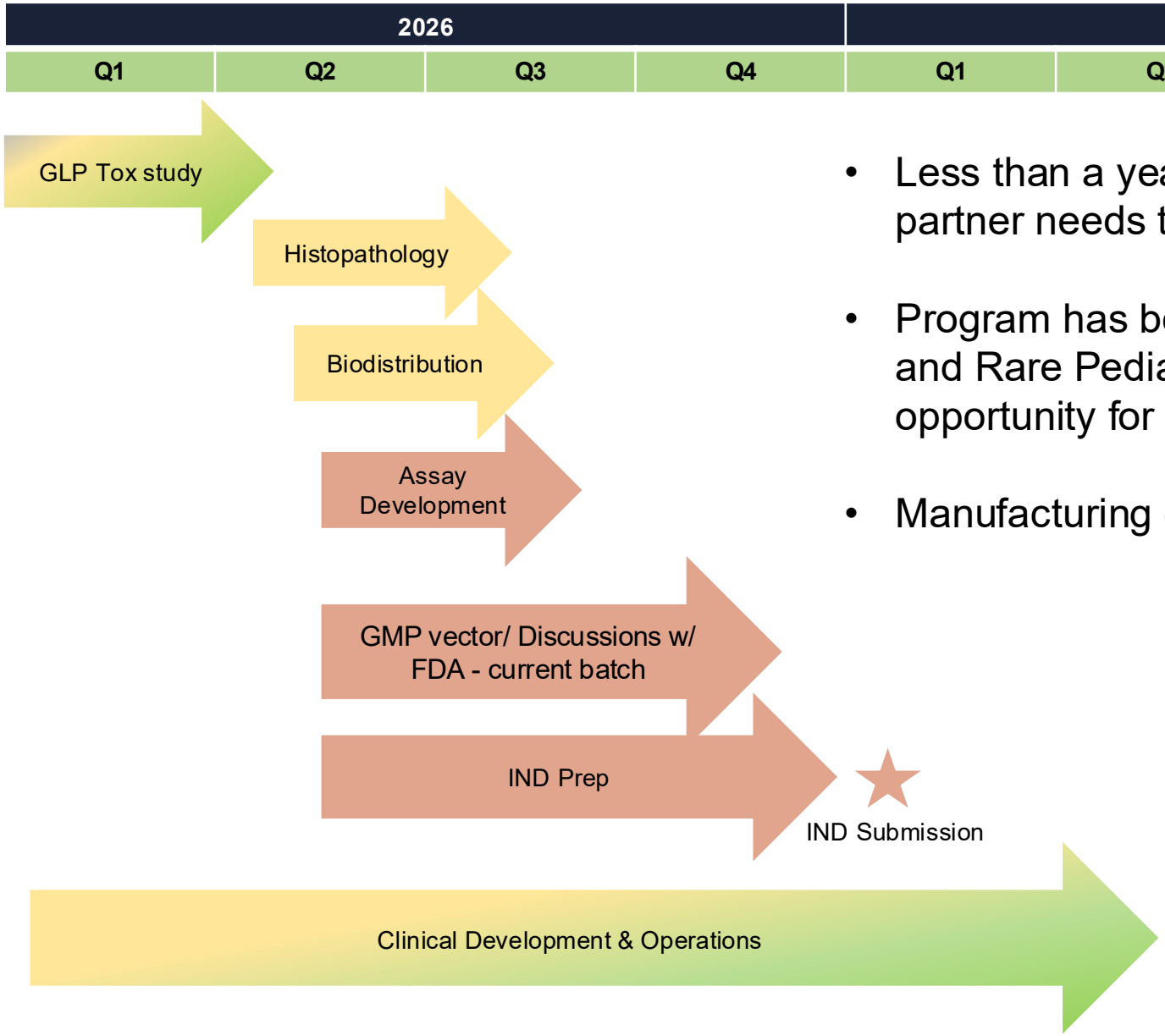
RPGRIP1 Program Timeline



- Less than a year to clinical trials (funding or partner needs to be identified)
- Program has both Orphan Drug Designation and Rare Pediatric Disease Designation, opportunity for PRV
- Manufacturing established

Clinical Activities

RPGRIP1 Clinical Advisory Group

Robert K. Koenekoop MD, MSc, PhD, FRCS(C), FARVO

Professor of Paediatric Surgery, Human Genetics and Adult Ophthalmology at McGill University
Director of the Laboratory for Retinal Genetics and Therapeutics
Chief Paediatric Ophthalmology

Montreal Children's Hospital

Jiong Yan, MD

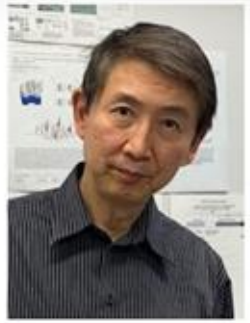
Associate Professor of Ophthalmology
Director, Vitreo-Retina Surgery Fellowship

Emory University School of Medicine

Thaddeus (Ted) Dryja, MD

Professor of Ophthalmology, Harvard Medical School
Physician and Surgeon, Massachusetts Eye and Ear Infirmary

Massachusetts Eye and Ear Infirmary



Tiansen Li, PhD

Senior Investigator, Retinal Cell Biology and Degeneration Section

National Eye Institute

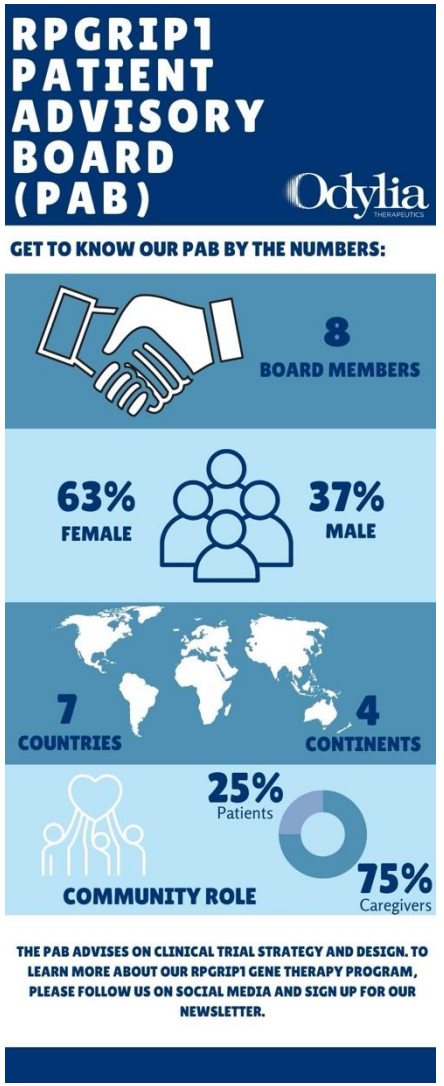
Eric A. Pierce, MD, PhD

Director, Inherited Retinal Disorders Service, Massachusetts Eye and Ear
William F. Chatlos Professor of Ophthalmology, Harvard Medical School

Massachusetts Eye and Ear

RPGRIP1 Patient Advisory Board

Assembled in November 2022, the RPGRIP1 Gene Therapy Patient Advisory Board provides critical insight into the patient and caregiver experience.



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We are actively seeking partners for our clinical stage activities

- License and commercialization out-licensing or partnership for RPGRIP1, and if interest, first right of refusal on current and future programs
- Additional Anc80 programs on a gene-by-gene basis
- Odylia R&D team can handle, share, or hand-off preclinical through early clinical phase for RPGRIP1 or other programs
- Co-discovery for other rare diseases. Not restricted to retinal diseases or gene therapy. Anc80 has biological advantages compared to other capsids, but if the biology necessitates a different capsid Odylia has the ability to shift for each program's needs
- Philanthropic support, venture philanthropy, or impact investing opportunities

Turning Hope into Impact

Odylia THERAPEUTICS

Contact Us

info@odylia.org

www.odylia.org

Odylia Therapeutics

Odylia Therapeutics

Ashley Winslow
awinslow@odylia.org

Charity Navigator
FOUR-STAR

Platinum Transparency 2025
Candid.