Corporate Overview

1Q2023

The Opus Mission

Gene therapies focused on patients with rare inherited retinal diseases (IRDs)

Jean Bennett, MD, PhD
F.M. Kirby Professor of Ophthalmology
University of Pennsylvania
Co-founder and Board Member, Opus Genetics
Co-founder Spark Therapeutics & inventor of LUXTURNA®

Executive Management Team

Ben Yerxa, Ph.D.
CEO

Ash Jayagopal, Ph.D.
CSO

Joe Schachle
COO

Jennifer Hunt
CDO

Matt Farber
CFO

FOUNDATION FIGHTING BLINDNESS

CLEARSIDE BIOMEDICAL

INSPIRE

envisia THERAPEUTICS

VANDERBILT UNIVERSITY MEDICAL CENTER

Roche

KODIAK

gsk GlaxoSmithKline

INSPIRE

PARION SCIENCES

GRIFOLS

genzyme

QUINTILES

editas MEDICINE

Retrophin

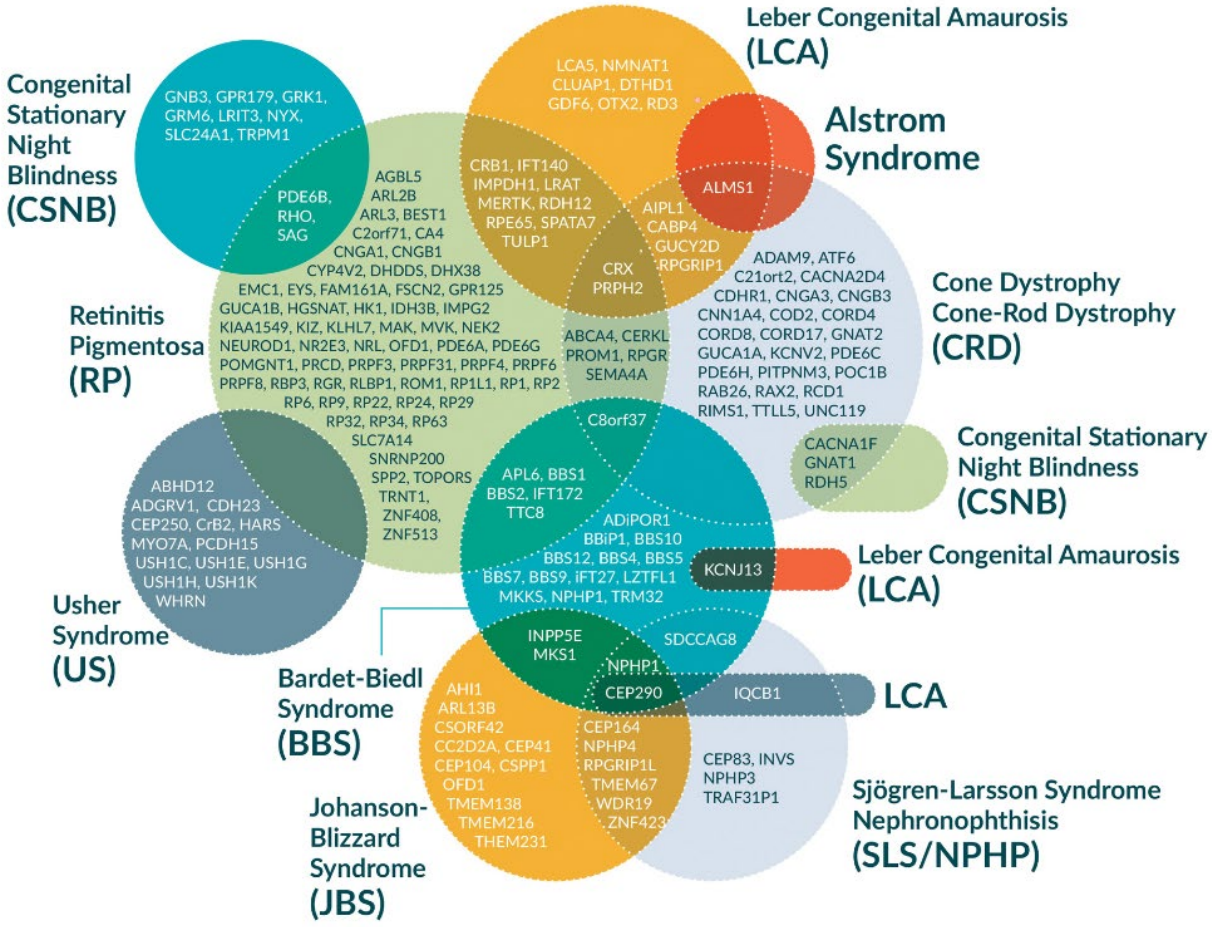
FUJIFILM Diosynth biotechnologies

istari ONCOLOGY

JOHN DEERE

Millions Affected by Inherited Blindness

- IRDs account for up to 20% of all blindness in individuals 16-64[(1)]
- 280+ genes to date have been associated with IRDs[(1)(2)]
- Estimated ~430,000 patients affected in the U.S.[(3)]
- Estimated ~5.5 million patients affected worldwide[(4)]

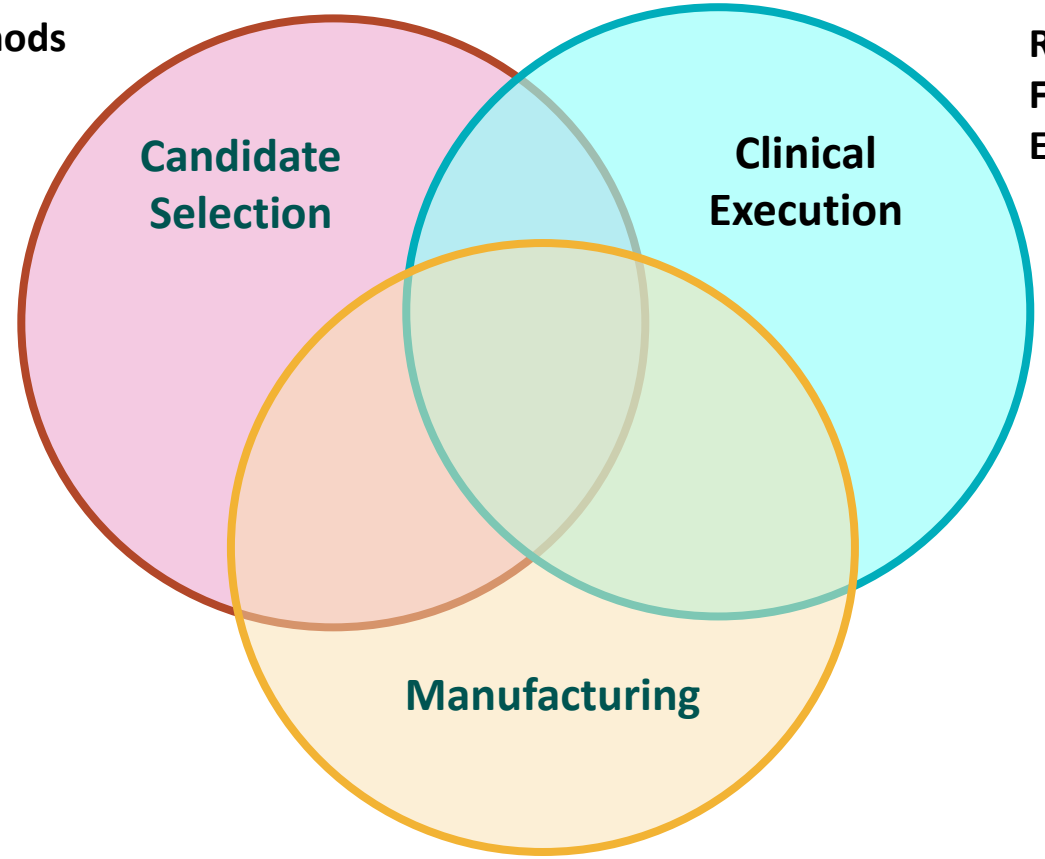


Source: EyeNet. https://www.aao.org/Assets/bebfbaef-a092-45b0-9883-c563331546ae/636649294795430000/july-2018-eyenet-supplement-pdf?inline=1.

(1) Shughoury et al, Intl Ophtha Clinics 2021 doi: 10.1097/IIO.0000000000000377
(2) RetNet; University of Texas Houston https://sph.uth.edu/retnet/
(3) Gong et al, Clin Ophth 2021
(4) Hanany et al, PNAS 2020 www.pnas.org/cgi/doi/10.1073/pnas.1913179117

Opus Approach

Tried and true materials & methods
Strong in vivo efficacy
Creative asset acquisition



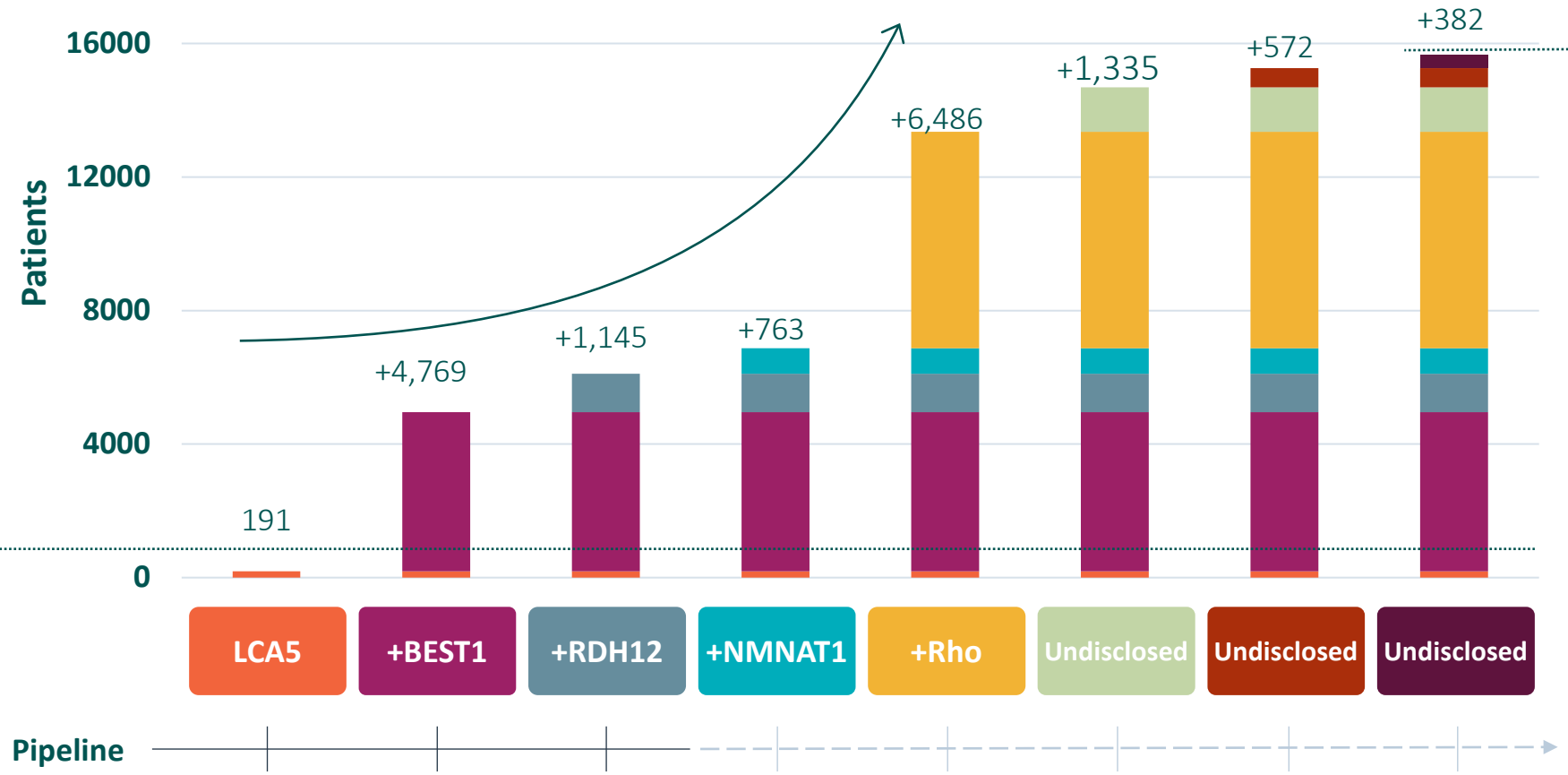
Relationships with top clinical centers
Foundation Fighting Blindness collaborations
Efficient trials

High quality
Scale that makes sense
Affordable contracts

Sustainable Pipeline With Annual IND Filings

Genetic Target	Preclinical	IND Enabling	Phase 1/2	Phase 2/3	Key Milestones
LCA5					• IND cleared 4Q 2022
BEST1					• IND 2H 2023
RDH12					• IND 1H 2024
NMNAT1					• IND 2H 2025
RHO					
Undisclosed					
Undisclosed					
Undisclosed					

Significant Prevalence of Patients for Treatment (U.S. Only)



LUXTURNA®: marketed for **572 patients**

Over 15,500 patients in U.S. alone with first 8 proposed programs

Significant Ex-U.S. prevalence

Note: Estimated U.S. prevalence only; Stone et.al. Ophthalmology 2017.

IRD Landscape

- Limited number of clinical-stage pure play IRD companies
- Numerous IRD diseases to target with high unmet need
- Minimal competition with programs across the IRD landscape
- Opportunity to lead the field with a dedicated portfolio
- Relationship with FFB further strengthens strategy

FOUNDATION FIGHTING BLINDNESS

RD FUND

FOUNDATION FIGHTING BLINDNESS CONSORTIUM

FOUNDATION FIGHTING BLINDNESS My Retina Tracker® Registry

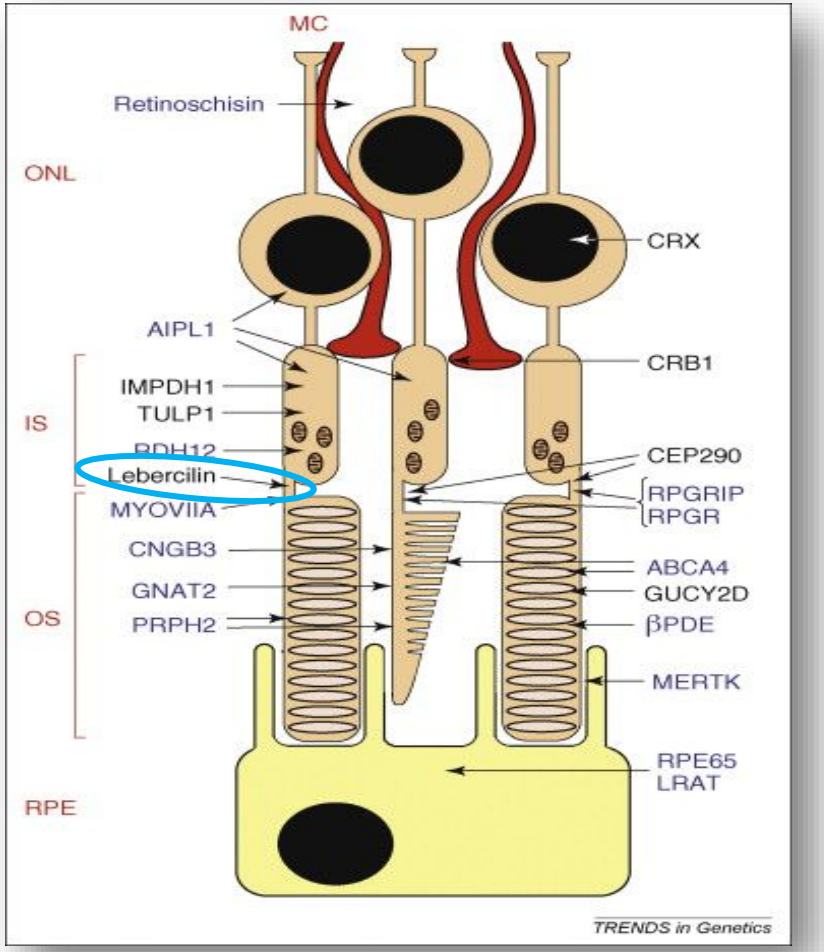
- Prevalence Estimates
- Natural History / Window of Intervention
- Efficient Clinical Trial Execution
- Opus as BD Partner of Choice
- Leverage Across Ecosystem

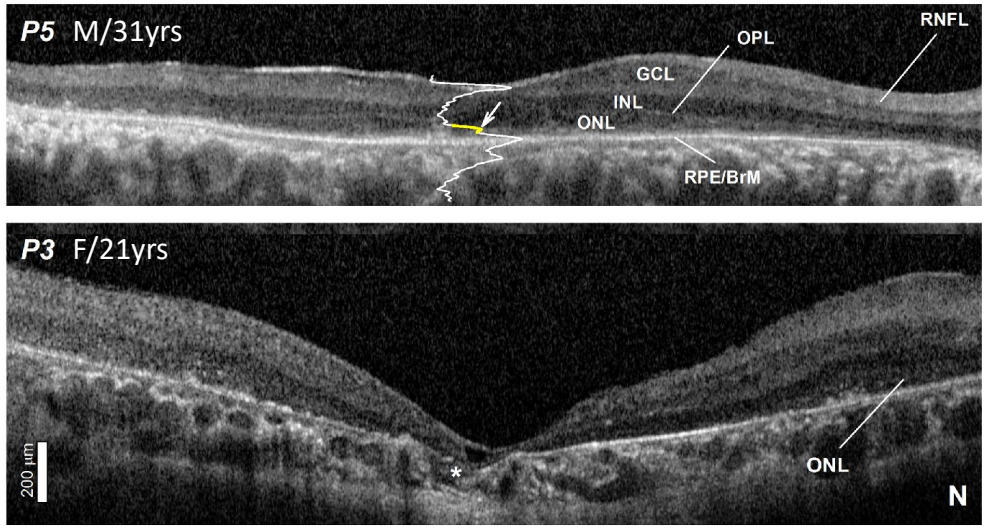
Intellectual Property & Regulatory Designations

- Opus patent protection in the major geographic markets includes a combination of IP
  - Patents licensed from academia
  - Patents internally conceived and developed
- Opus seeks and expects regulatory designations
  - Orphan drug
  - Rare pediatric
  - Breakthrough therapy
  - EU and Japanese equivalents of U.S. designations
- Opportunities for multiple priority review vouchers, if development is successful

LCA5

LCA5: An Early-Onset Retinal Degeneration

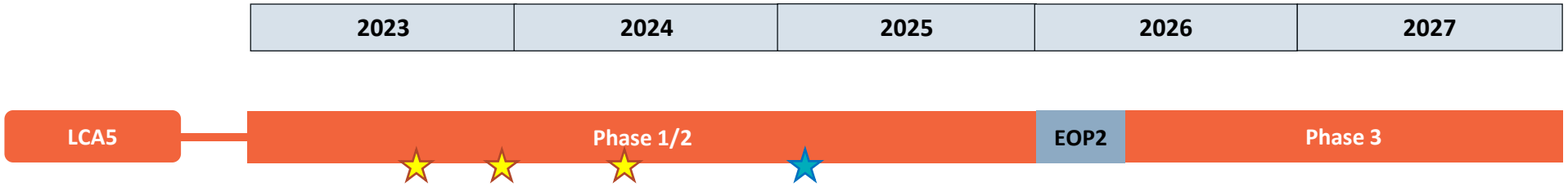




Structural-functional dissociation

Uyhazi et al., IOVS 2020
Boldt et al., JCI 2011

LCA5 Development Plan

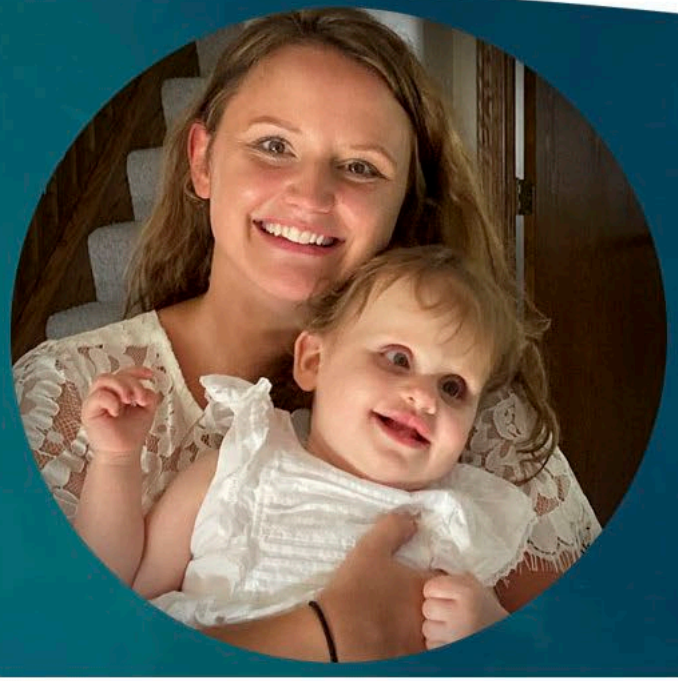


= Adult cohort data

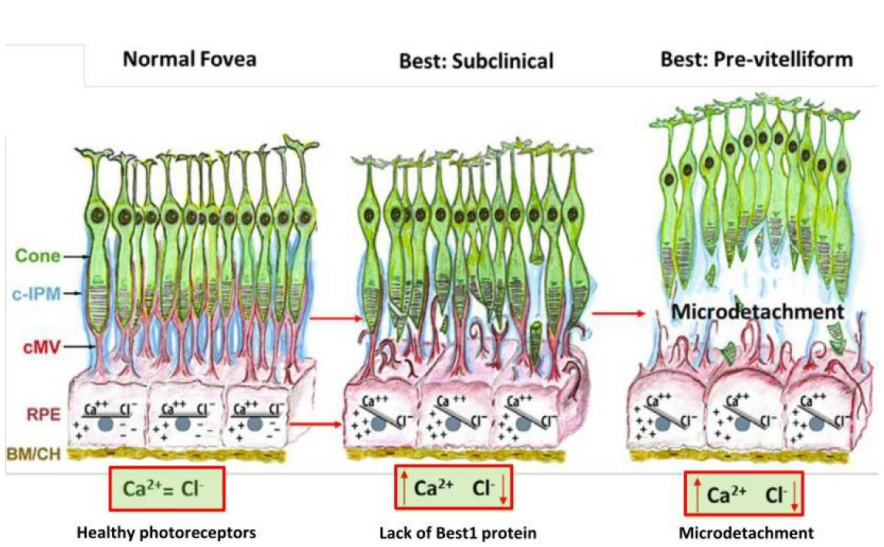
= Pediatric cohort data

- IND submitted and cleared by FDA in 4Q 2022
- Single center, open label Phase 1/2 trial to commence 1Q 2023
  - 3 + 3 + 3 design of three dose levels in adult subjects
  - Once safety is cleared in adults, will amend and proceed with pediatric subjects
- End of Phase 2 meeting to be held with FDA once there is 1 year of safety and durability data from all cohorts

BEST1



BEST1: Biology and Clinical Staging



BVMD

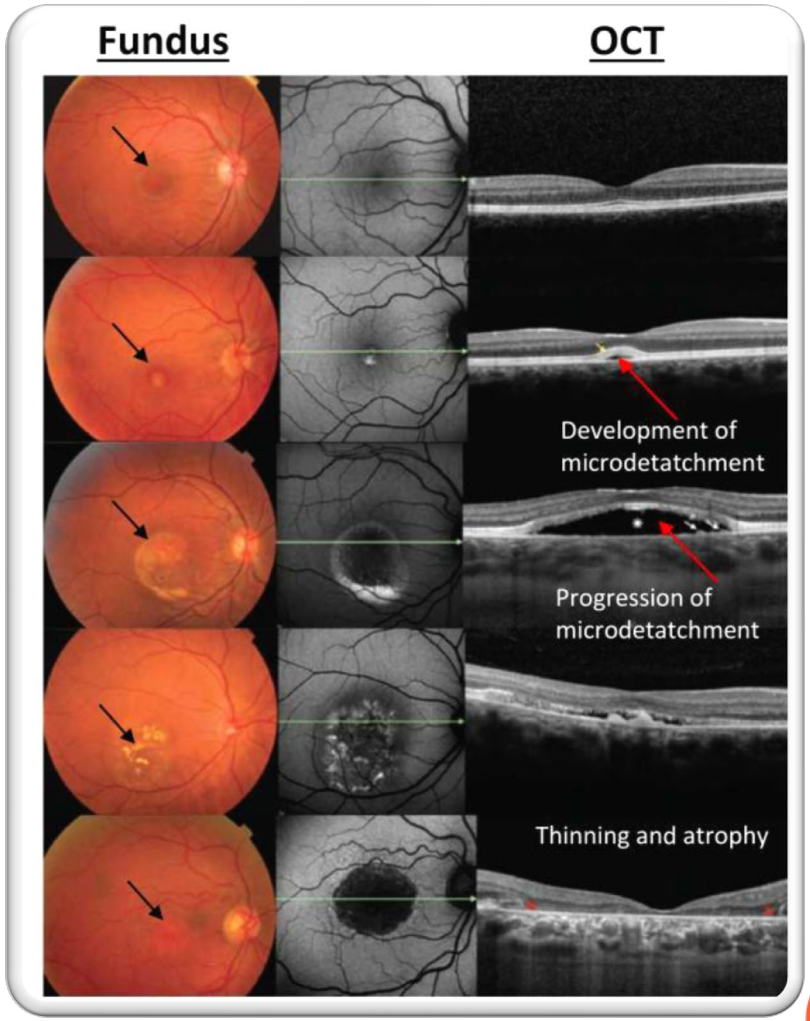
Stage 1: Pre-vitelliform

Stage 2: Vitelliform

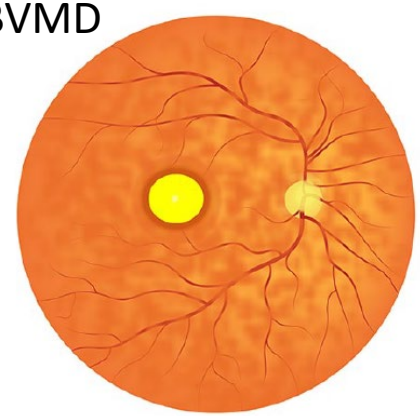
Stage 3: Pseudohypopyon

Stage 4: Vitelliruptive

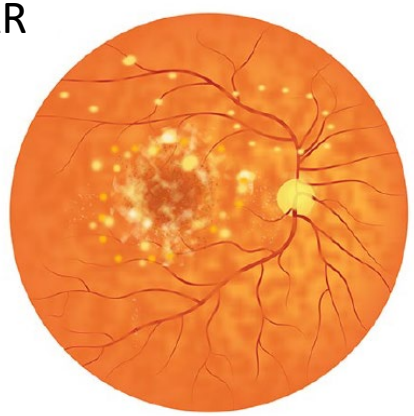
Stage 5: Atrophy/Fibrosis



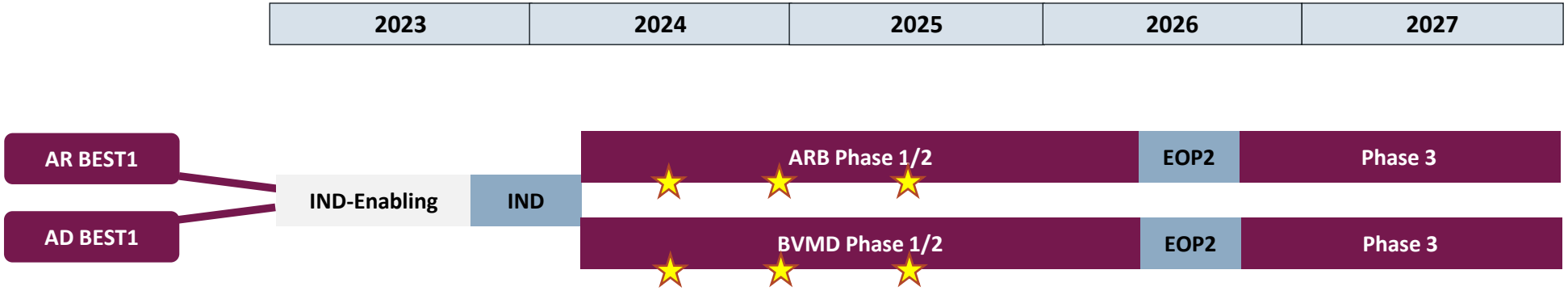
BVMD



AR



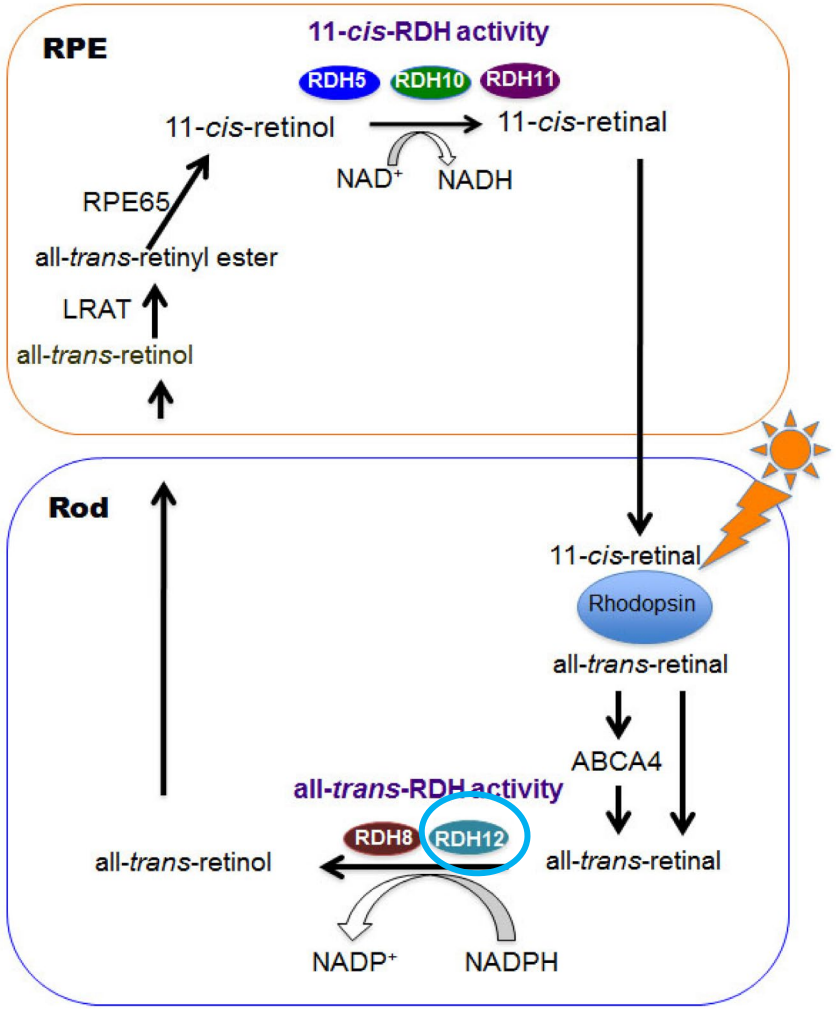
BEST1 Development Plan



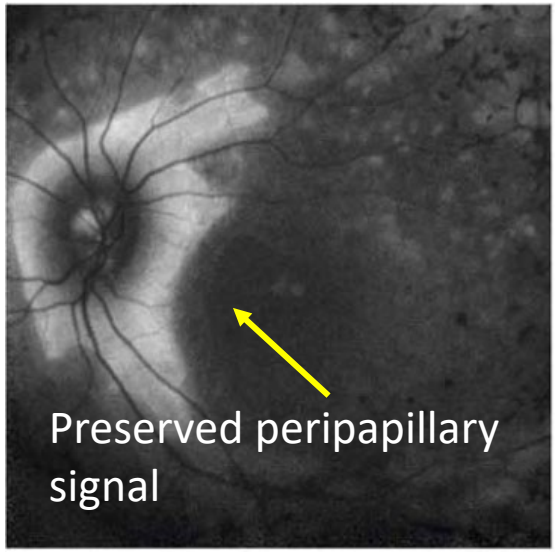
- Pre-IND meeting with FDA held on March 20, 2019
- cGMP clinical batch completed and on stability
- Additional toxicology in 1H 2023 to complete preclinical package
- IND filing 2H 2023
- Uni-Rare natural history study to enroll 40 BEST1 patients
- 2 Parallel Phase 1/2 trials: autosomal recessive and autosomal dominant

RDH12

RDH12: Early Blindness Due to Defect in Visual Cycle Enzyme

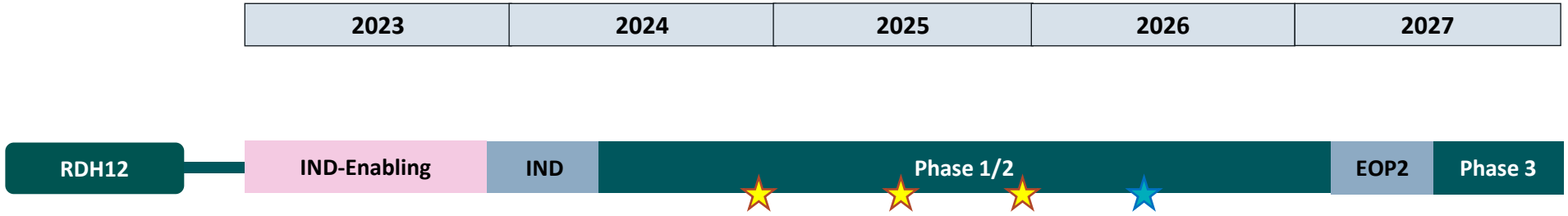


- Success with LUXTURNA® indicates restoration of retinoid cycle can rescue retinal function
- Predictable areas of functional and anatomic preservation can be observed for efficacy signals
- Large, engaged, existing cohort at primary recruitment site



Aleman et al, IOVS 59:5225 (2018)

RDH12 Development Plan

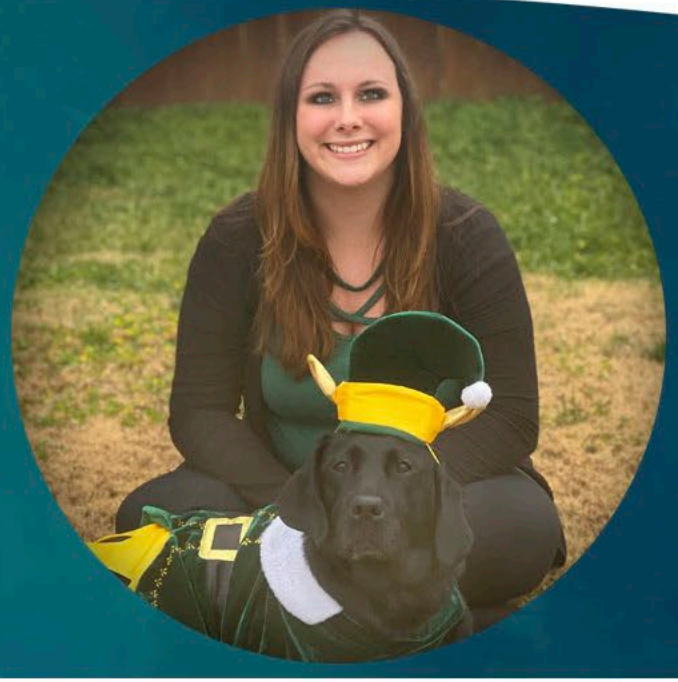


= Adult cohort data

= Pediatric cohort data

- Pre-IND meeting with FDA held in June 2022
- Manufacturing proceeding at CDMO Resilience
- GLP toxicology in NHPs to commence 2Q 2023
- cGMP clinical batch end of 2023
- IND filing 1H 2024
- Uni-Rare natural history study to enroll 20 RDH12 patients

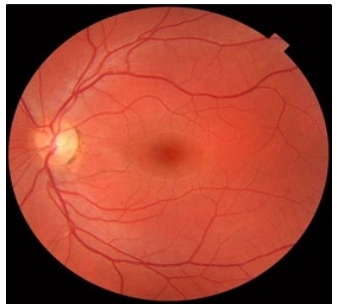
Preclinical & Research Programs



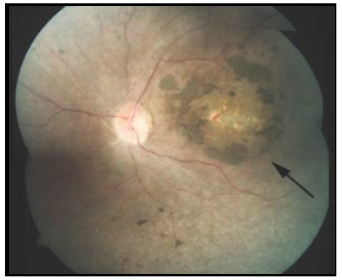
Preclinical Programs: NMNAT1 and RHO

NMNAT1 modulates retinal NAD^{+}, critical for photoreceptor maturation and function

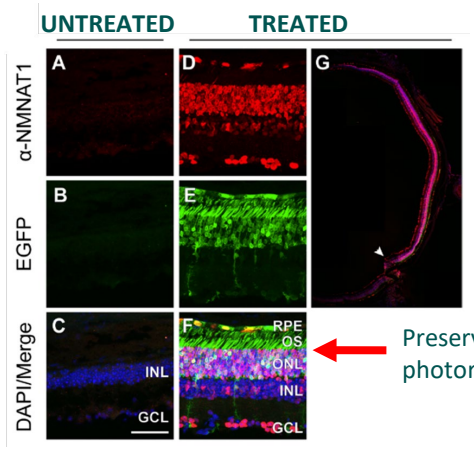
Healthy patient

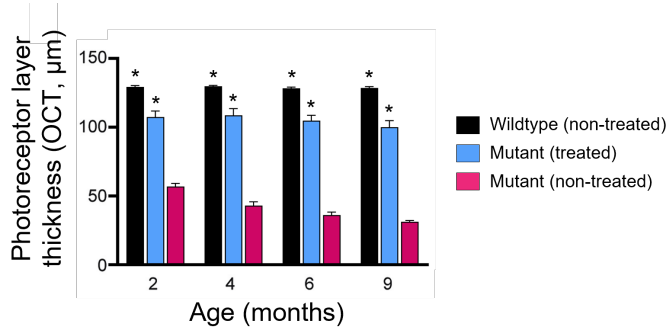


NMNAT1 patient



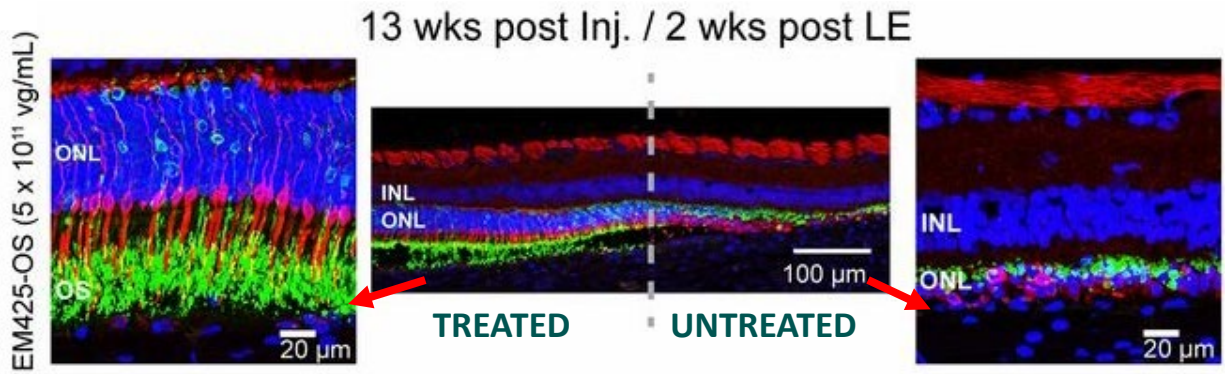
Gene augmentation therapy preserves photoreceptor structure in *NMNAT1* mutant mouse model of IRD

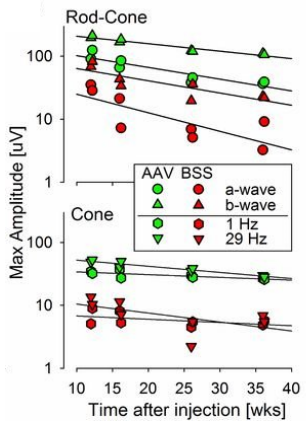




Falk et al, Nature Genetics (2012)
Greenwald et al., Molecular Therapy – Methods & Clinical Development (2020)
Greenwald et al, Human Molecular Genetics (2021)

Proof of concept in mutation-independent treatment of **autosomal dominant RP (adRP)** using a adRP-RHO canine model comparing AAV- and vehicle-treated retinas





- Improvement in photoreceptor viability and morphology in treated eyes 13 weeks post-AAV injection (top)
- Improvement in electroretinography (ERG) readouts of visual function in AAV-treated eyes compared to vehicle (BSS, left)

Cideciyan et al., PNAS 2018

Focused Internal Research Engine

- Dedicated laboratory for AAV validation using *in vitro* and *in vivo* IRD models
  - Retinal organoids and transgenic mice
  - Subretinal delivery
  - Retinal imaging (OCT, fundus photography)
  - Visual function assays (ERG, optokinetics)
  - Immunohistochemistry and microscopy
- In-house, co-located vivarium for animal breeding and *in vivo* procedures
- Analytical method development (e.g. potency assays, biodistribution)
- Generate strong IND-ready preclinical packages to support each program

Dedicated research space in Alexandria Center for Advanced Technologies, Research Triangle Park, NC

Focused, Efficient and Scalable Business Model

R&D/CMC

Efficient Product Development

Commercialization

~$60 - $100M Potential Revenue per 100 Patients Treated (U.S.)

- Small clinical trials
- Small number of clinical sites
- Open label phase 1/2 with early safety & efficacy read
- Limited/no clinical trial competition

- Rare disease category
- Small number of treatment sites
- Small/focused distribution
- Limited/no competition
- Limited commercial infrastructure/expenses
- Multiple regulatory exclusivity designations

Building on Stable Base of Investor Funding

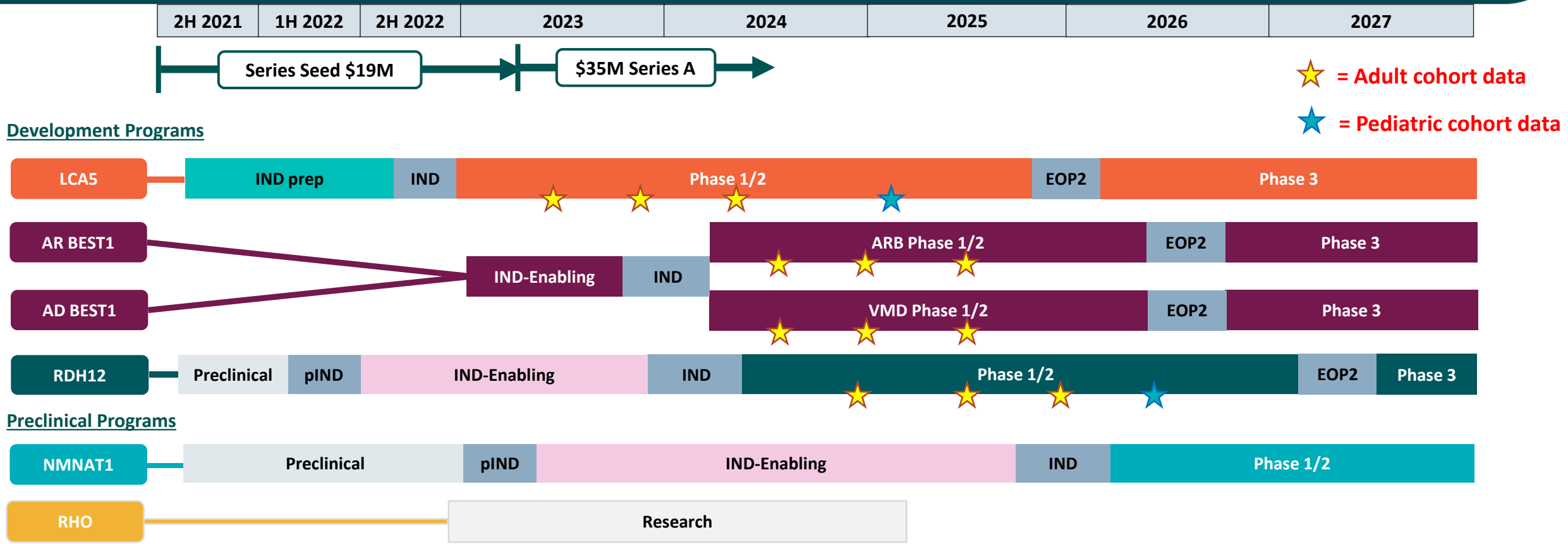
- $19M Seed round completed in Sept. 2021
  - Foundation Fighting Blindness RD Fund
  - Manning Family Foundation
  - Bios Partners
- >$4M in cash at beginning of 2023
- $35M Series A in progress for 1Q 2023 closing
  - Provides runway to mid-2024 and several key clinical data points
- Seeking nondilutive capital to extend runway and support pipeline

RD FUND

MANNING FAMILY FOUNDATION

Bios | Partners

Wholly Owned Pipeline



Undisclosed Research Programs

OPUS GENETICS

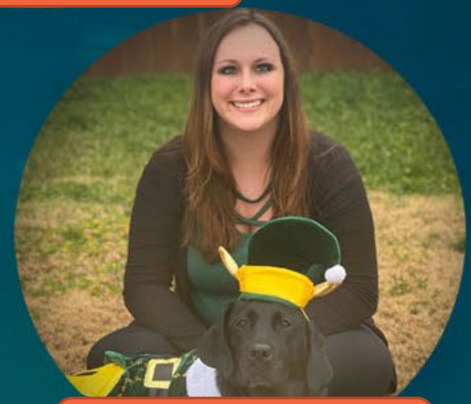
Braydon RDH12

Bella RDH12

Abigail RDH12

Maci NMNAT1 with Mom Jenna

Alan LCA5



Kendall RDH12 with Maya

For BD/Investor Contact:
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