



# Kiora Pharmaceuticals, Inc.

NASDAQ: KPRX

————— Q4 2023 | Corporate Overview

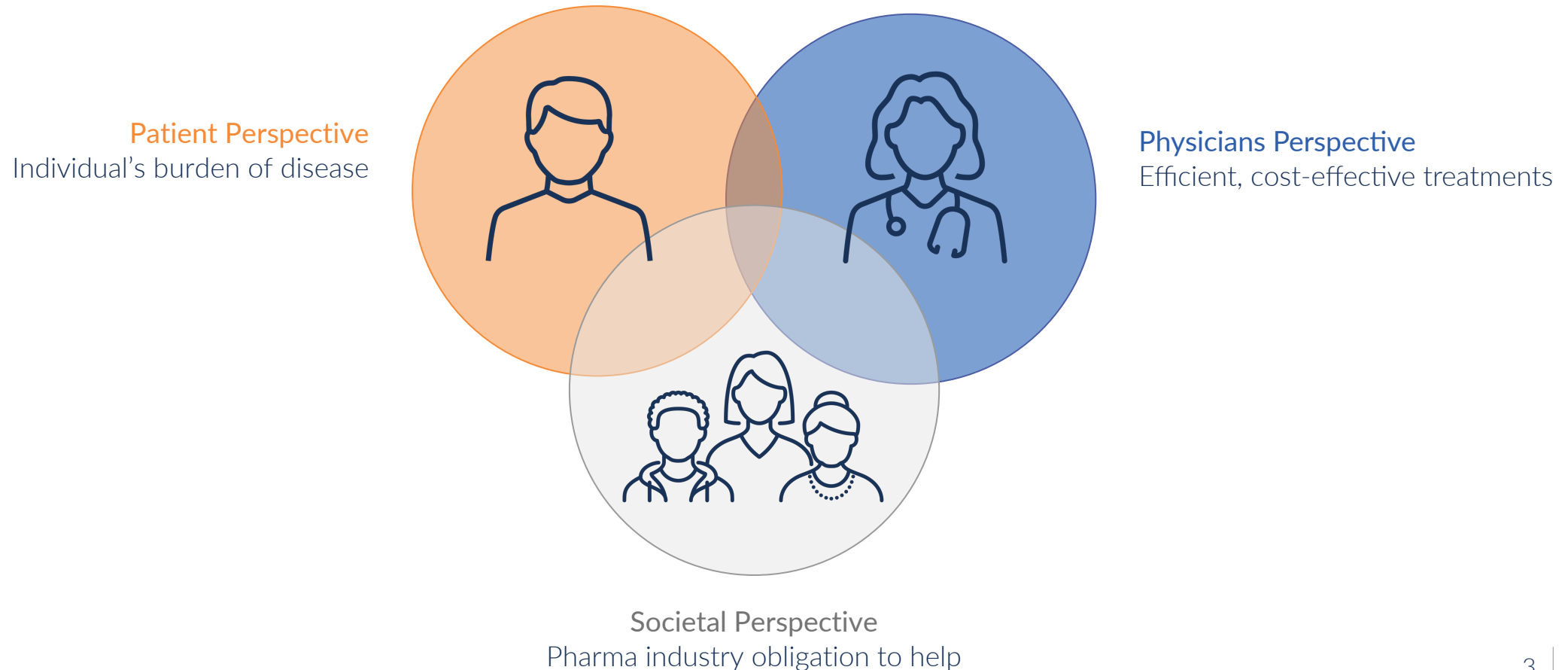


# Forward Looking Statements

Some of the statements in this presentation are "forward-looking" and are made pursuant to the safe harbor provision of the Private Securities Litigation Reform Act of 1995. These "forward-looking" statements include statements relating to, among other things, the development and commercialization efforts and other regulatory or marketing approval efforts pertaining to Kiora's development-stage products, including KIO-301 and KIO-104, as well as the success thereof, with such approvals or success may not be obtained or achieved on a timely basis or at all, the potential ability of KIO-301 to restore vision in patients with RP, the expecting timing of enrollment, dosing and topline results for the ABACUS study, the ability to develop KIO-301 for Choroideremia and Stargardt Disease and KIO-104 for posterior non-infectious uveitis, the ability to utilize strategic relationships to develop certain product candidates, Kiora's ability to draw on its equity line of credit, and Kiora's ability to achieve the specific milestones described herein. These statements involve risks and uncertainties that may cause results to differ materially from the statements set forth in this presentation, including, among other things, the ability to conduct clinical trials on a timely basis, the ability to obtain any required regulatory approvals, market and other conditions and certain risk factors described under the heading "Risk Factors" contained in Kiora's Annual Report on Form 10-K filed with the SEC on March 23, 2023, or described in Kiora's other public filings. Kiora's results may also be affected by factors of which Kiora is not currently aware. The forward-looking statements in this presentation speak only as of the date of this presentation. Kiora expressly disclaims any obligation or undertaking to release publicly any updates or revisions to such statements to reflect any change in its expectations with regard thereto or any changes in the events, conditions, or circumstances on which any such statement is based, except as required by law.

# Sharpened Focus on Orphan Retinal Diseases

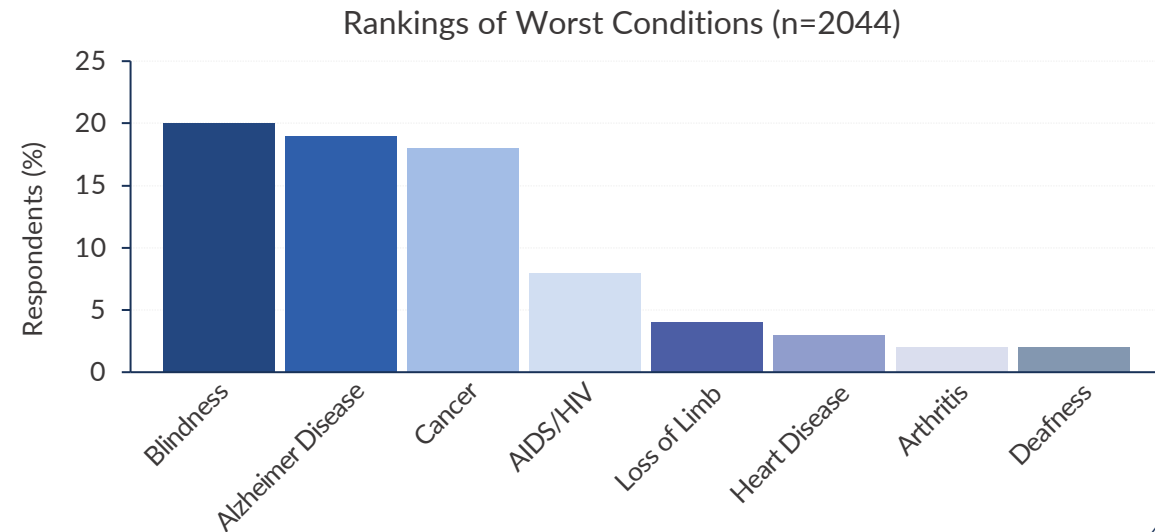
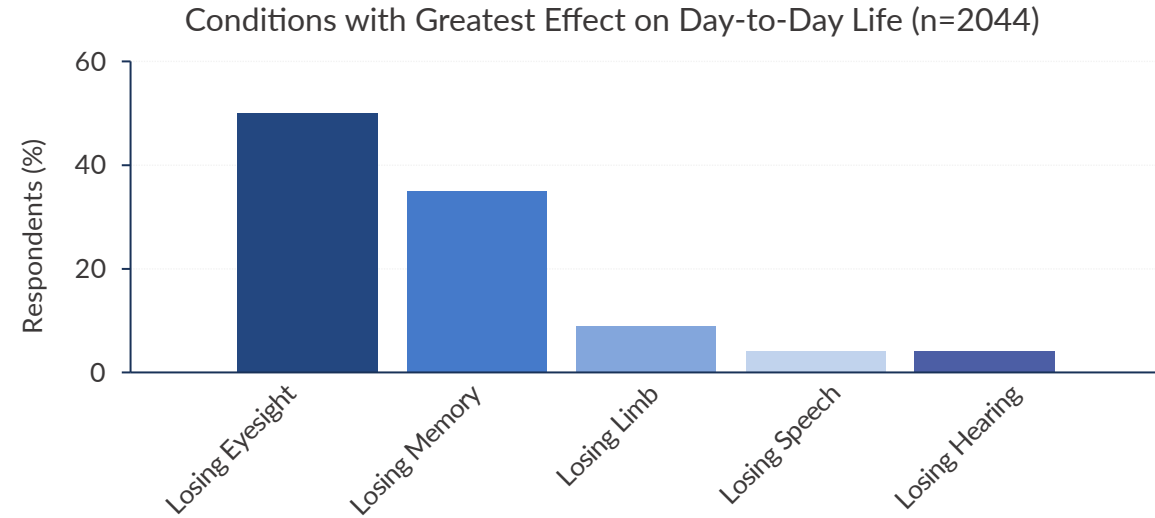
Kiora is developing retinal therapeutics to improve sight in patients with severe vision loss due to inherited or age-related diseases



## Why Retinal Diseases?

“...the last light sensations faded and the dark discs had finally overwhelmed me. I had fought them bravely, as it seemed to me, for thirty-six years, but to no avail. It was then I began to sink into the deep ocean, and finally learn how to touch the rock on the far side of despair.”

- John M. Hull, *Touching the Rock*

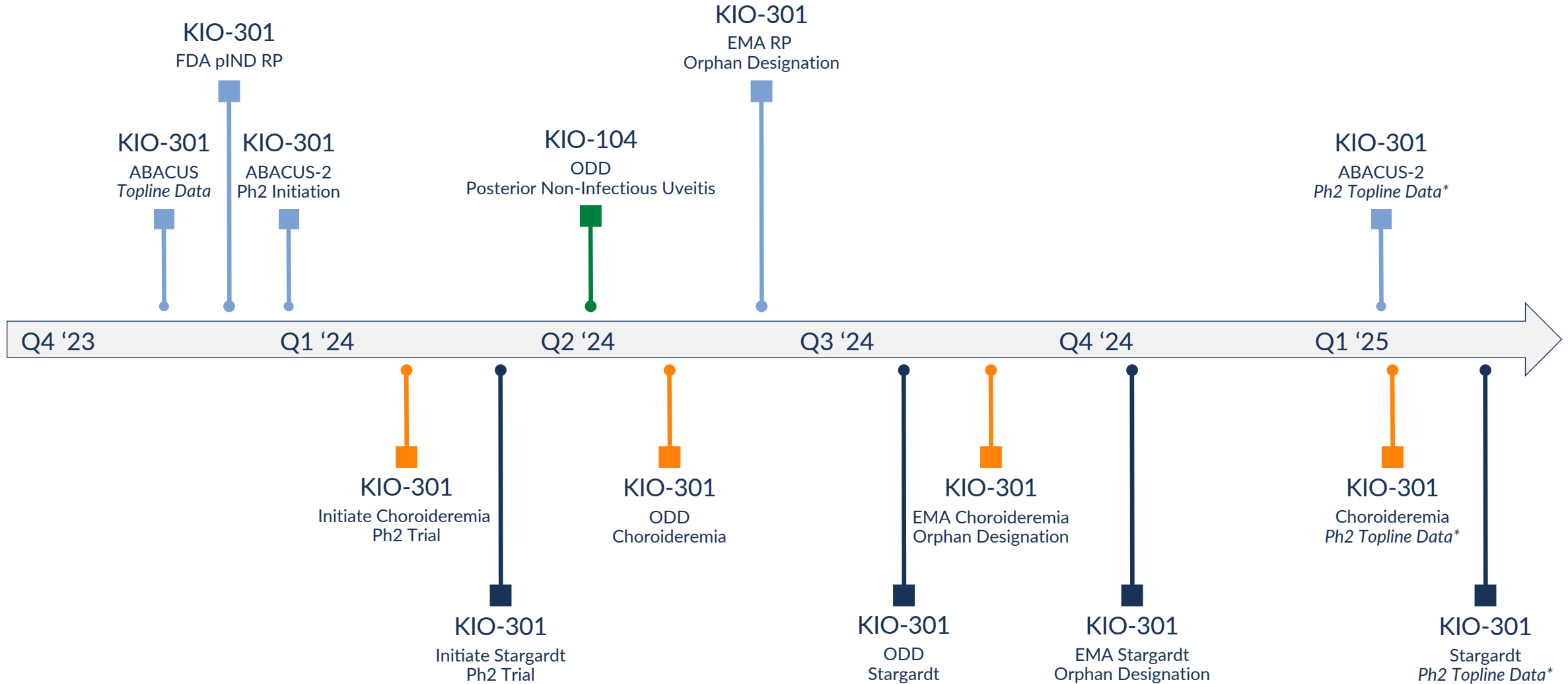


# Pipeline

Product Route of Delivery	Indication	Development Stage				Prevalence* (US, EU5, JP)
		Pre-clinical	Phase 1	Phase 2	Phase 3	
KIO-301 Intravitreal	Retinitis Pigmentosa (Mutation Agnostic)	Granted Orphan Drug Designation (USA) – Mar 2022				250,000
	Choroideremia					16,000
	Stargardt Disease					99,000
KIO-104 Intravitreal	Posterior Non-Infectious Uveitis	Granted Orphan Drug Designation (EU) – May 2015				180,000

\* Approximate 2023 populations.  
Orpha.net, NORD, Ophthalmol Ther 2021 Sep; 10(3)

# Upcoming Clinical/Regulatory Milestones



\* Excludes open label extension  
 RP – Retinitis Pigmentosa, ODD – Orphan Drug Designation (USA), EMA – European Medicines Agency



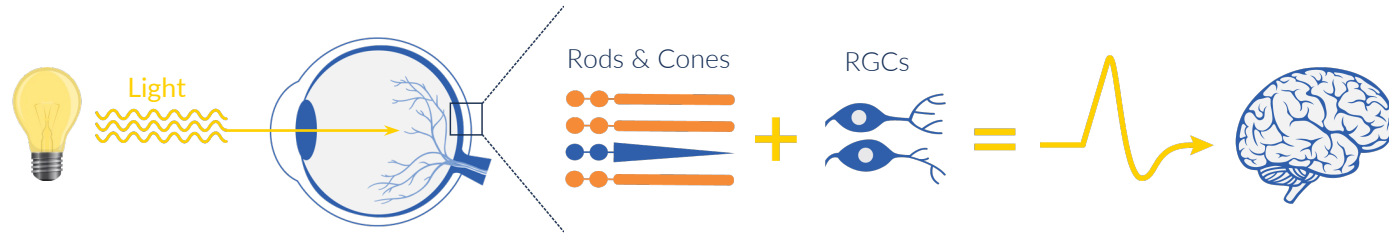
# KIO-301

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Small Molecule Targeting Vision Restoration

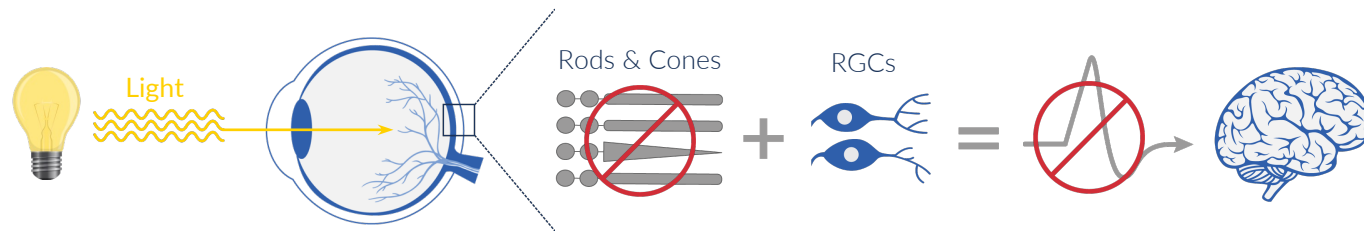
Retinitis Pigmentosa, Choroideremia, Stargardt Disease

# Inherited Retinal Diseases Lead to Loss of Vision



## Healthy Vision

- Rods and cones, the photoreceptors of the retina, process light and relay an electrical signal to downstream cells.
- One of these cell types, retinal ganglion cells (RGCs), transmit the signal to the visual cortex.
- The visual cortex is the part of the brain where vision is perceived.

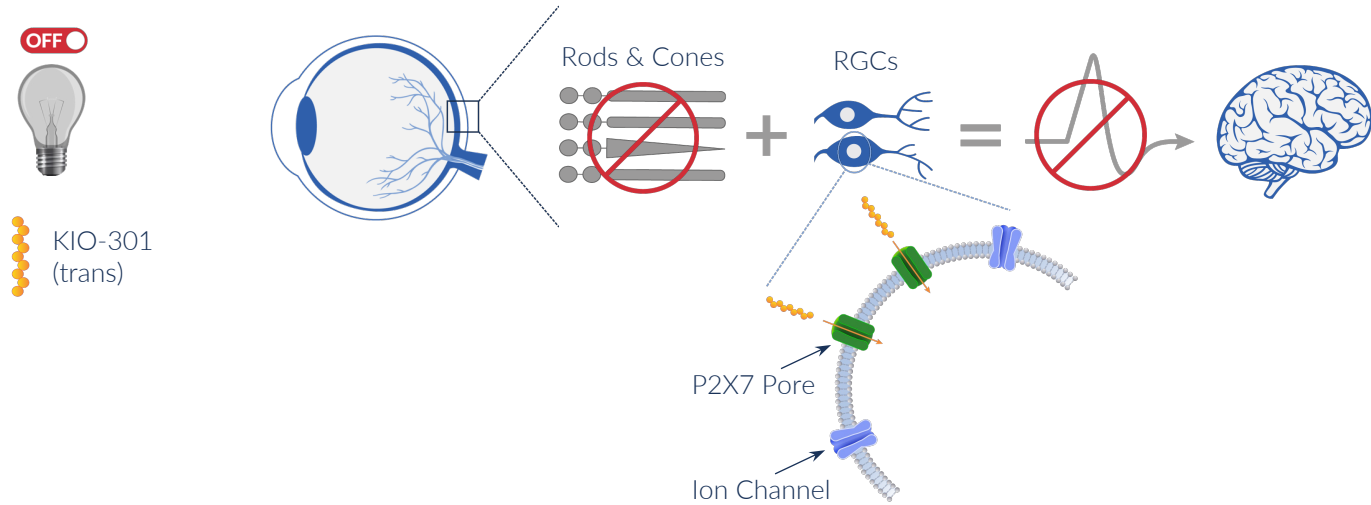


## Damage from Retinitis Pigmentosa

- Retinitis Pigmentosa (RP) results in progressive degeneration and loss of function of rods and cones.
- This causes continuous impairment of vision that often leads to blindness.
- Importantly, in RP and other inherited retinal diseases, the RGCs remain viable.

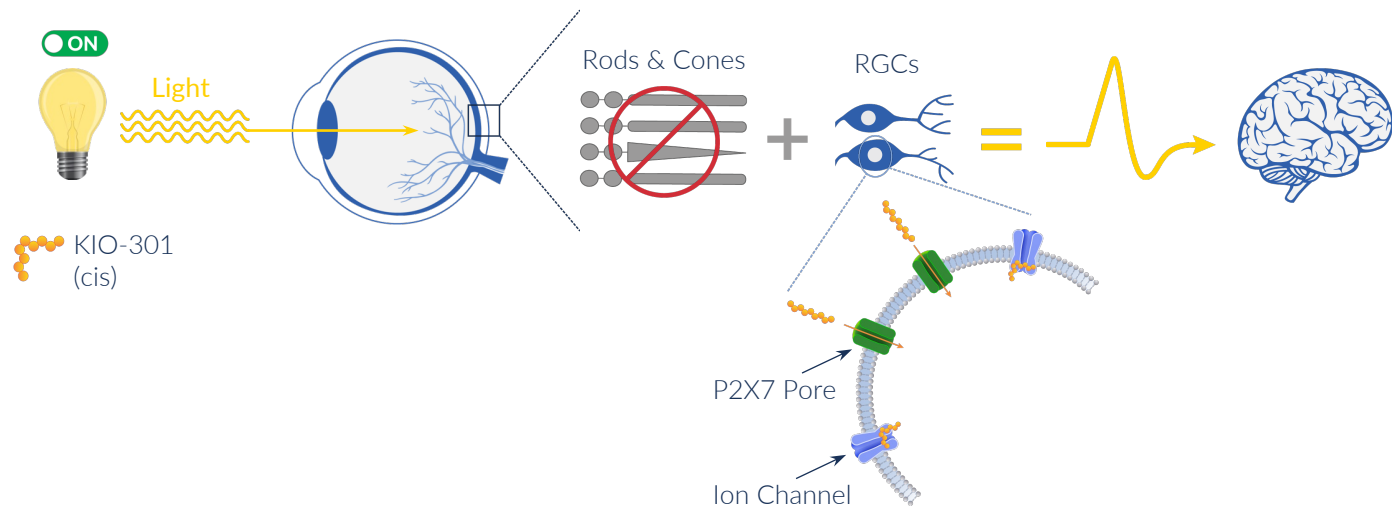


# KIO-301 is a Molecular Photoswitch Designed to Restore Vision



## KIO-301 without Light

- When photoreceptors die, RGCs undergo some remodeling, including expressing specific proteins that allow KIO-301 to selectively enter the cell with ion channels.
- Without light, KIO-301 remains in its linear “off” (trans) position.



## KIO-301 with Light

- With light, KIO-301 is activated and bends into its “on” (cis) formation.
- This physically blocks ion channels and activates the cell to transmit signals to the visual cortex.

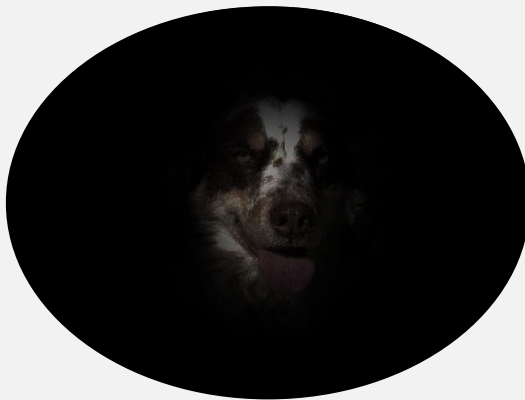
# Retinitis Pigmentosa

## A Disease with No Available Treatments

Normal Vision



Vision Declines over Time



### Clinical Presentation

- Night blindness, reduced visual field range and eventual loss of central vision
- Visual acuity declines
- 50% of patients are not qualified to drive by age 37 and legally blind by 55

### Etiology

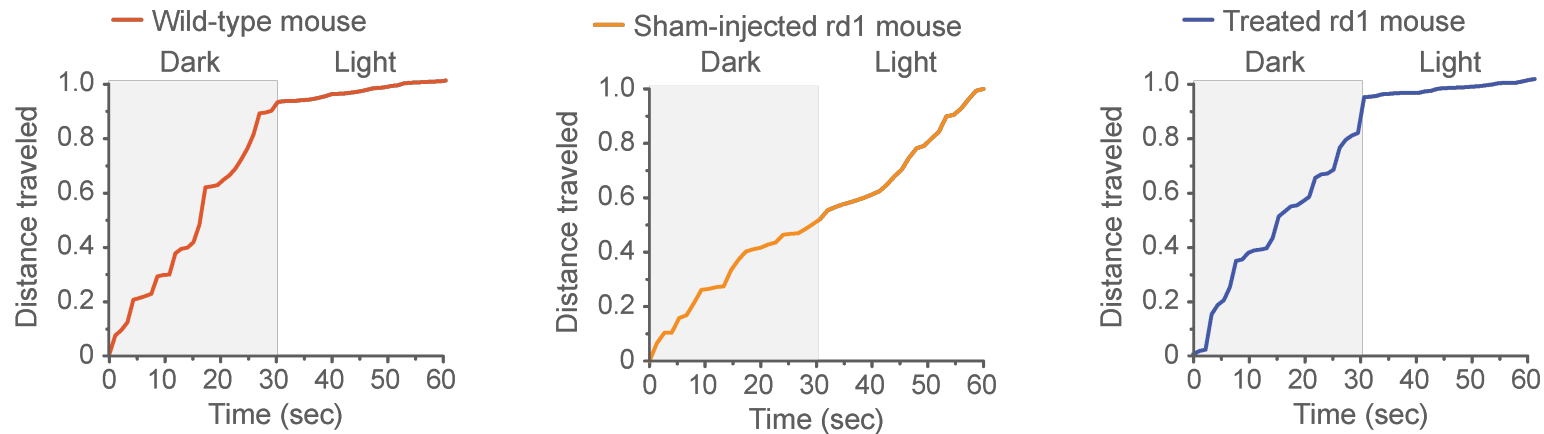
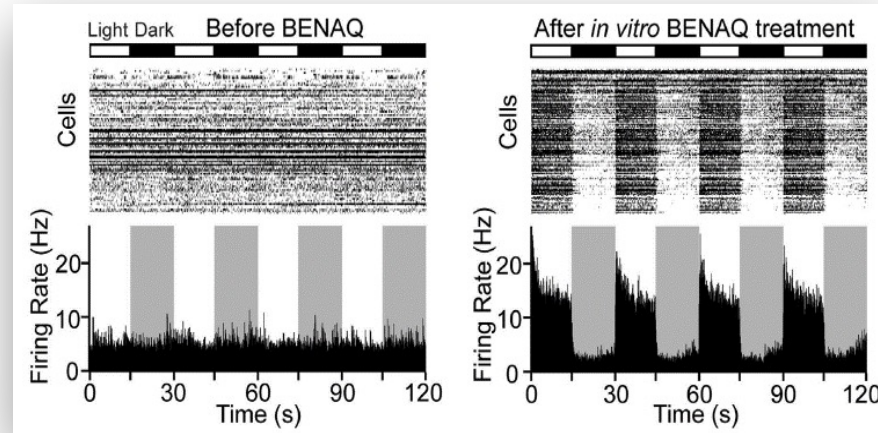
- 50+ genetically distinct subtypes from 150+ mutations
- Inherited disease

### Market Opportunity

- ~100k patients in US (Provider: Retina Specialists [~3k])
- Estimated total cost to US healthcare system in 2019: \$3.7B

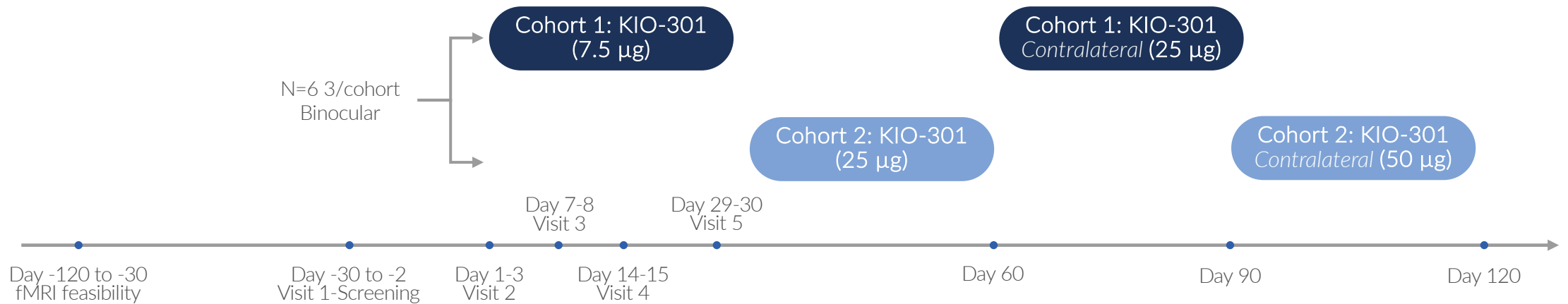
# KIO-301 Reanimates the Retina & Changes Behaviour

## Extensive Validation in Preclinical Models



# KIO-301-1101: Phase 1b Study Design (ABACUS)

Open Label, Single Ascending Dose Trial – 2 Sites (Australia)



## Study Design

- Two cohorts, non-randomized, open-label, single IVT injection per eye
- Cohort 1 – NLP/BLP patients; Cohort 2 – HM/CF patients

## Endpoints

- Primary – AEs, PK & labs
- Secondary – Assessment days (shown only for Cohort 1 above) is repeated for each cohort per eye; intensity & contrast assessment, kinetic perimetry, functional MRI, etc.

## Review

- Safety review conducted by Investigators between after sentinel subject

# — Patient Testimonials



Patient 1-02  
Baseline VA: NLP  
Cohort 1



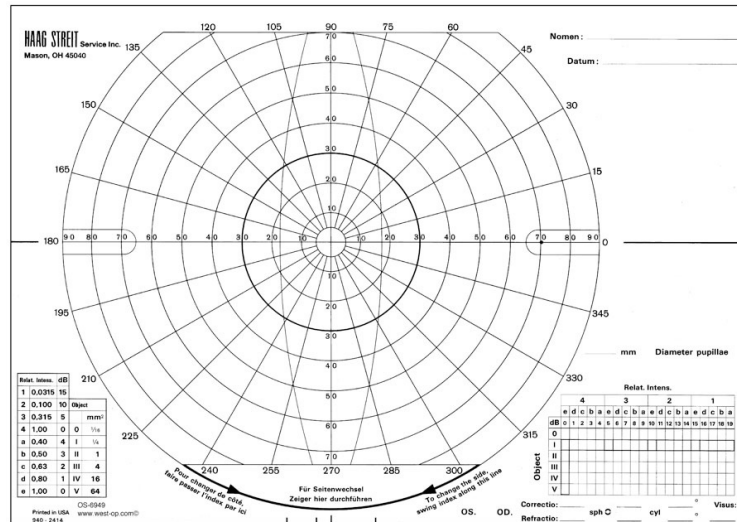
Patient 2-05  
Baseline VA: CF  
Cohort 2



Patient 1-03  
Baseline VA: HM  
Cohort 2

Videos also available at: <https://kiorapharma.com/technology/kio-301/>

# Kinetic Visual Field (Goldmann Perimetry)



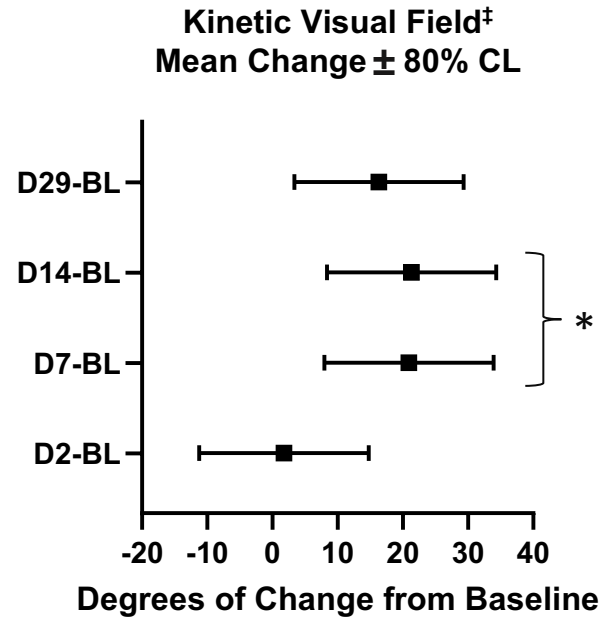
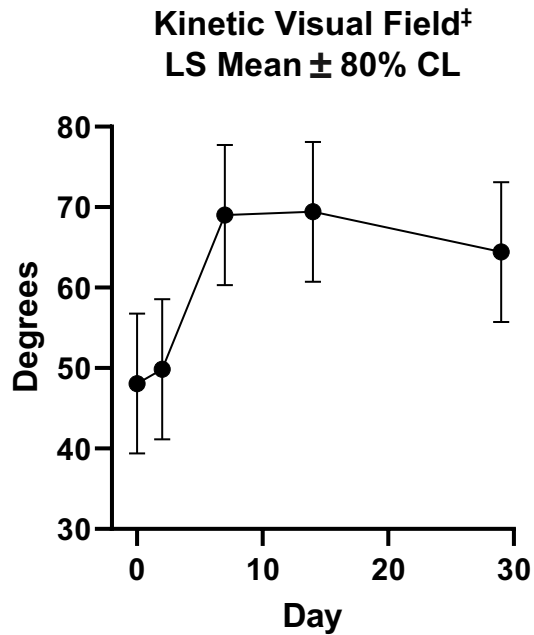
Aim: Evaluate Peripheral Vision at a Basic Level

Assessment & Insights:

- Applicability in this population
- Performed by experienced orthoptists
- Limited to 2-axis
- The patient is asked to acknowledge (using a buzzer) when light stimulus is visualized within the dome
- Method facilitates limitation of fixation
  - > Proof-of-feasibility achieved
  - > Will expand scope of evaluation to capture increased degrees

# Kinetic Visual Field

## KIO-301 May Improve Visual Field



\*p<0.05

## Kinetic Visual Field

- Goldmann perimetry
- Performed at baseline (BL), and each study visit
- Performed by same group of orthoptists to reduce variability
- Greater improvement observed in Cohort 2a

<sup>‡</sup> Cohort 2 includes 3 patients (6 eyes)



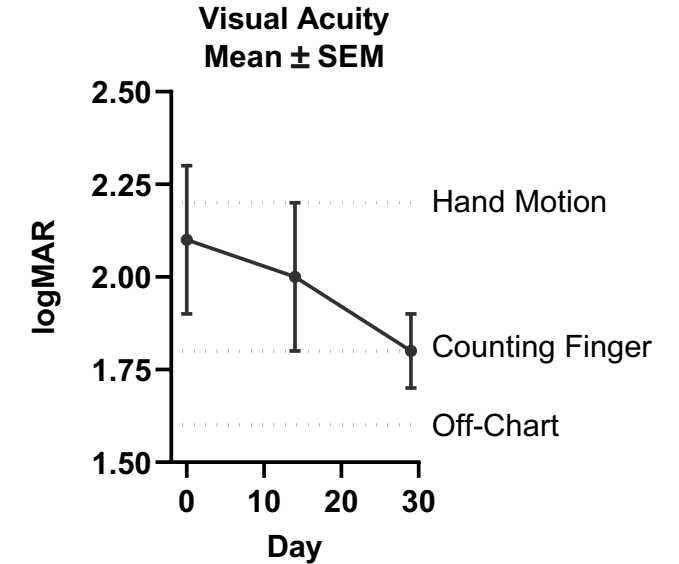
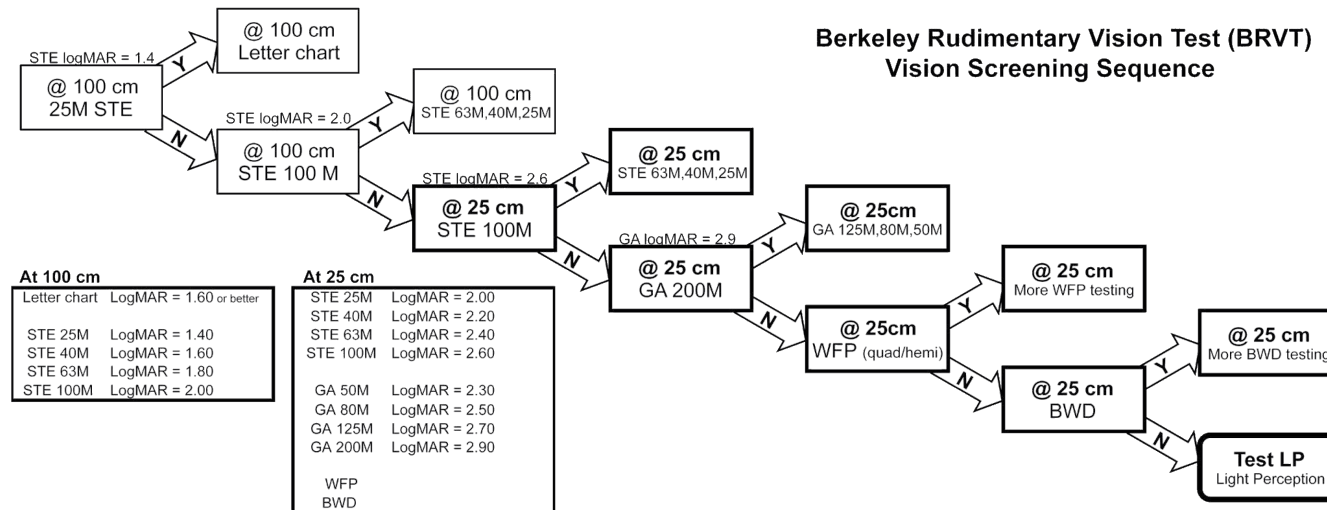
# Visual Acuity – Berkeley Rudimentary Vision Test (BRVT)

1040-5488/12/8909-1257/0 VOL. 89, NO. 9, PP. 1257-1264  
OPTOMETRY AND VISION SCIENCE  
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ORIGINAL ARTICLE

## The Berkeley Rudimentary Vision Test

Ian L. Bailey\*, A. Jonathan Jackson†, Hasan Minto‡, Robert B. Greer‡, and Marlena A. Chu§



Cohort 2 (3 patients, 3 eyes)



# Light Perception (Intensity & Contrast Assessment)



Aim: Evaluate Light Perception at a Basic Level

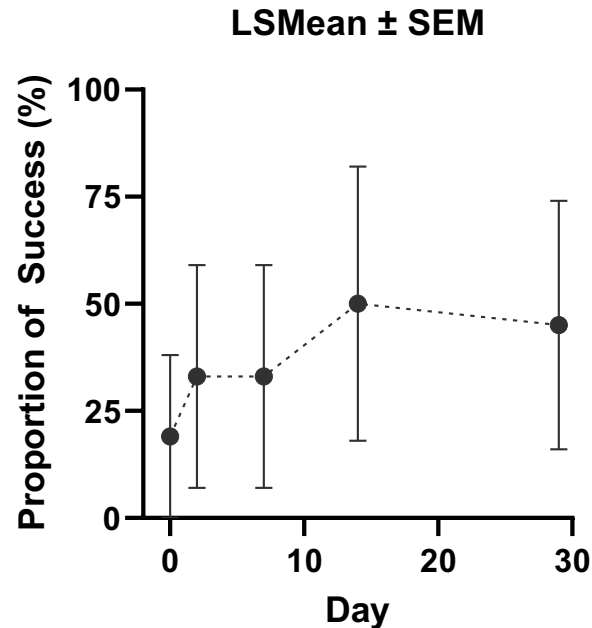
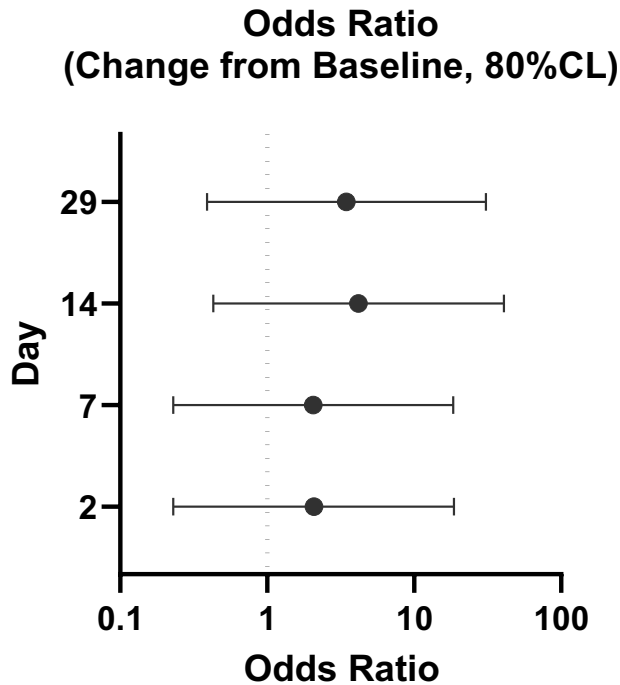
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Assessment:

- Series of visual stimuli (a series of letters are presented on a screen to the patient via a rear projector)
- Binary outcome (yes/no)
- The subject is asked to acknowledge (verbally and/or physically) when a change in light is perceived
- Asked to also identify object, if possible

# Light Perception – Cohort 1

KIO-301 May Improve Light Perception in the NLP/BLP Population



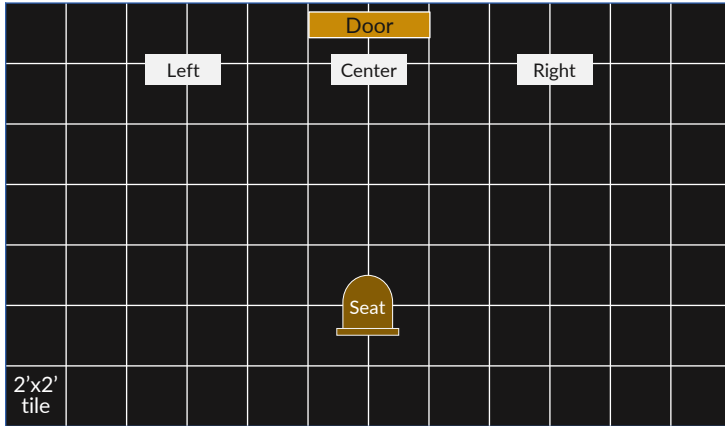
## Light Perception

Insights:

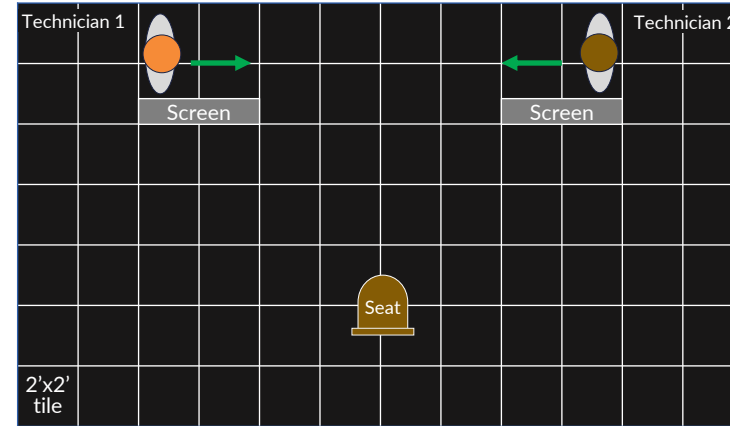
- Cohort 1 subjects demonstrate improved odds ratio on drug
- Odds Ratio – strength of association, OR=2 → 100% increase in the odds of an outcome
  - > e.g., duration of diabetes mellitus (> 15 years) with diabetic retinopathy is >9.0\*
- Cohort 2 subjects are existing light perception patients; therefore, expect little change

# Functional Vision - Multiluminance Orientation & Mobility (MLOM)

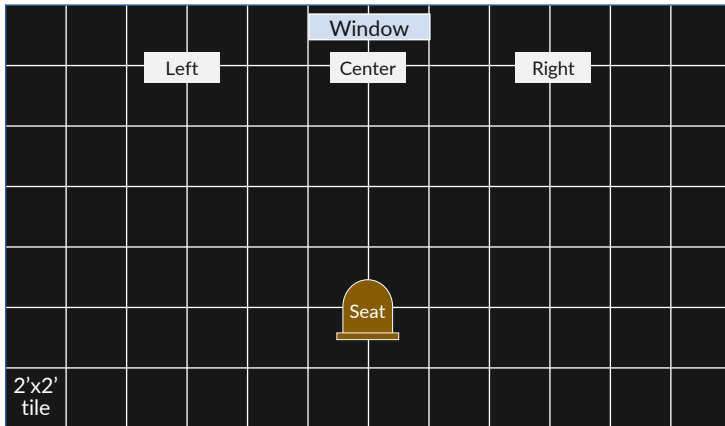
### Door Location Test Setup



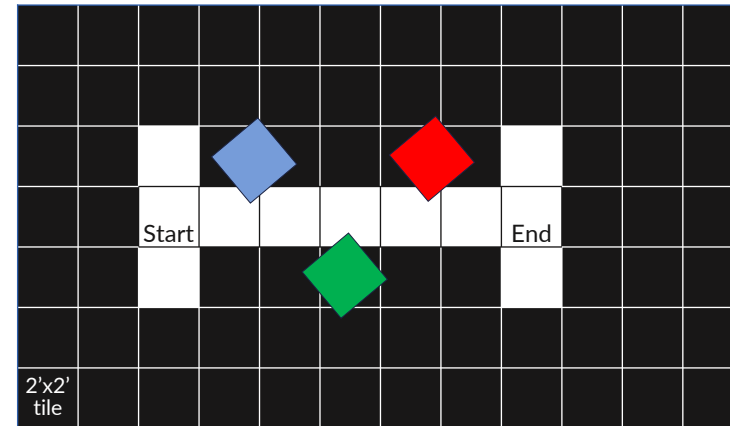
### Walking Direction Test Setup



### Window Location Test Setup



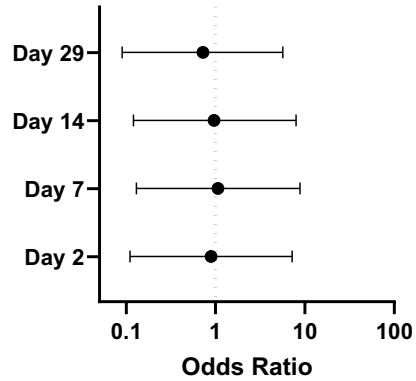
### HCRE Course Setup



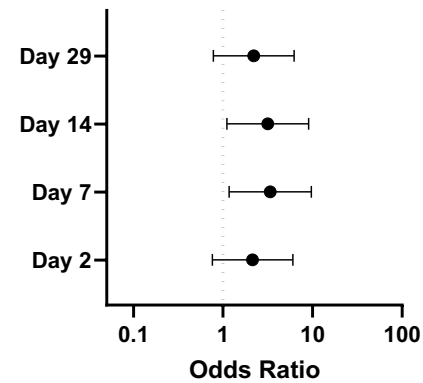
# Functional Vision - Multiluminance Orientation & Mobility (MLOM)

## KIO-301 May Improve Functional Vision

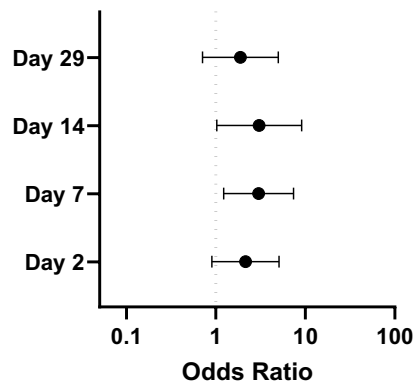
Door Location Test\*  
Change From Baseline  $\pm$  80% CL



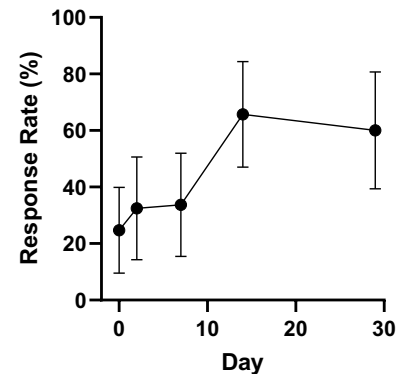
Walking Direction Test<sup>‡</sup>  
Change From Baseline  $\pm$  80% CL



Window Location Test<sup>§</sup>  
Change From Baseline  $\pm$  80% CL



High Contrast Room Exit Test<sup>‡</sup>  
Mean  $\pm$  SEM



## MLOM

### Overview

- First time used in ultra-low vision patients
- Question: "Is this test valuable?"

### Takeaways:

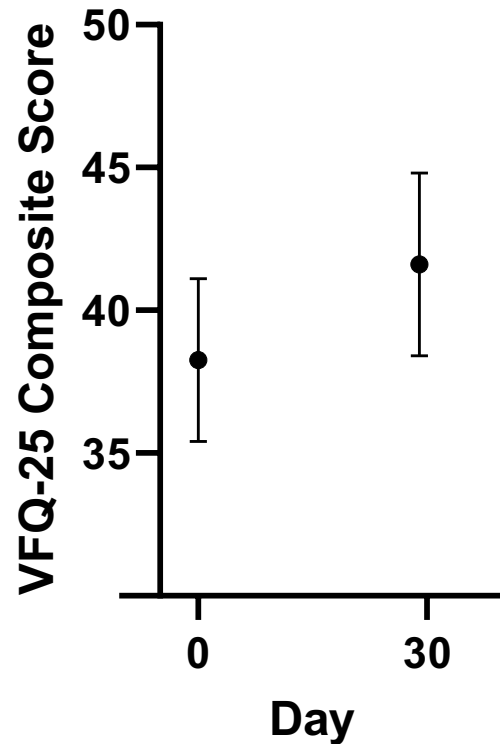
- Important aspect of documenting vision driven movement
- Not all "functional" tests relevant to the population tested
- One or two clinically meaningful functional tests will remain in Phase II
- Will incorporate light-level changes into Phase II

\* Analysis of 4 patients (7 eyes)    ‡ Analysis of 6 patients (10 eyes)  
§ Analysis of 3 patients (5 eyes)    Ora-MLOM™ Suite of Tests

# Visual Function Questionnaire (NEI VFQ-25)

KIO-301 May Improve Patients' Overall Quality of Life

## Quality of Life Survey



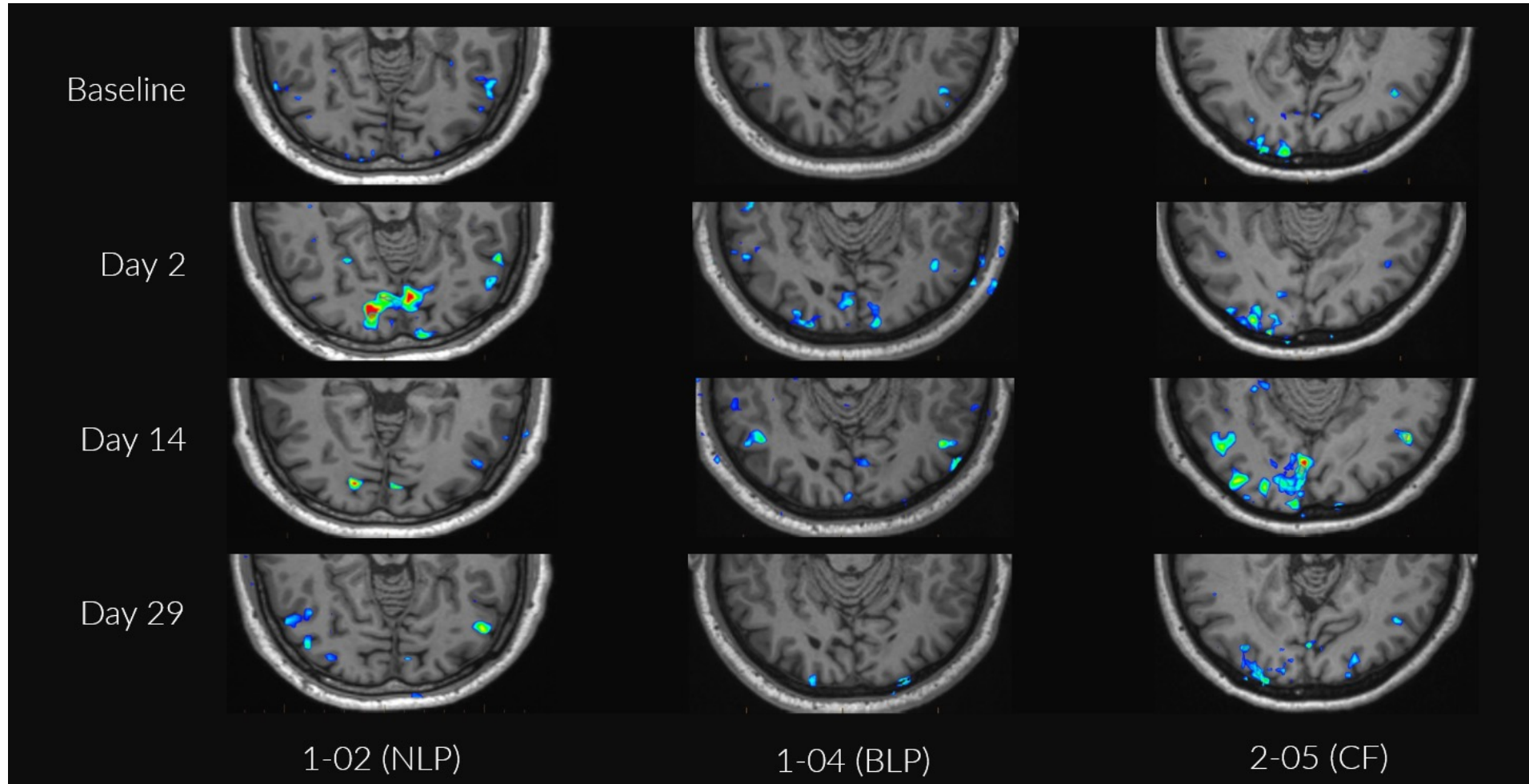
## Quality of Life

National Eye Institute generated survey assesses daily functions related to general health & vision, ocular pain, near & distance activities, social functioning, mental health, dependency, driving, color vision, and peripheral vision.

2-4 point increase is considered clinically meaningful\*

# Functional MRI

Supportive of Cortical Activation



# ABACUS-1 Takeaways

## No Safety & Tolerability Concerns

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1

KIO-301 appears to reanimate the retina

2

Approvable outcome assessments need to be defined

- US FDA pIND scheduled for Q4 2023

3

Patients report improvements in vision

- Consistent with objective clinical assessments
- Follow-on study will include sham group

4

Pilot study limitations

- Non-controlled
- Small sample size

# KIO-301-2101: Phase 2 Study Design (ABACUS-2)

Sham Controlled, Masked, Randomized, Multiple Ascending Dose Trial – 4 Sites (Australia)

## Key Elements

### Study Design ABACUS-2

- Controlled
- N=20 patients
- Includes higher dose\* (100µg)
- Multiple injections over 3 months
- Binocular injections
- Open label extension for controls
- Endpoints incorporating US FDA feedback

### Cohort 1A

5 RP pts with NLP randomized to receive 50µg KIO-301 or sham (3:2) OU monthly for 3 months

### Cohort 1B

5 RP pts with LP randomized to receive 50µg KIO-301 or sham (3:2) OU monthly for 3 months

### Open Label Extension

Patients randomized to control will be eligible to receive 50µg KIO-301 OU monthly for 3 months

### Cohort 2A

5 RP pts with NLP randomized to receive 100µg KIO-301 or sham (3:2) OU monthly for 3 months

### Cohort 2B

5 RP pts with LP randomized to receive 100µg KIO-301 or sham (3:2) OU monthly for 3 months

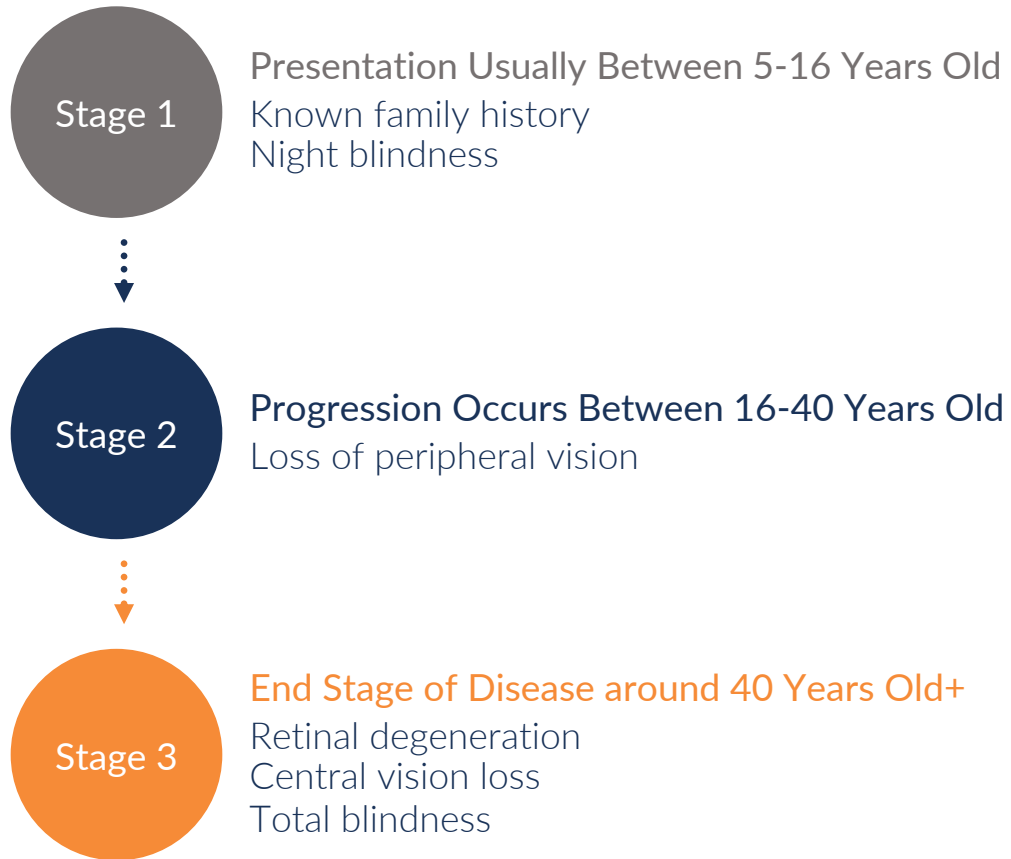
### Open Label Extension

Patients randomized to control will be eligible to receive 100µg KIO-301 OU monthly for 3 months



# Choroideremia: Inherited Disease that Leads to Blindness

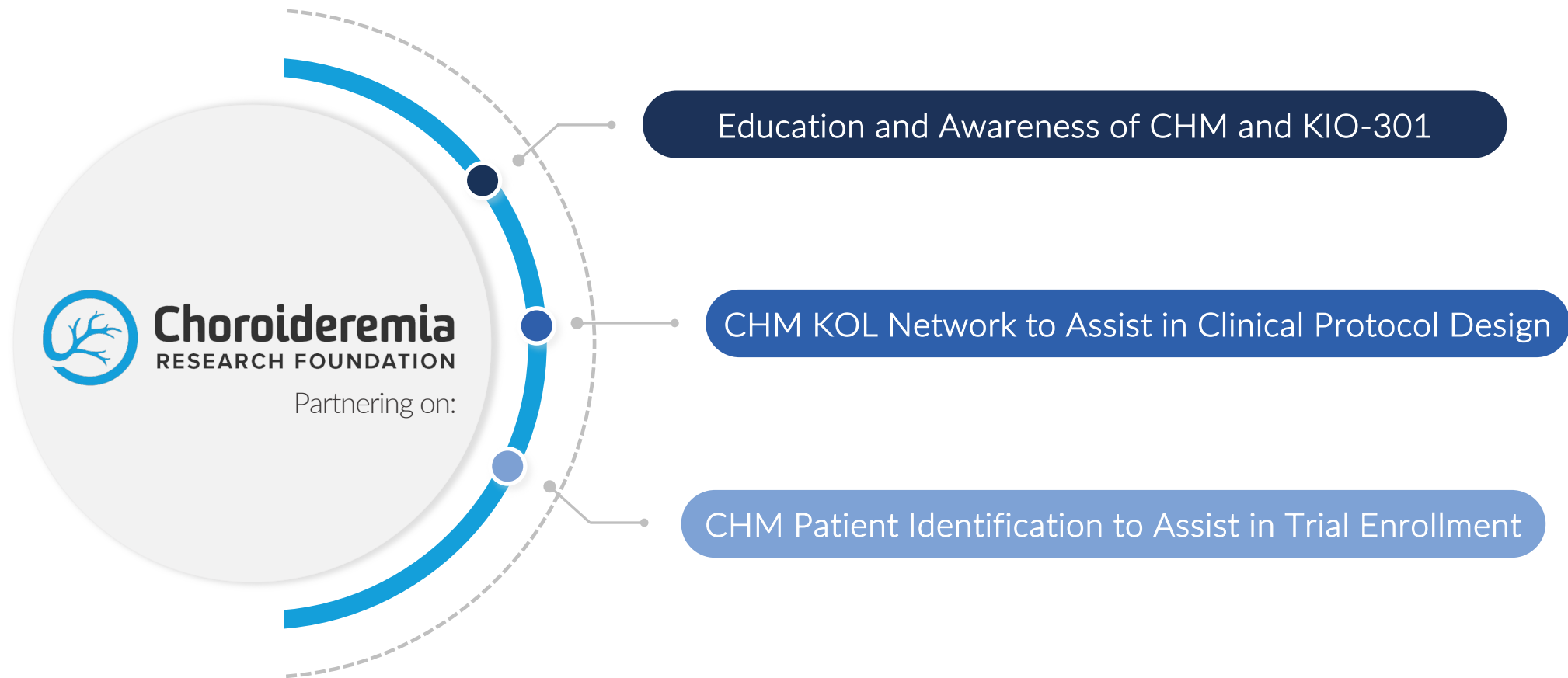
No Approved Therapeutics and Only ONE Active Therapeutic Clinical Trial\*



- **Orphan Disease:** prevalence of 1:50,000, ~12,000 patients in US/EU
- **X-linked** recessive disease primarily affecting males
- **Cause:** Inherited mutation in the Choroideremia (CHM) gene encoding Rab escort protein-1 (REP1)
- **REP1** is involved in the regulation of intracellular trafficking of Rab proteins
- **Vision Loss:** Degeneration in the photoreceptors, retinal pigment epithelium (RPE), and choroid. Retinal ganglion cells remain viable.

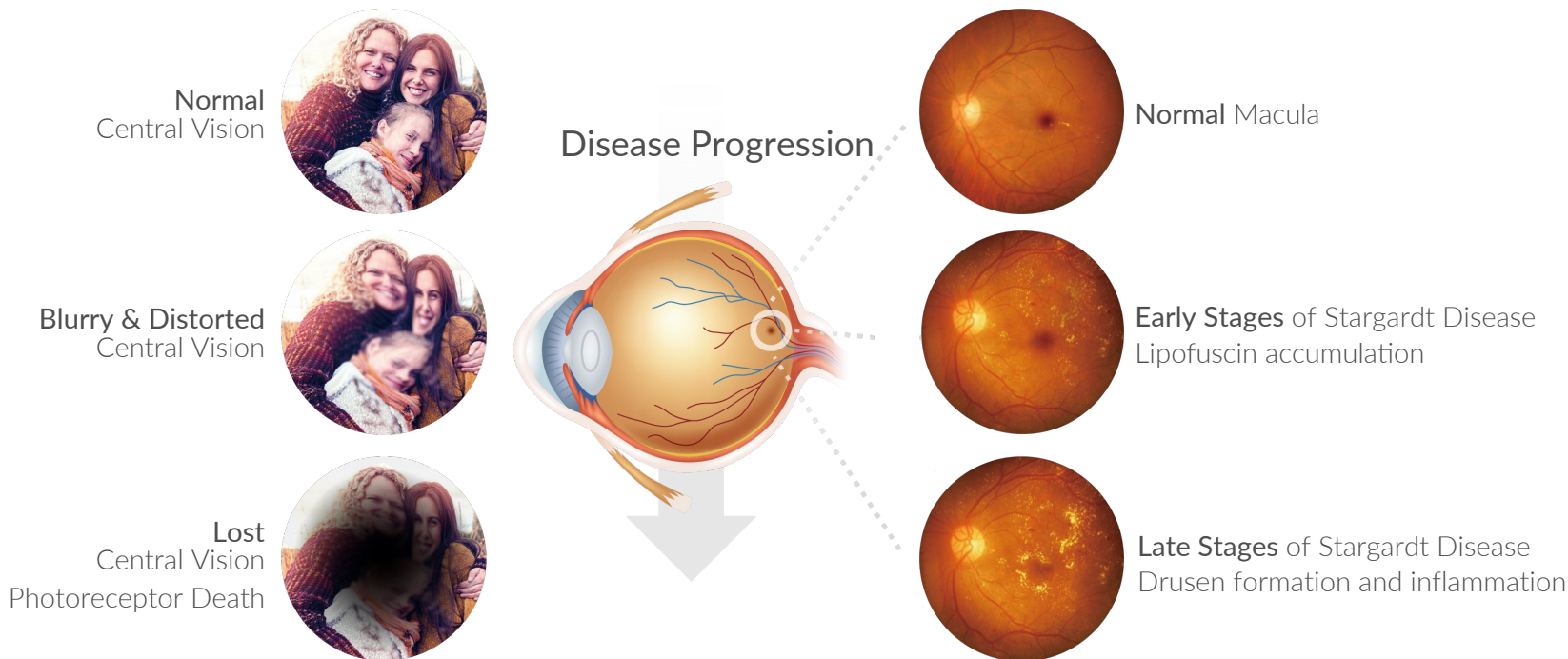
# Partnership with the Choroideremia Research Foundation

The Choroideremia Research Foundation (CRF) is the largest global not-for-profit organization focused on the search for a cure for Choroideremia (CHM).



# Choroideremia: Inherited Disease that Leads to Blindness

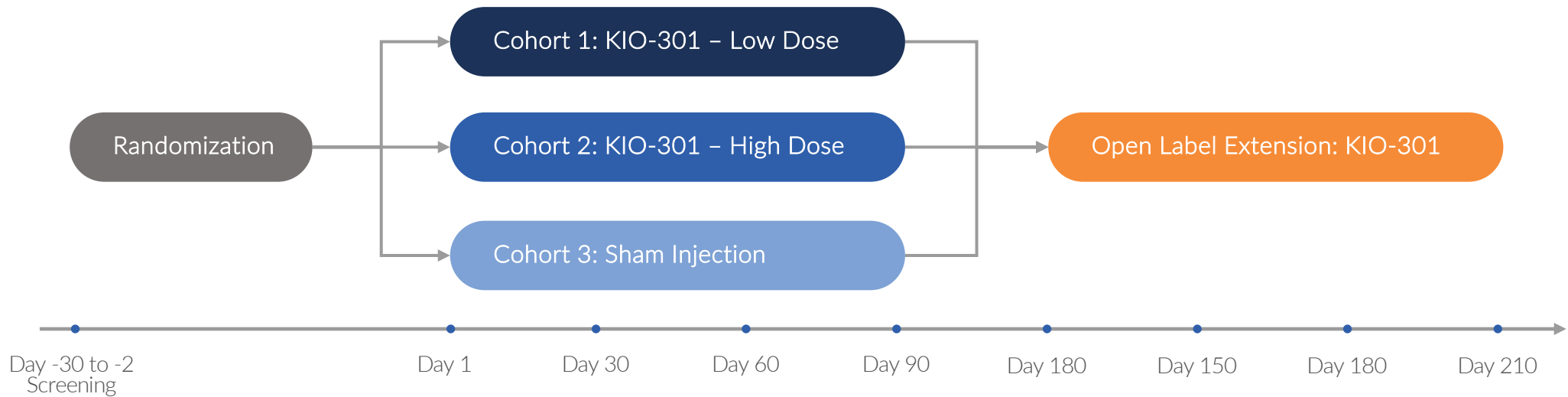
No Approved Therapeutics and Only ONE Active Therapeutic Clinical Trial\*



- **Orphan Disease:** prevalence of 1:10,000 ~30,000 patients in US
- **Autosomal** recessive disease inherited from parent carriers, typical onset in 2<sup>nd</sup> decade of life, vision loss in 4<sup>th</sup>-5<sup>th</sup> decade
- **Cause:** Mutation in the ABCA4 or ELOVL4 gene
- **Accumulation** of lipofuscin plaques in the retinal pigment epithelium (RPE), leading to inflammation and cell death
- **Vision Loss:** Degeneration of the photoreceptors and RPE. Retinal ganglion cells remain viable. Often, some peripheral vision is retained.

# KIO-301-3101: Phase 2 Study Designs (CHM & Stargardt)

## Sham Controlled, Randomized Clinical Trial – Australia



- Study Design
  - Three cohorts, randomized, controlled, multiple bilateral IVT injections (days 1, 30, 60)
- Endpoints
  - Primary – AEs, PK & labs
  - Secondary – Light perception, kinetic perimetry, functional vision, navigation, etc.
- Review
  - Safety review conducted by Investigators after sentinel subject

# KIO-104

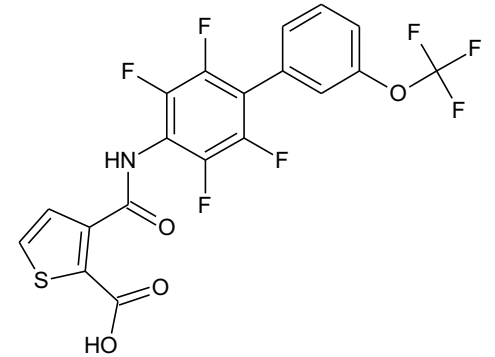
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Intravitreal Small Molecule DHODH Inhibitor  
Steroid Sparing Approach to Retinal Inflammation

# KIO-104 Overview (DHODH Inhibitor)

KIO-104 is an intravitreal, non-steroidal, novel small molecule which mitigates:

- Metabolic activity and proliferation of T-cells
- Secretion of IL-17, VEGF and IFN- $\gamma$



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Existing immunosuppressive agents have a fundamentally different mode of action on T-cells compared to KIO-104

- KIO-104 is best-in-class inhibitor of DHODH (lowest IC<sub>50</sub>)\*
- KIO-104 is first-in-class in ophthalmology

*\*1,000x more potent than Teriflunomide (Aubagio<sup>®</sup> – Sanofi)*

# Non-Infectious Uveitis

Uveitis is a group of eye disorders affecting the uvea and characterized by intraocular inflammation that is often chronic, can flare up at any time, and can lead to visual impairment and vision loss.

## Clinical Symptoms

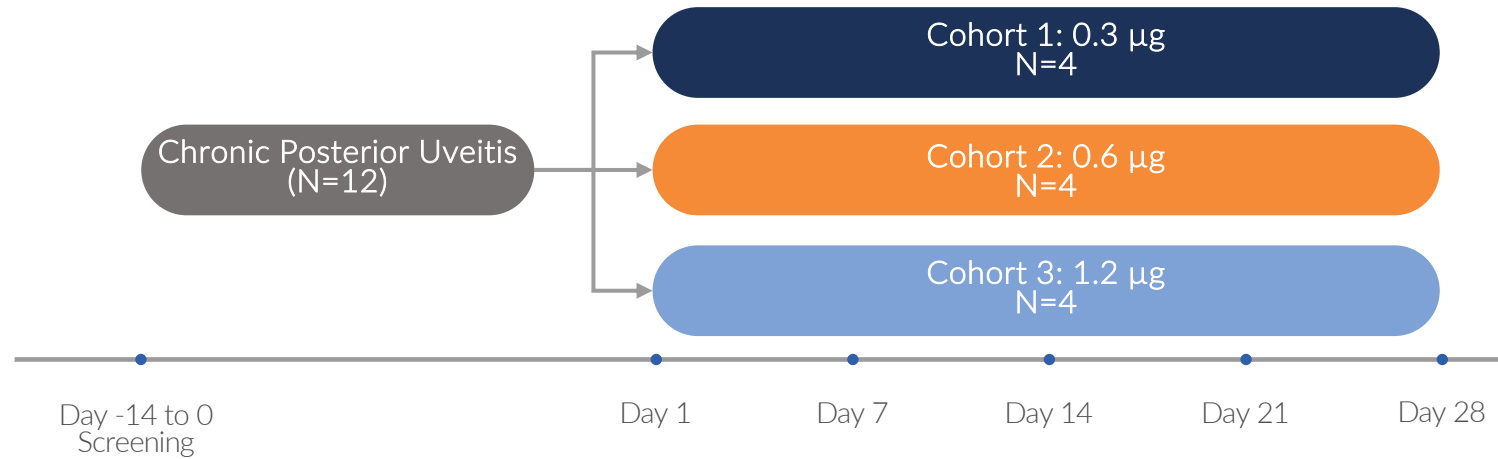
- Redness and pain in the eye
- Sensitivity to light
- Blurred vision
- Dark floating spots in the vision
- Vision loss

## Statistics

- ~**15%** of all cases of legal blindness and visual handicap in the US and EU
- ~**25%** of all cases of blindness globally
- ~**20%** posterior segment manifestation of uveitis
- 6.9%** CAGR 2020-2027
- 20-50 years old** most common age affected in the United States

**Significant unmet need for a steroid sparing approach**

# KIO-104-1101: Phase 1 Study Design



## Study Design

- Prospective, multi-center, open-label, dose ranging, single IVT injection in worse eye

## Endpoints

- Primary – Ocular & systemic safety, pK, safety labs
- Secondary – Visual acuity, visual field, anterior chamber inflammation, vitreous haze

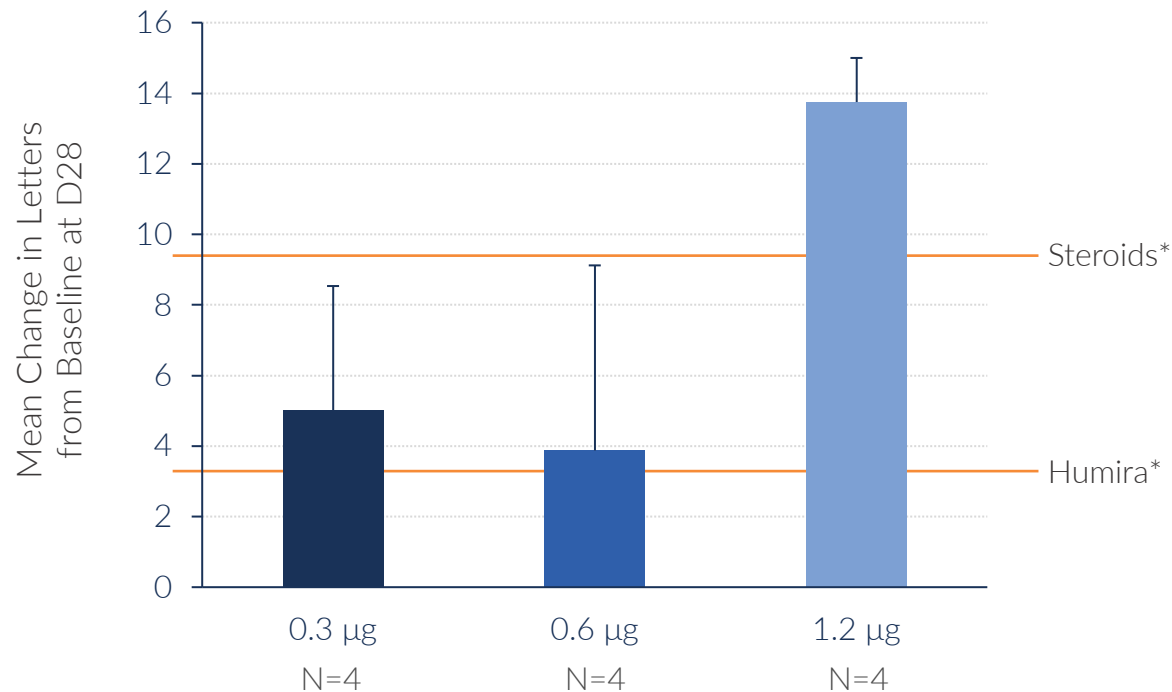
## Key Results

- Visual acuity improved in all patients (in all dose groups)
- Anterior chamber inflammation and vitreous haze decreased
- Evidence of reduced macular edema
- No SAEs, excellent tolerability, no peripheral blood detection (at any timepoint)

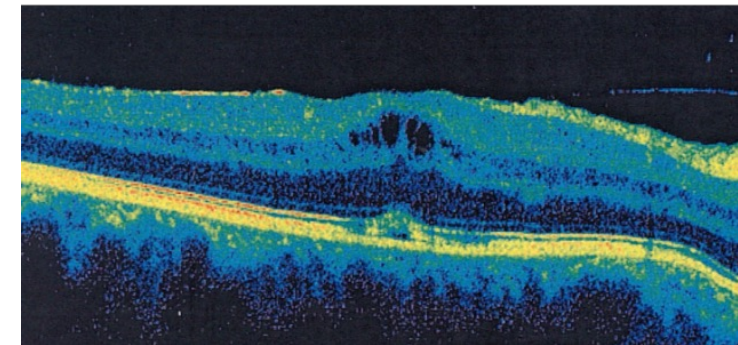
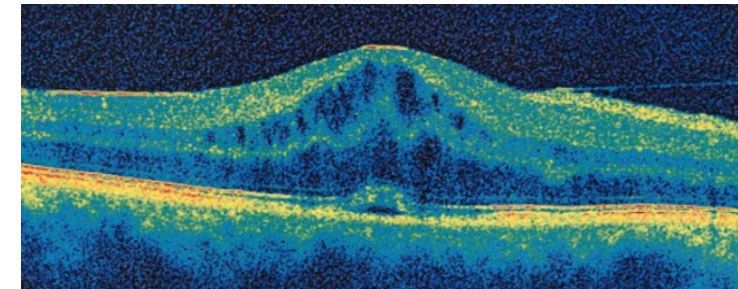


# KIO-104 Improves VA and CME After Single IVT Dose

## Visual Acuity



## Cystoid Macular Edema<sup>‡</sup>



<sup>‡</sup> 40% of eyes with vision threatening cystoid macular edema at baseline had clinically meaningful improvement

\* Historical Controls (Yeh et al, Retina 00 2018: 1-9. Suhler et al, Visual III Ophthalmology 2018:125, 7)  
IVT - Intravitreal

# CORPORATE OVERVIEW

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# Leadership Team



Brian M. Strem, PhD  
President & CEO



Eric J. Daniels, MD, MBA  
Chief Development Officer



Melissa Tosca, CPA  
EVP – Finance



Stefan Sperl, PhD  
EVP – CMC & Operations



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Erin Parsons



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Christine Kay, MD, PhD



Mark Pennesi, MD, PhD



Russel Van Gelder, MD, PhD



Charlie Wykoff, MD, PhD



Retina  
Consultants  
of Texas™



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