

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): **October 29, 2024**

**KIORA PHARMACEUTICALS, INC.**  
(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of incorporation)

**001-36672**  
(Commission File Number)

**98-0443284**  
(IRS Employer Identification No.)

**332 Encinitas Blvd.**  
**Suite 102**  
**Encinitas, CA 92024**

**(858) 224-9600**  
(Registrant's telephone number, including area code)

(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:

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- ☐ 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	KPRX	NASDAQ

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 ☐

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. ☐

**Item 7.01. Regulation FD Disclosure.**

On October 29, 2024, Kiora Pharmaceuticals, Inc. (the “Company”) issued a press release announcing the regulatory approval to initiate ABACUS-2, a Phase 2 clinical trial of KIO-301 for the treatment of retinitis pigmentosa. A copy of the press release is furnished herewith as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated by reference herein.

In addition, the Company hereby furnishes the updated investor presentation attached as Exhibit 99.2 to this Current Report on Form 8-K, which the Company may use in presentations to investors from time to time.

The information furnished pursuant to Item 7.01, including Exhibits 99.1 and 99.2, shall not be deemed “filed” for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”) and will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that the Company specifically incorporates it by reference.

The information furnished in this report, including Exhibits 99.1 and 99.2, shall not be deemed to constitute an admission that such information or exhibit is required to be furnished pursuant to Regulation FD or that such information or exhibit contains material information that is not otherwise publicly available. In addition, the Company does not assume any obligation to update such information or exhibit in the future.

**Item 9.01. Financial Statements and Exhibits.****(d) Exhibits.**

Exhibit Number	Title
<a href="#"><u>99.1</u></a>	<a href="#"><u>Press Release of Kiora Pharmaceuticals, Inc., dated as of October 29, 2024</u></a>
<a href="#"><u>99.2</u></a>	<a href="#"><u>Company Presentation</u></a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

\*Schedules and exhibits have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The registrant hereby undertakes to furnish copies of any of the omitted schedules and exhibits upon request by the U.S. Securities and Exchange Commission.

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## **SIGNATURES**

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

### **KIORA PHARMACEUTICALS, INC.**

By: /s/ Melissa Tosca  
Melissa Tosca  
Chief Financial Officer  
(Principal financial and accounting officer)

Date: October 29, 2024

## **Kiora Pharmaceuticals Receives Investigational New Drug Approval to Initiate ABACUS-2, a Phase 2 Clinical Trial of KIO-301 for the Treatment of Retinitis Pigmentosa**

*Encinitas, California – October 29, 2024* - Kiora Pharmaceuticals, Inc. (NASDAQ: KPRX) ("Kiora" or the "Company") today announced it received regulatory approval to initiate a Phase 2 clinical trial to investigate KIO-301 for vision restoration in patients with retinitis pigmentosa. The ABACUS-2 trial will be a 36 patient, multi-center, double-masked, randomized, controlled, multiple dose study enrolling patients with ultra-low vision or no light perception regardless of their underlying gene mutation associated with retinitis pigmentosa. Dosing of the first patient with KIO-301 is expected to begin next year following validation of novel functional vision endpoints. These functional assessments may serve as approvable primary endpoints in subsequent registration studies in the United States, Europe and other major regions.

"There are unfortunately no approved therapies for patients with retinitis pigmentosa," said Eric Daniels, M.D., Chief Development Officer at Kiora. "This study represents a significant step toward addressing this challenge. Prior to submission for approval, we engaged with European and US regulators to incorporate their expectations and guidance for approvable endpoints. Consistent with historical approval in other inherited retinal disease, both regulatory bodies emphasized the need to measure a therapy's effect on everyday functional vision. For this reason, we are investing time upfront to validate the functional endpoints for ABACUS-2, increasing our likelihood of success in a potential single Phase 3 trial for market approval in the US and Europe. This validation work is being performed, in collaboration with our partner Théa Open Innovation, with the support of the Choroideremia Research Foundation as part of a grant to design a standard endpoint for investigational therapies of inherited retinal diseases."

Kiora has identified and is now contracting with trial sites and clinical investigators at major inherited retinal disease reference centers across Australia. Patients will be randomized 2:1 to receive KIO-301 intravitreally or control. Treatment will be delivered to both eyes. Participants will be randomized to receive either a high dose (100 micrograms) or low dose (50 micrograms) of KIO-301 and after trial participants receive three consecutive doses (6 weeks apart), they will be followed for three months. Following this phase of the study, patients in the control arm may elect to cross-over into the active arm. Primary endpoints will consist of safety and tolerability, and key efficacy assessments include: functional vision; visual acuity as measured by the Berkeley Rudimentary Vision Test; visual fields as measured by perimetry, and a validated ultra-low vision quality-of-life questionnaire. The trial will be conducted across five centers within Australia.

"In a short window since entering our partnership with Kiora, tremendous progress has been made on advancing KIO-301 toward ABACUS-2," said Dr. Céline Olmiere, Head of Théa Open Innovation. "What makes KIO-301 compelling is that it appears, based on the Phase 1b data, to have potential for meaningful vision restoration. Further, because of its unique mechanism of action, it has the potential to work across all 150-plus underlying gene mutations associated with retinitis pigmentosa and other inherited retinal diseases."

KIO-301 is a small molecule that acts as a light-sensitive photoswitch that has the potential to return vision to patients living with reduced sight due to inherited retinal diseases. The novel compound is activated in the presence of light and deactivated in the absence of light. Because hundreds of gene mutations underlie inherited retinal diseases like retinitis pigmentosa, there is an important need for therapies like KIO-301 that have the potential to act in a gene mutation agnostic manner.

KIO-301 targets and enters specialized cells of the retina (retinal ganglion cells or RGCs) that are located 'downstream' of degenerated rods and cones, the cells normally responsible for converting light to vision. KIO-301, when inside RGCs, is activated by visible light and confers light-sensing capabilities by altering the flow of ions in and out of the cell, thus giving RGCs the ability to facilitate visual processing. This is a reversible process allowing turning on and off the RGCs in the presence and absence of light.

About Kiora Pharmaceuticals

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Kiora Pharmaceuticals is a clinical-stage biotechnology company developing and commercializing products for the treatment of retinal diseases. KIO-301 is being developed for the treatment of retinitis pigmentosa, choroideremia, and Stargardt disease. It is a molecular photoswitch that has the potential to restore vision in patients with inherited and/or age-related retinal degeneration. KIO-104 is being developed for the treatment of retinal inflammation. It is a next-generation, non-steroidal, immuno-modulatory, and small-molecule inhibitor of dihydroorotate dehydrogenase.

In addition to news releases and SEC filings, we expect to post information on our website ( [www.kiorapharma.com](http://www.kiorapharma.com)) and social media accounts that could be relevant to investors. We encourage investors to follow us on Twitter and LinkedIn as well as to visit our website and/or subscribe to email alerts.

#### **About Théa Open Innovation**

Théa Open Innovation (TOI) is a sister company of Théa, the leading independent European pharmaceutical company specialized in the research, development, and commercialization of eye care products. TOI's mission is to establish partnerships with startups, biotech companies, and universities to help bring the most cutting-edge eye care products to the market. For more information, visit [www.theaopeninnovation.com](http://www.theaopeninnovation.com)

#### **Forward-Looking Statements**

Some of the statements in this press release 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, Kiora's ability to execute on development and commercialization efforts and other regulatory or marketing approval efforts pertaining to Kiora's development-stage products, including KIO-104 and KIO-301, 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 sufficiency of existing cash on hand to fund operations for specific periods, the projected cash runway, and Kiora's plans to further fund development of KIO-104. These statements involve risks and uncertainties that may cause results to differ materially from the statements set forth in this press release, including, among other things, the ability to satisfy the closing conditions related to the offering, the ability to conduct clinical trials on a timely basis, 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 25, 2024 or described in Kiora's other public filings including on Form 10-Q filed with the SEC on August 9, 2024. Kiora's results may also be affected by factors of which Kiora is not currently aware. The forward-looking statements in this press release speak only as of the date of this press release. 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.

Contacts:  
Investors [investors@kiorapharma.com](mailto:investors@kiorapharma.com)



# Kiora Pharmaceuticals, Inc.

NASDAQ: KPRX

————— Q4 2024 | Corporate Overview



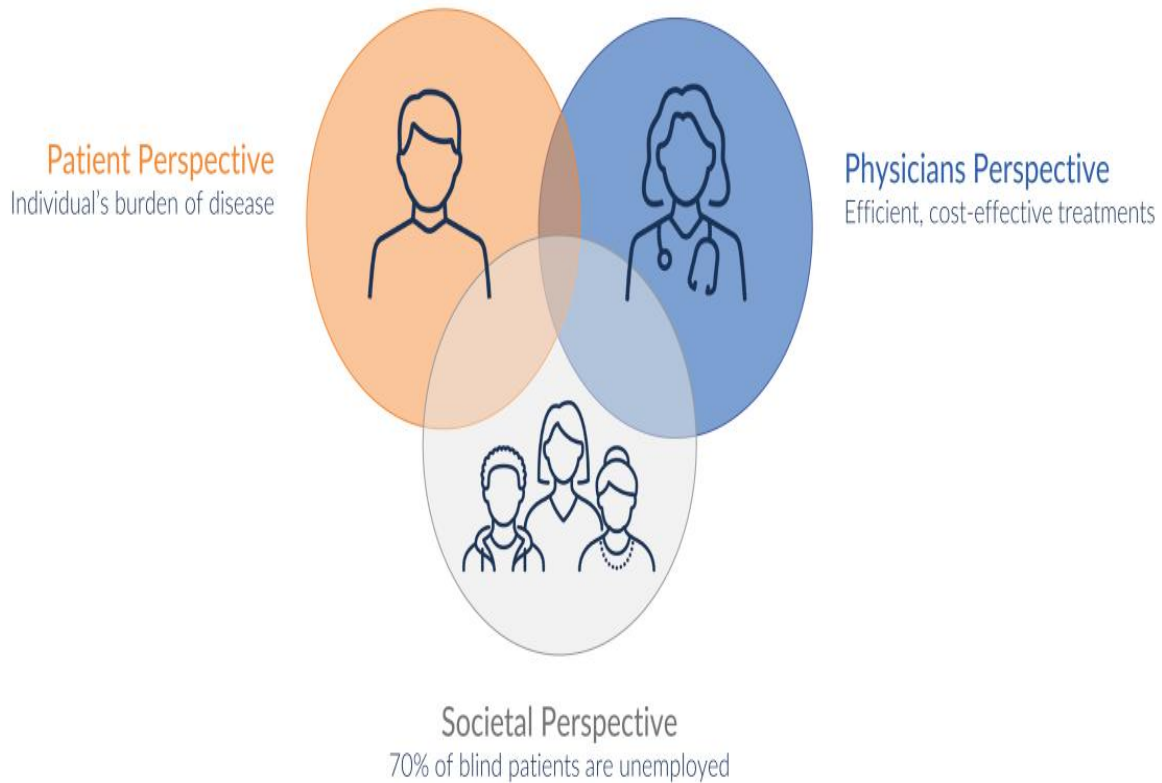
## Forward Looking Statements

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 maintain the listing of our common stock on a national securities exchange, 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 25, 2024, 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.



# Developing Treatments for Retinal Diseases

Improve Sight in Patients with Severe Vision Loss and High Unmet Needs





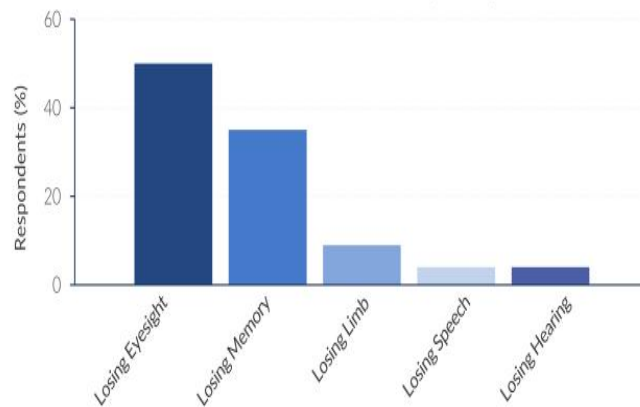
## Why Retinal Diseases?

"...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."

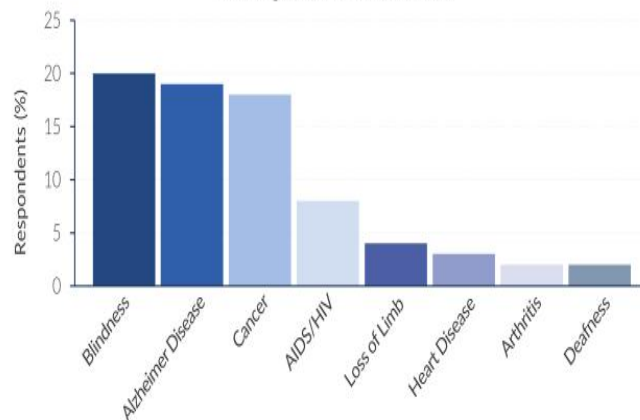
- John M. Hull, *Touching the Rock*

JAMA Ophthalmol. 2016;134(10)

Conditions with Greatest Effect on Day-to-Day Life



Rankings of Worst Conditions



# Investment Highlights

Innovative Modalities	KIO-301	Molecular photoswitch
	KIO-104	Anti-inflammatory DHODH inhibitor
Significant Market Need	KIO-301	100K patients in US with RP and other IRDs
	KIO-104	<ul style="list-style-type: none"> <li>&gt;50M patients in US with pseudophakia</li> <li>17M patients in US with retinal vein occlusion</li> <li>800k patients in US with diabetic macular edema</li> <li>400k patients in US with non-infectious uveitis</li> </ul>
Commercial Rights & Partners	KIO-301	<ul style="list-style-type: none"> <li>Théa reimburses and/or funds development through Phase 3</li> <li>\$285 MM in development, regulatory, and commercial milestones</li> <li>Tiered royalties can exceed 20%</li> <li>Pursuing partnership for Asia rights</li> </ul>
	KIO-104	Kiora controls worldwide rights
Balance Sheet (30 June 2024)	<ul style="list-style-type: none"> <li>KIO-301 R&amp;D reimbursed quarterly</li> <li>\$27.8+ MM in cash*; Funds operations into 2027</li> </ul>	

\* - Cash, cash equivalents and short-term investments



# Targeting the Retina to Slow, Stop, or Restore Vision Loss

## Development Pipeline of Proprietary Small Molecule Therapeutics

Product	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Commercial Rights
KIO-301 Intravitreal	Retinitis Pigmentosa (Mutation Agnostic)*	<div></div>	<div></div>	<div></div>		Théa Open Innovation (global less Asia)
	Choroideremia	<div></div>	<div></div>	<div></div>		
	Stargardt Disease	<div></div>	<div></div>	<div></div>		
KIO-104 Intravitreal	Retinal Inflammation	<div></div>	<div></div>	<div></div>		Kiora Pharmaceuticals
	Proliferative Vitreoretinopathy	<div></div>				

\* - Orphan Disease Designation granted in the USA and EU



# KIO-104

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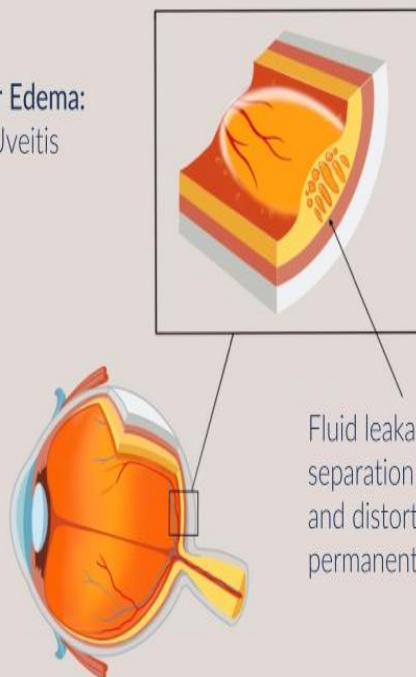
Intravitreal Small Molecule DHODH Inhibitor

Steroid-Sparing Approach to Retinal Inflammation

## Retinal Inflammation Underlies Macular Edema

### Common Causes of Macular Edema:

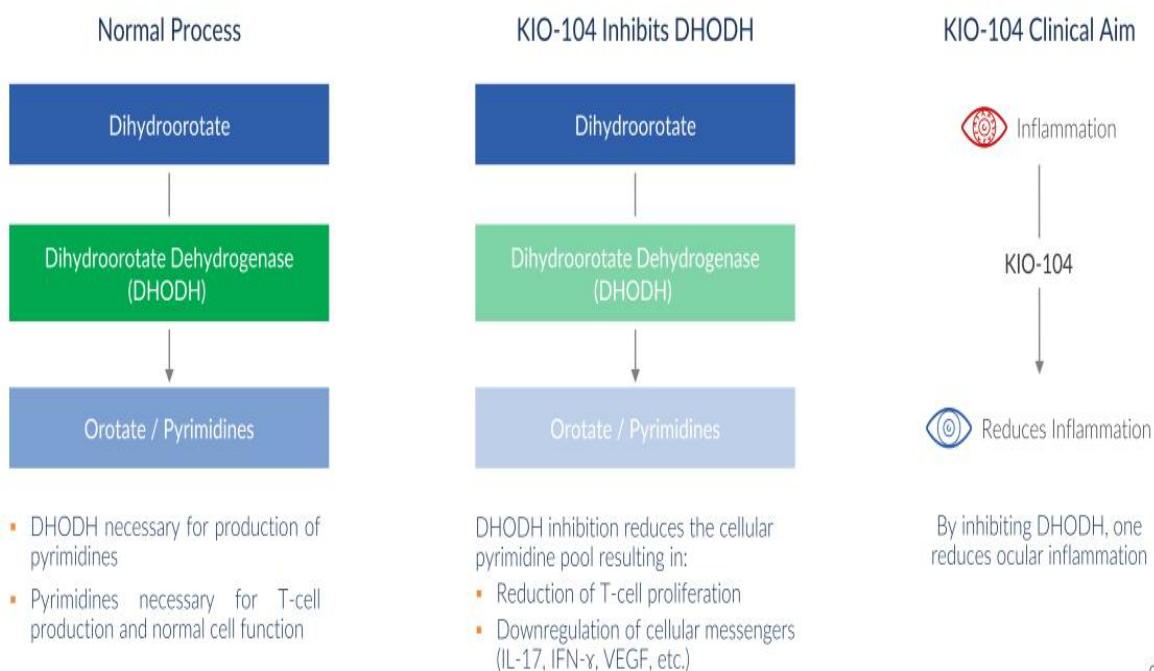
- Posterior Non-Infectious Uveitis
- Diabetic Macular Edema
- Retinal Vein Occlusion
- Post Cataract Surgery



Fluid leakage into the retina can cause separation of cell layers leading to blurring and distortion of vision and potentially permanent vision loss

## KIO-104 Mechanism of Action

- T-cells are known to play a fundamental role in inducing ocular inflammation
- Dihydroorotate Dehydrogenase (DHODH) inhibitors are well established, disease-modifying agents in autoimmune conditions

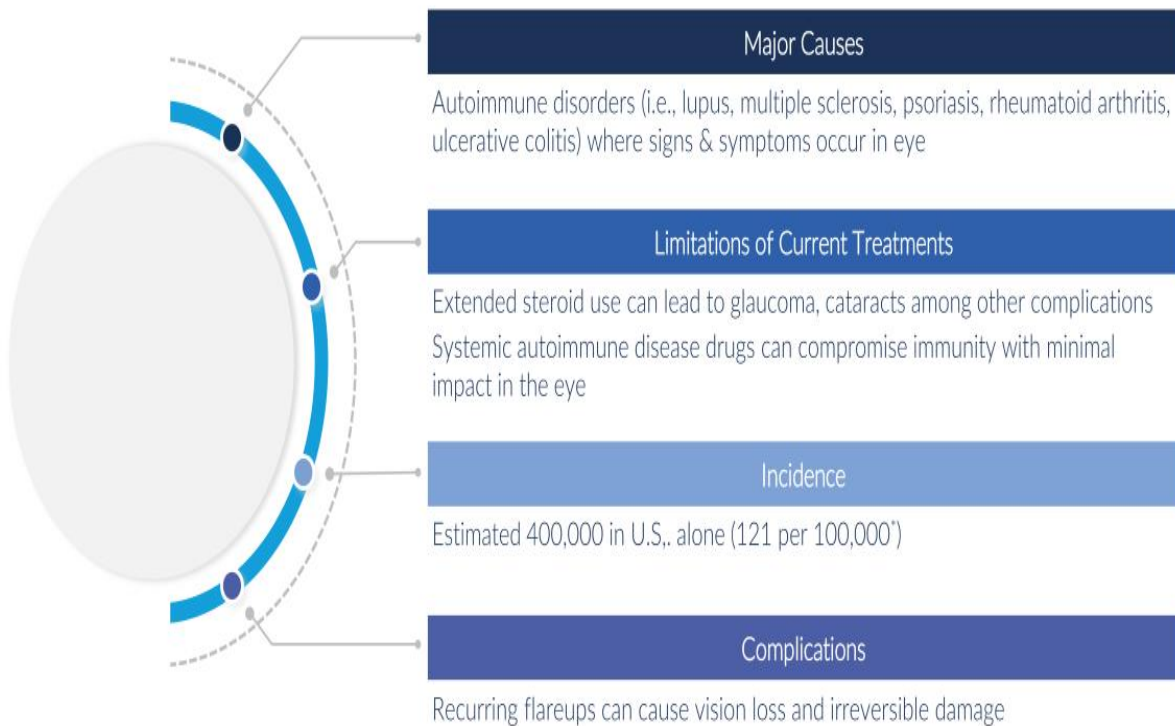




# Posterior Non-Infectious Uveitis: a T-Cell Driven Disease

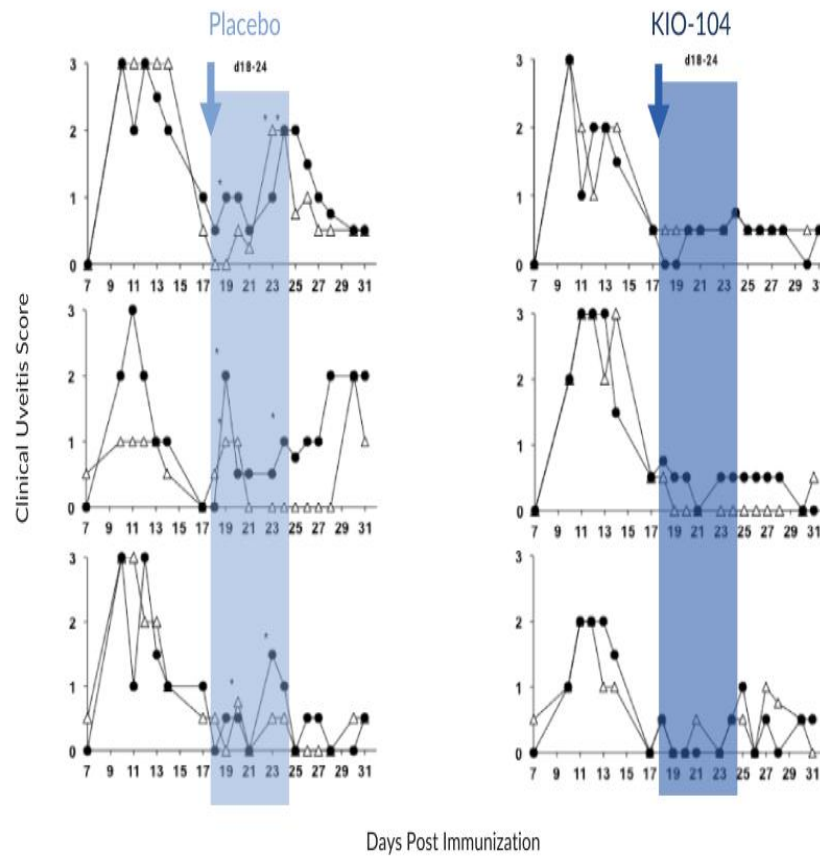
## Retinal Inflammation in the Back of the Eye Can Lead to Vision Loss

Need for steroid-sparing anti-inflammatory delivered locally to the eye



\* JAMA Ophthalmol. 2016;134(11):1237-1245

## KIO-104 in Uveitis – Preclinical



KIO-104 reduced the number of relapses



## Phase 1/2a Study Design

Duration [Study Days]	-14	-7	0	2	7	14	21	28
Tasks	Screening / Baseline	KIO-104 Injection	Exam	Exam	Exam	Exam	Exam	Exam

### Study Design

- Patients with chronic, posterior non-infectious uveitis
- Prospective, open label, multi-center, dose escalating, 4 patients in each Cohort, 12 patients in total

### Objectives

- Safety and tolerability
- Improvement of inflammation
- Blood PK of KIO-100

### PaniJect Administration

- Single intravitreal injection of 300ng, 600ng, and 1200ng (any systemic therapy at enrollment was continued)

## — KIO-104 Phase 1/2a Results

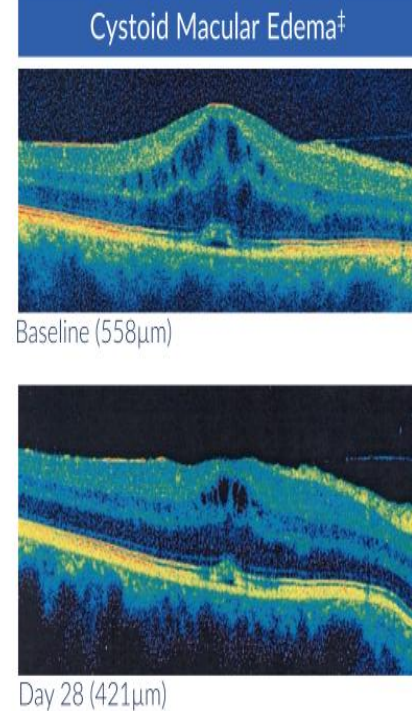
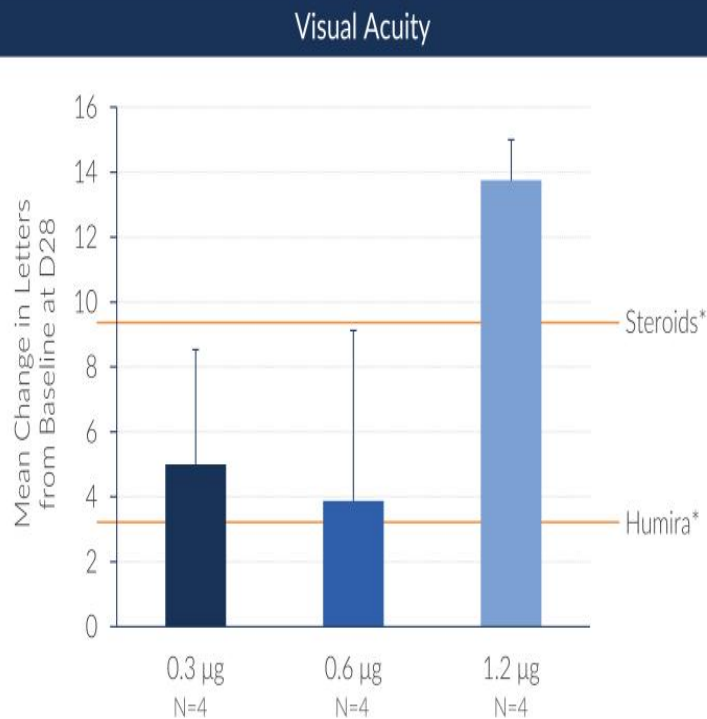
### Safety & Tolerability

- ✓ No SAE
- ✓ Excellent tolerability at all doses

### Pharmacokinetics

- ✓ KIO-100 was not detected in the peripheral blood at any time

# Phase 1/2a Results: KIO-104 Improved Visual Acuity and Reduced Cystoid Macular Edema

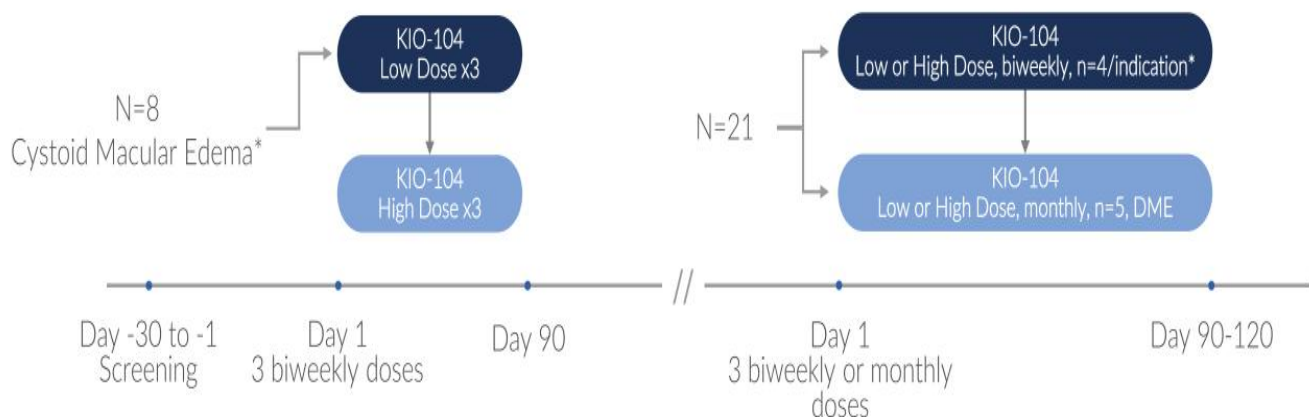


<sup>‡</sup> 40% of eyes with vision threatening cystoid macular edema at baseline had clinically meaningful improvement

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

# KIO-104: Phase 2 Study Design (KLARITY)

2-Step, Randomized, Open-Label, Steroid-Sparing, Dose Expansion Trial



## Study Design

- Step 1: Dose informing short-term study in macular edema (multiple clinical indications)
- Step 2: Assuming positive data from Step 1, dose expansion study with highest tolerated dose

## Endpoints

- Primary: AEs, PK, labs
- Secondary: BCVA, CST, PROs

\* Indications include Diabetic Macular Edema (DME), Posterior Non-Infectious Uveitis, others



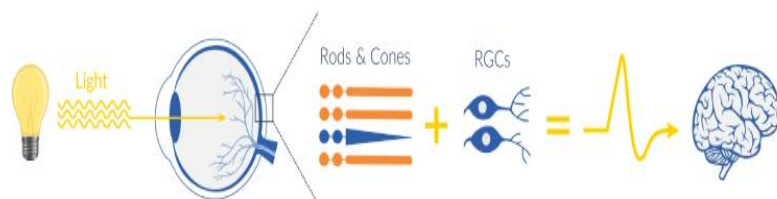
# KIO-301

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Small Molecule Targeting Vision Restoration

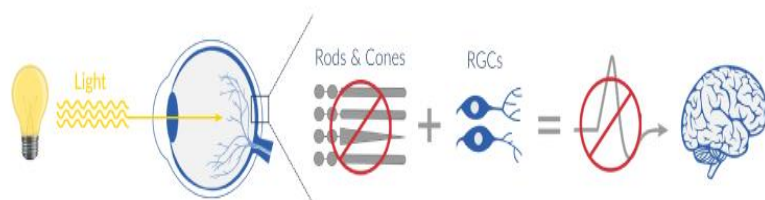
Inherited Retinal Diseases

# Inherited Retinal Diseases (IRDs) Lead to Loss of Vision



## Healthy Vision

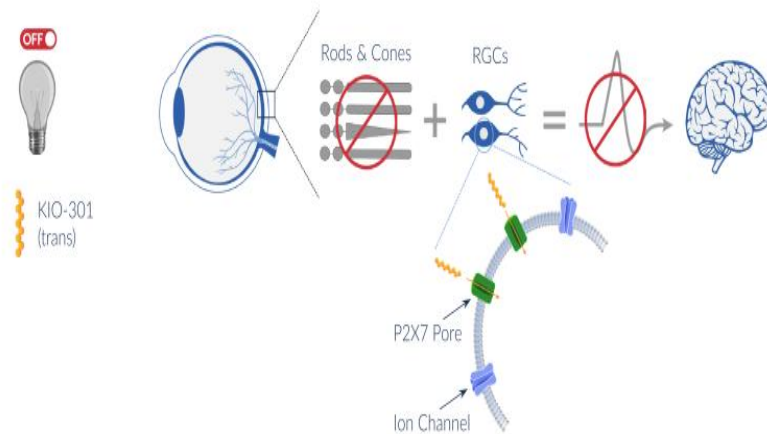
- Rods and cones, the photoreceptors of the retina, process light and relay an electrical signal to downstream cells
- One of these cell types, retinal ganglion cells (RGCs), transmit the signal to the visual cortex
- The visual cortex is the part of the brain where vision is perceived



## Damage from IRDs

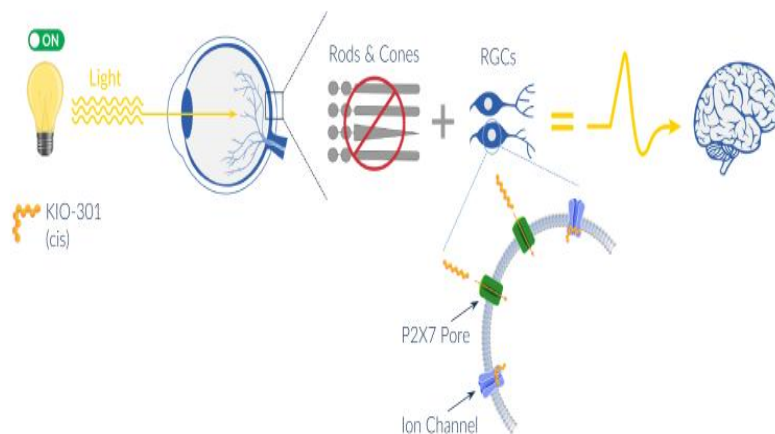
- IRDs, including Retinitis Pigmentosa (RP), result in progressive degeneration and loss of function of rods and cones
- This causes continuous impairment of vision that often leads to blindness
- Importantly, many IRDs do not affect the RGCs

# 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



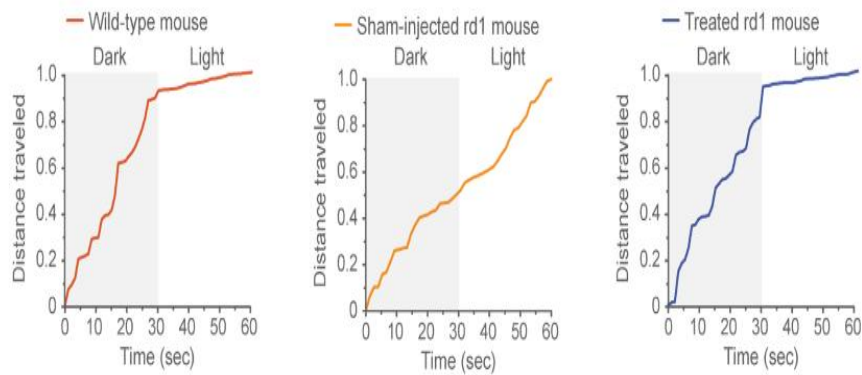
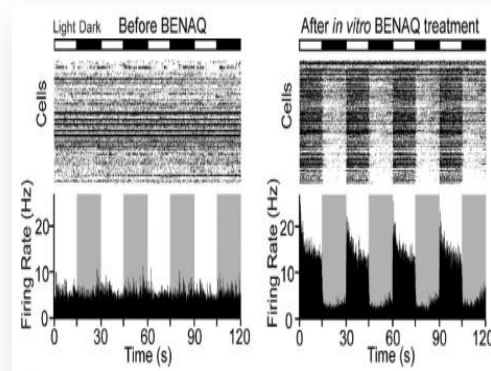
## KIO-301 with Light

- 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



# KIO-301 Reanimates the Retina & Changes Behavior

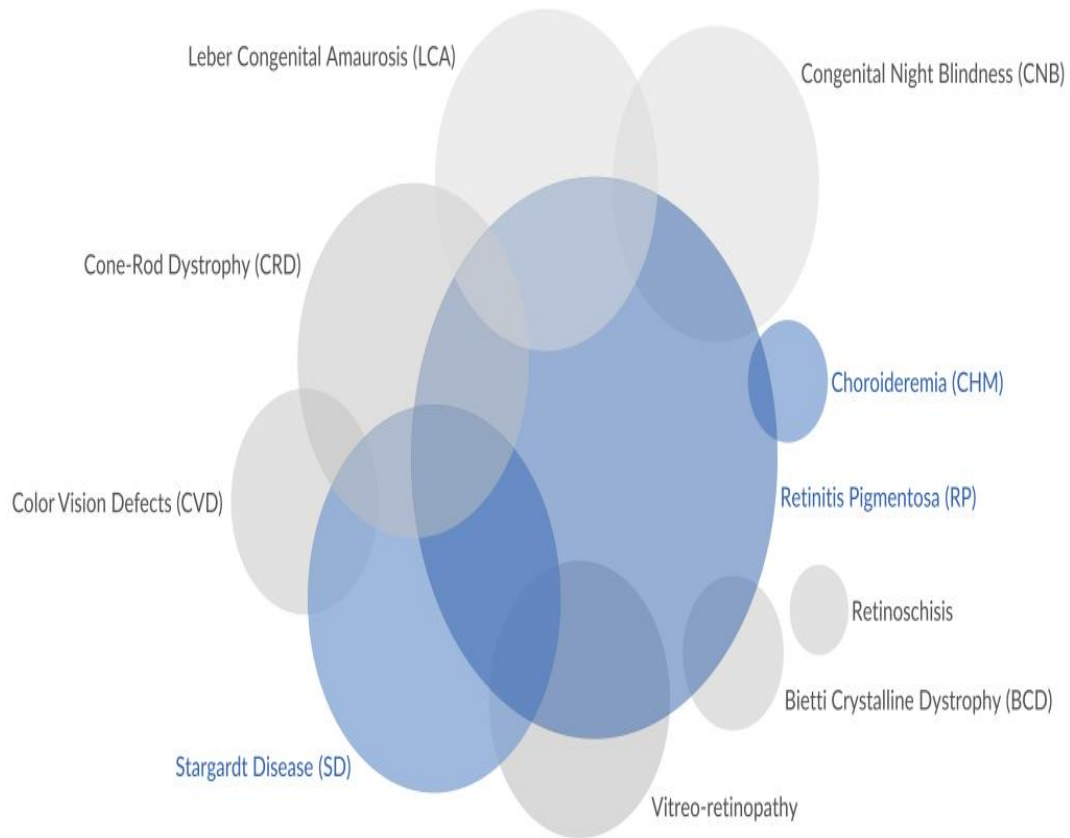
## Extensive Validation in Preclinical Models



Neuron 2014; 81, 800-813. Behavioral study used a homologue molecule to KIO-301 API



## Inherited Retinal Disease Landscape



# Retinitis Pigmentosa

## A Disease with No Available Treatments

Normal Vision



Vision Declines over Time



### Market Opportunity

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

### Clinical Presentation

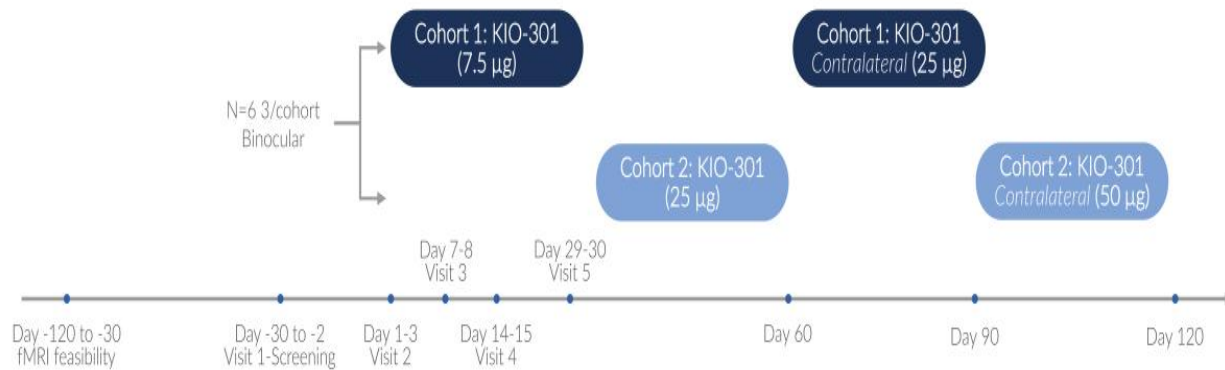
- Night blindness, reduced visual field range and eventual loss of central vision
- Visual acuity declines
- 50% of patients are not qualified to drive by age 37 and legally blind by 55

### Etiology

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

# KIO-301: 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

# ABACUS-1 – Primary Endpoint Achieved

Single Dose IVT KIO-301 is Safe & Well Tolerated @ 7.5µg, 25µg, 50µg

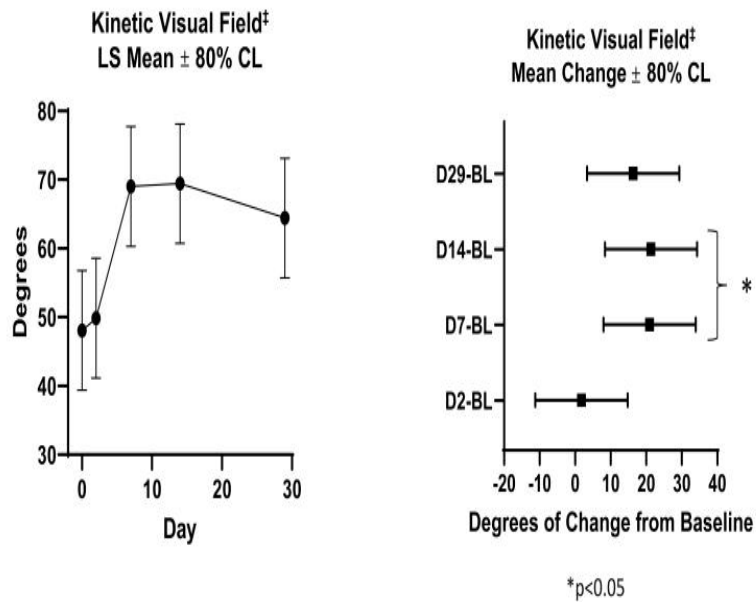
MedDRA Term	KIO-301 7.5µg (N=3); n (%)	KIO-301 25µg (N=6); n (%)	KIO-301 50µg (N=3); n (%)	Severity	Drug Related	Total N=12; n (%)
Ocular Hypertension	1 (33%)	0 (0%)	0 (0%)	Mild	Possible	1 (8.3%)
Eye Swelling	0 (0%)	1 (17%)	0 (0%)	Mild	Unlikely	1 (8.3%)
Eye Pain	0 (0%)	2 (33%)	0 (0%)	Mild	Unlikely	2 (17%)
Anterior Chamber Cell	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Anterior Chamber Flare	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Vitreous Cells	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Retinitis	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Vasculitis	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Iritis	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Keratic Precipitates	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Photophobia	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Photopsia	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Vitreous Floaters	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Punctate Keratitis	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)
Conjunctival Hyperemia	0 (0%)	0 (0%)	0 (0%)	N/A	N/A	0 (0%)

Ocular AEs only. N = number of patients reporting event, n (%) = % of patients reporting event



# Kinetic Visual Field

## KIO-301 May Improve Visual Field



## 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 2

<sup>†</sup> Cohort 2 includes 3 patients (6 eyes)  
CL = Confidence Limit



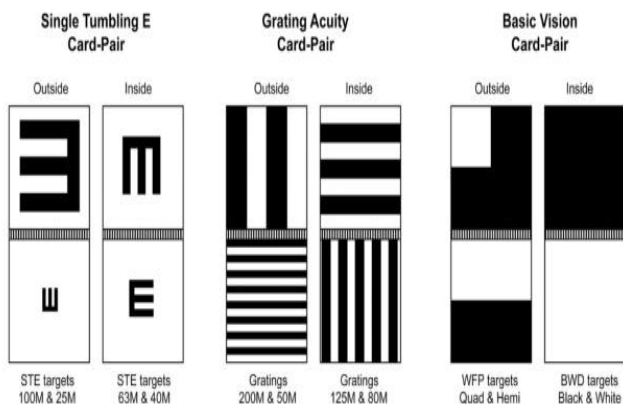
# Visual Acuity – Berkeley Rudimentary Vision Test (BRVT)

1046-5488/12/8909-1257/0 VOL. 89, NO. 9, PP. 1257-1264  
OPTOMETRY AND VISION SCIENCE  
Copyright © 2012 American Academy of Optometry

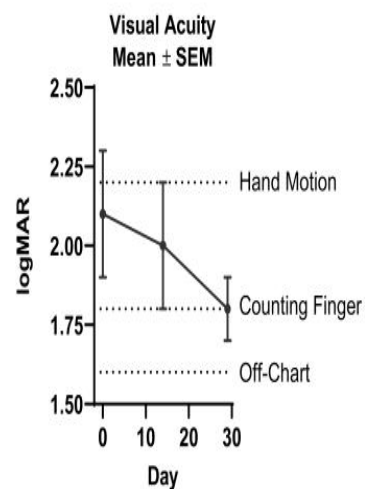
## ORIGINAL ARTICLE

### The Berkeley Rudimentary Vision Test

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



The BRVT has three pairs of hinged cards that are 25 cm square.



Cohort 2 (3 patients, 3 eyes)

## Light Perception (Intensity & Contrast Assessment)



Aim: Evaluate Light Perception at a Basic Level

Assessment:

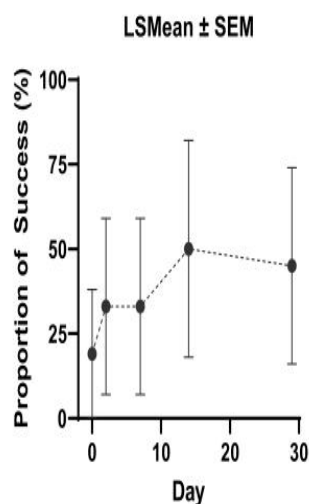
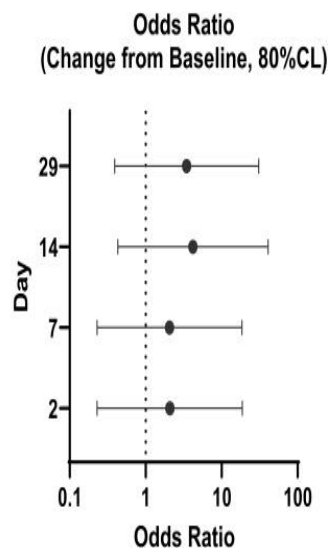
- Series of visual stimuli (a series of letters are presented on a screen to the patient via a rear projector)
- Binary outcome (yes/no)
- The subject is asked to acknowledge (verbally and/or physically) when a change in light is perceived
- Asked to also identify object, if possible





# Light Perception – Cohort 1

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



## Light Perception

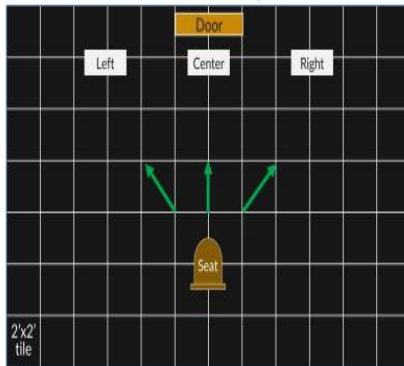
Insights:

- Cohort 1 subjects demonstrate improved odds ratio on drug
- Odds Ratio – strength of association,  $OR=2 \rightarrow 100\%$  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

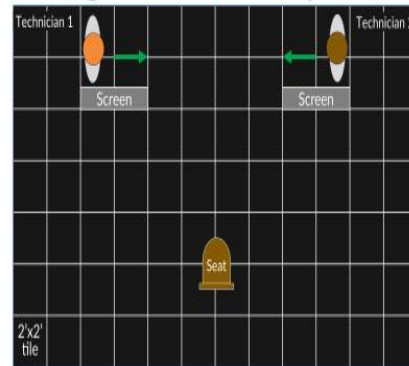


## Functional Vision - Multiluminance Orientation & Mobility (MLOM)

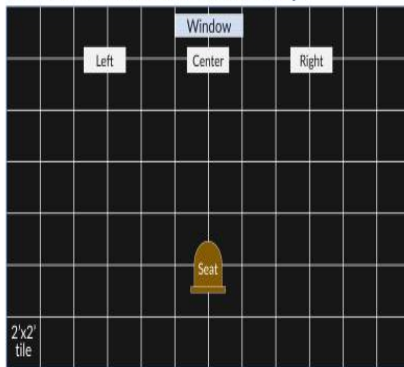
Door Location Test Setup



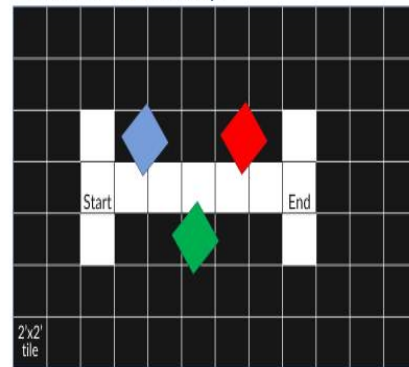
Walking Direction Test Setup



Window Location Test Setup

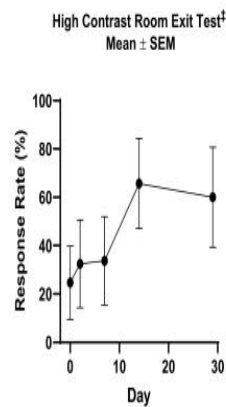
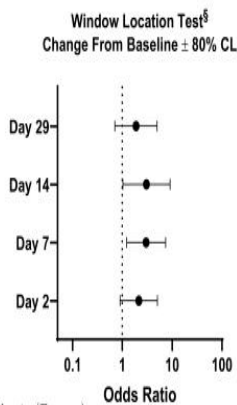
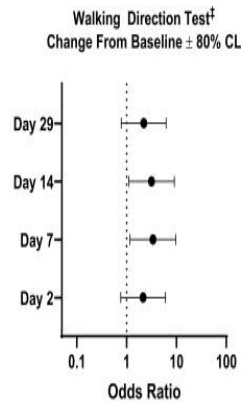
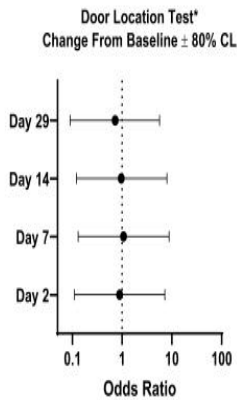


HCRE Course Setup



# Functional Vision – Multiluminance Orientation & Mobility (MLOM)

## KIO-301 May Improve Functional Vision



## 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)

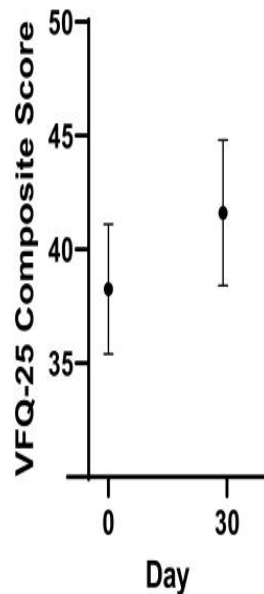
Ora-MLOM™ Suite of Tests



# Visual Function Questionnaire (NEI VFQ-25)

KIO-301 May Improve Patients' Overall Quality of Life

## Quality of Life Survey



## Quality of Life

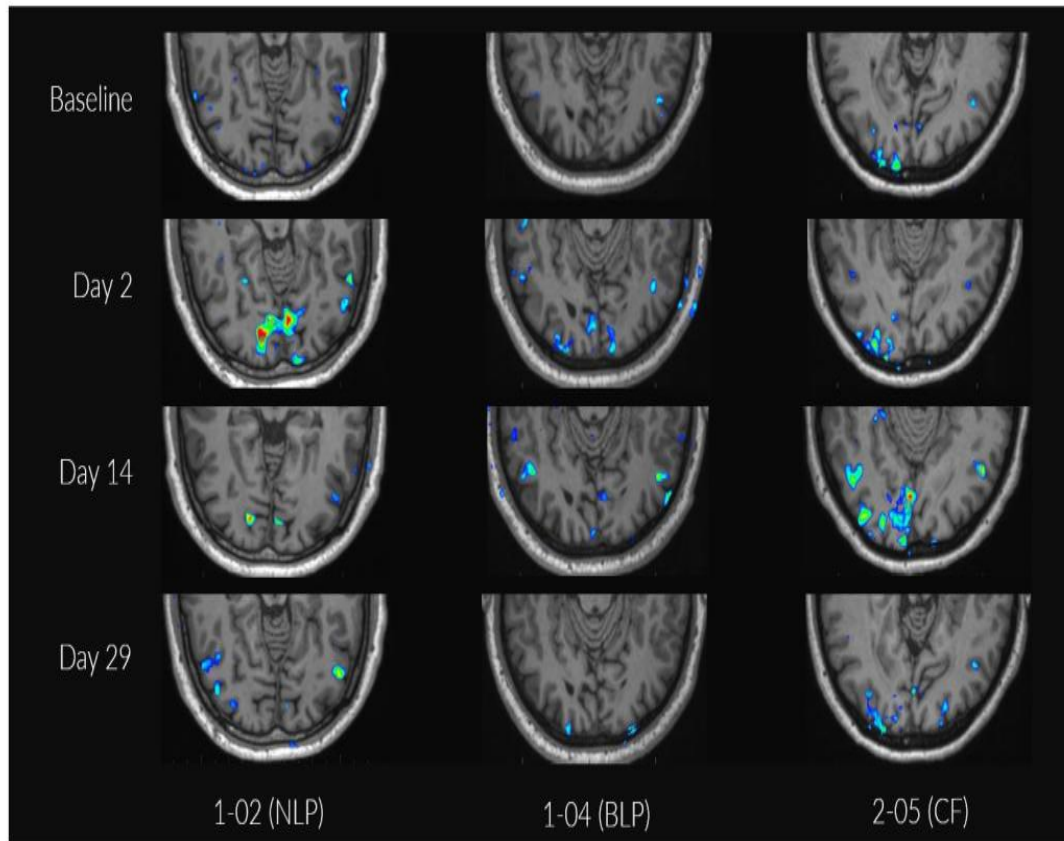
National Eye Institute generated survey assess daily functions related to general health & vision, ocular pain, near & distance activities, social functioning, mental health, dependency, driving, color vision, and peripheral vision.

2-4 point increase is considered clinically meaningful\*



# Functional MRI

Qualitative Supportive of Cortical Activation

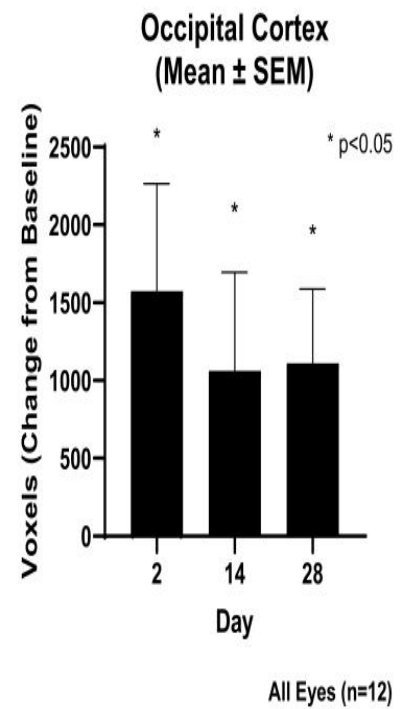
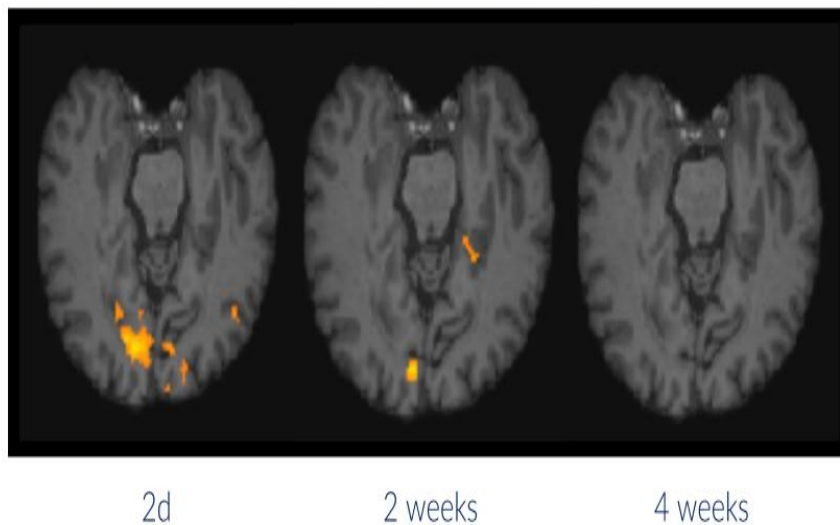


NLP - No Light Perception, BLP - Bare Light Perception, CF - Counting Fingers

# Functional MRI

## Quantification of Checkerboard Stimulus

Statistical Activation Map (Subject 2-05 [CF] - 50 $\mu$ g)



## — Patient Testimonials

"Before the study, I had no light perception whatsoever. And at the start of the study, I had a couple of MRIs; and while I was there, they flashed lights there and I saw absolutely nothing. 48 hours after the drug, I had another MRI; and **I think that was the first OMG moment, to say, that I can actually see flashing lights.**"

Subject 1-02 ~ Baseline NLP

"...in my last few months and years, my vision has been deteriorating fairly rapidly. It's like a dimmer switch has been turned down and the rooms were getting darker. Going into areas would be like going into a cave. In my living room, there are two lights in the ceiling, cluster lights. I would have to look to find one, then look to find the other.

**When I sat down three days after the injection, I looked up and I could see both quite clearly and with better illumination (brightness)..."**

~ Subject 1-03 ~ Baseline HM

"We went for a walk, a couple of weeks ago, into the supermarket, and I thought I was in a sci-fi movie. **I could see everything, and I could see every individual item which I would not have been able to do..."**

Subject 2-05 ~ Baseline CF

## ABACUS-1 Takeaways

### No Safety & Tolerability Concerns

1

KIO-301 appears to reanimate the retina

- Concordance of improvement in visual acuity, visual field & functional vision
- Increased visual cortex activity

2

Approvable outcome assessments discussed with regulators

- Positive US FDA pIND meeting in 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



# Théa Open Innovation Partnership: Co-Development & Commercialization



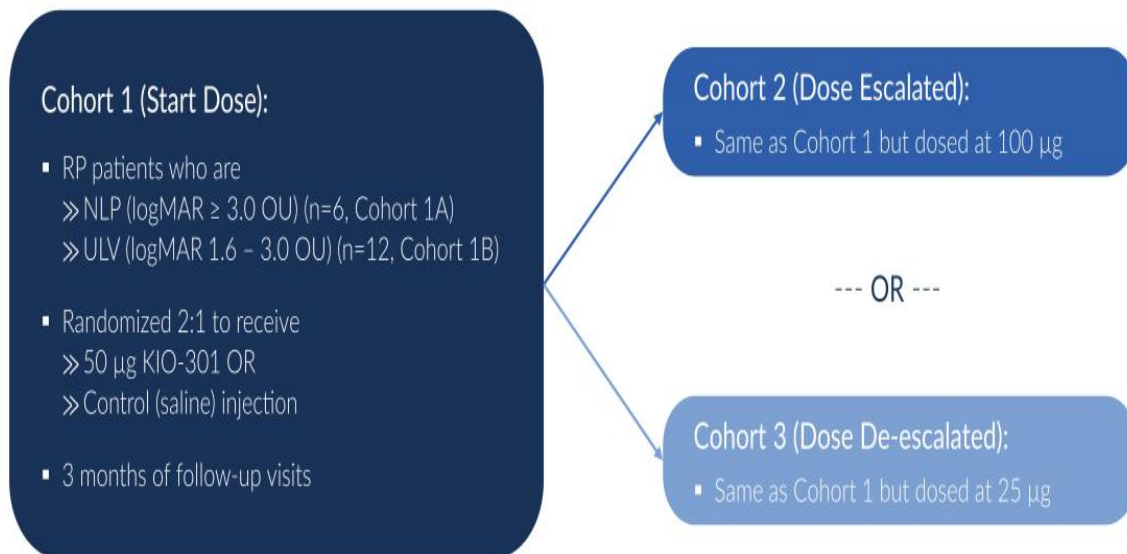
## Transaction Highlights

- Théa Open Innovation (TOI) granted exclusive development & commercialization rights to KIO-301 for IRDs (≠ Asia)
- \$16M cash upfront payment to Kiora
- Kiora eligible for up to an additional \$285M in clinical development, regulatory and commercial milestones
- Kiora to conduct and be reimbursed for Phase 2 trials
- TOI to conduct and fund Phase 3 trials
- Kiora to receive tiered royalties up to low 20%



## KIO-301-2101: Phase 2 ABACUS-2 | Received Approval to Initiate

Double-masked, Randomized, Controlled, Multiple Dose Study



Recruitment in each Cohort begins with 6 patients (1:1) for SRC review and escalation/de-escalation recommendation

# ABACUS-2 (IVT Q6W for 3 Months)

Double-masked, Randomized, Controlled, Multiple Dose Study – 5 Sites (Australia), 36 Patients

## Study Visits

- **Treatment Period**  
Study visits occur every 3 weeks for study assessments with KIO-301 or control administered every 6 weeks (OU) for 3 consecutive doses
- **Follow-up Period**  
Four visits 3 weeks apart with the final, end-of-study visit occurring 3 months after the last dose of KIO-301 or control

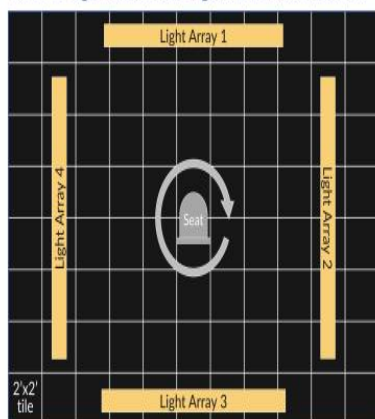
## Study Assessments

- **Safety Assessments**  
Adverse events, vital signs, ECG, clinical chemistry & hematology, pharmacokinetics, SD-OCT, dilated funduscopy, slit lamp, intraocular pressures
- **Efficacy Assessments**
  - » Visual acuity as measured by BRVT
  - » Visual field as measured by digitized Goldmann perimetry
  - » Ultra-low Vision Quality of Life Questionnaire
  - » Functional vision as measured by a validated
    - 360-degree door location exit test (patients with NLP)
    - 360-degree room light direction test (patients with NLP)
    - 360-degree object identification test (patients with ULV)
    - High contrast room exit test (patients with ULV)

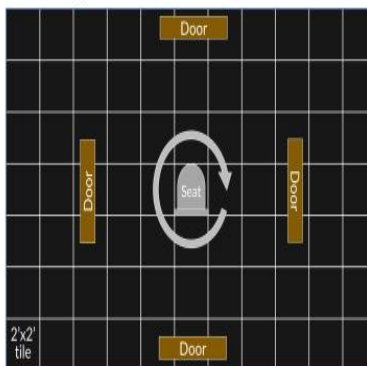
# Choroideremia Research Foundation Grant & Ora Partnership

Run-in Study Ongoing to Validate Novel Functional Vision Endpoints

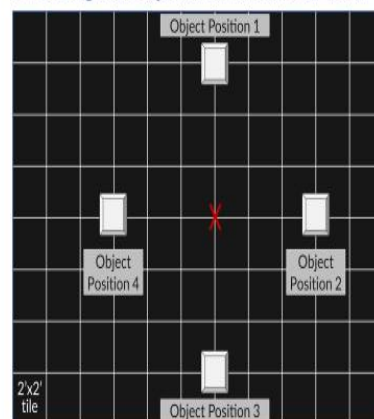
360-Degree Room Light Direction Test



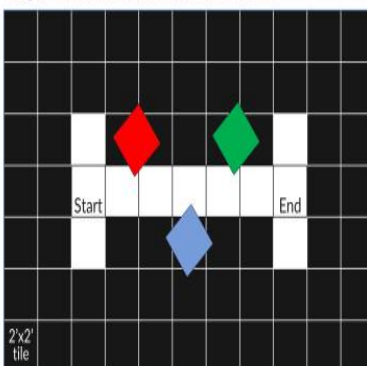
360-Degree Door Location Test



360-Degree Object Identification Test



High Contrast Room Exit Test



Ora

Choroideremia  
RESEARCH FOUNDATION

## Leadership Team



Brian M. Strem, PhD  
President & CEO



Eric J. Daniels, MD, MBA  
Chief Development Officer



Melissa Tosca, CPA  
Chief Financial Officer



Stefan Sperl, PhD  
EVP – CMC & Operations





## Upcoming Milestones

### KIO-301

- Initiate ABACUS-2 Phase 2 clinical trial 2H 2024
- Complete run-in study to validate novel functional vision endpoints Q1 2025
- Complete enrollment of ABACUS-2 2H 2025

### KIO-104

- Round out non-clinical package Q4 2024
- Initiate KLARITY Phase 2 clinical trial 1H 2025



# Thank You

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NASDAQ: KPRX

