



# Kiora Pharmaceuticals, Inc.

NASDAQ: KPRX

————— Q2 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 products, including KIO-101, KIO-201 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. 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, 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.



## Corporate Highlights

Developing Therapeutics for Rare & Underserved Ophthalmic Diseases

### Priority Asset – KIO-301: Vision Restoration in Retinitis Pigmentosa (RP)

- Small molecule “photoswitch” is gene mutation agnostic, easy to deliver
- Study fully enrolled, dosing ongoing
- Case study presented in Q2 2023, anticipate full results in Q4 2023

### KIO-101: Ocular Surface Disease in Rheumatoid Arthritis & Other Autoimmune Diseases (OPRA+)

- Small molecule inhibitor of a validated, disease modifying target
- First patient, first visit in Q2 2023
- Anticipate full results in Q3 2024

### KIO-201: A Novel, Modified Hyaluronic Acid (HA) Molecule for Ocular Wound Healing

- Successful Phase 2 Persistent Corneal Epithelial Defects (PCED) trial
- Results reported in Q2 2023
- Initiate discussions with FDA for registration trial in 2H 2023

Efficient operating structure with low monthly burn (~\$850K)

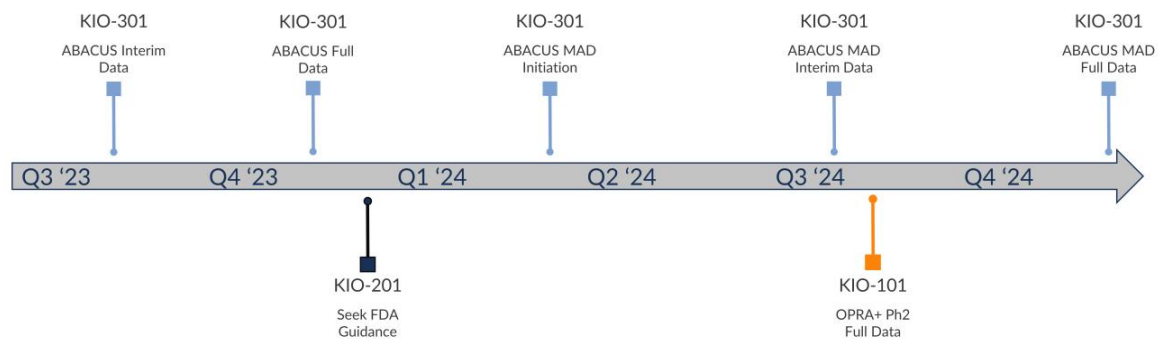


## Diverse Pipeline Offers Near Term Milestones

|                   | Indication                                    | Product Formulation | Development Stage                            |         |         |         |
|-------------------|-----------------------------------------------|---------------------|----------------------------------------------|---------|---------|---------|
|                   |                                               |                     | Pre-clinical                                 | Phase 1 | Phase 2 | Phase 3 |
| Posterior Segment | Retinitis Pigmentosa (Mutation Agnostic)      | KIO-301 IVT*        | Granted Orphan Drug Designation – March 2022 |         |         |         |
| Anterior Segment  | Ocular Presentation of Rheumatoid Arthritis + | KIO-101 Eye Drop    |                                              |         |         |         |
|                   | Persistent Corneal Epithelial Defects         | KIO-201 Eye Drop    |                                              |         |         |         |

\* IVT – Intravitreal Injection

## Upcoming Milestones





# KIO-301

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Vision Restoration in Retinitis Pigmentosa

Normal Vision



Vision Declines over Time



# Initially Targeting RP

## A Disease with No Available Treatments

### 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

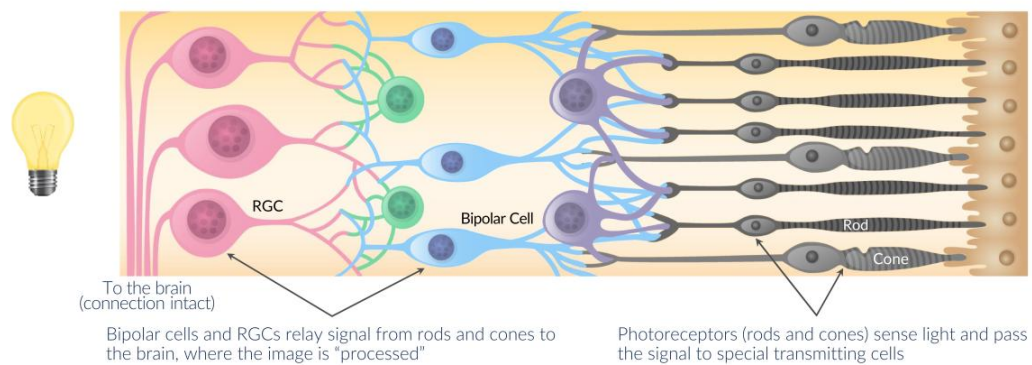
- 50+ genetically distinct subtypes from 150+ mutations
- Inherited disease

### Market Opportunity

- ~100k patients in US (Provider: Retina Specialists [~3k])
- Estimated Total Cost to US Healthcare System in 2019: \$3.7B

IOVS: Visual Field Progression in Retinitis Pigmentosa, American Academy of Ophthalmology, Clinical Ophthalmology 2021;15:2855–2866

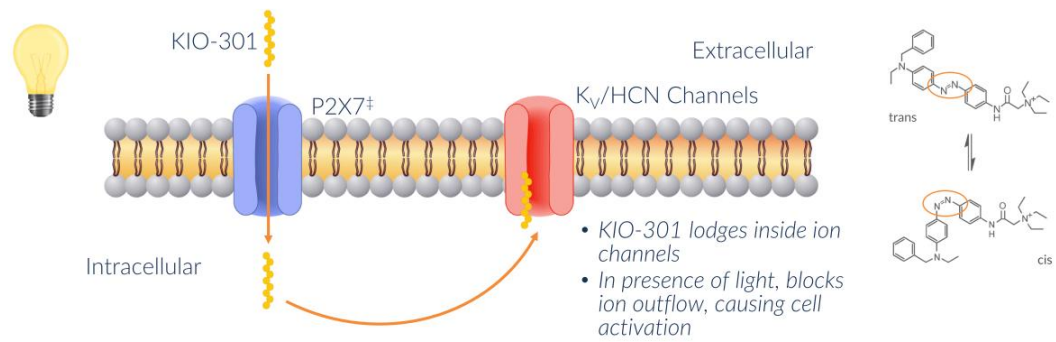
## Downstream Neurons Remain Viable in RP



- RP results in death of photoreceptors
- Bipolar cells and Retinal Ganglion Cells (RGCs) remain intact and retain ability to send signals to the brain

## KIO-301 (MOA): Turns RGCs "ON" in the Presence of Light

- In RP, photoreceptors die → downstream neurons (RGCs) are not capable of being activated
- KIO-301 preferentially enters these RGCs and turns them "ON" in the presence of light\*



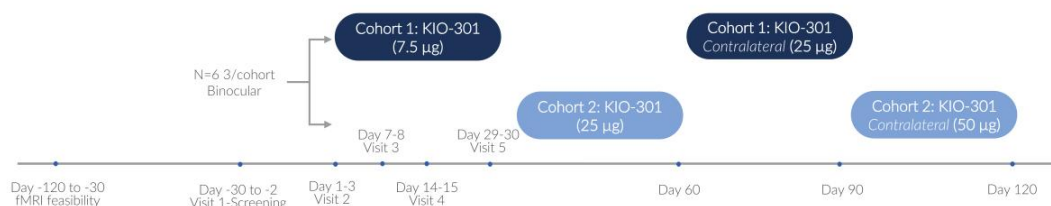
<sup>‡</sup> P2X7 is solely expressed on RGCs and amacrine cells in the retina

\* Visual light causes reversible isomeric shift, blocking ion efflux through K<sub>v</sub>/HCN channels



## 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

## — KIO-301: ABACUS Aims



1

Demonstrate Proof-of-Mechanism

- Reanimating the retina

2

Explore objective assessments in these cohorts

- No predefined approvable endpoints in ultra-low vision patients

3

Obtain patient feedback

- What patients report is important at this stage

4

Do all of the above in an environment of safety and tolerability

## — KIO-301: ABACUS – What is Measured?

|            | Assessment           | Description                  |
|------------|----------------------|------------------------------|
| Objective  | Intensity & Contrast | Light Perception             |
|            | Goldmann Perimetry   | Visual Field                 |
|            | MLOM                 | Suite of 'functional' tests  |
|            | fMRI                 | Cortical Imaging             |
| Subjective | Interviews           | Subject Feedback             |
|            | VFQ-25               | Quality of Life (QoL) Survey |

## KIO-301: ABACUS - Subject Status Update

|                     | Subject ID | 1 <sup>st</sup> Eye Completed* | Dose   | Responder† | 2 <sup>nd</sup> Eye Completed* | Dose  | Responder† |
|---------------------|------------|--------------------------------|--------|------------|--------------------------------|-------|------------|
| Cohort 1<br>NLP/BLP | 1-01       | Completed                      | 7.5 µg | ✓          | Completed                      | 25 µg | ✓          |
|                     | 1-02       | Completed                      | 7.5 µg | ✓          | In-process                     | 25 µg |            |
|                     | 1-04       | Completed                      | 7.5 µg | ✓          | To Be Scheduled                | 25 µg |            |
| Cohort 2<br>CF/HM   | 1-03       | In-process                     | 25 µg  |            | To Be Scheduled                | 50 µg |            |
|                     | 1-05       | Completed                      | 25 µg  | ✓          | To Be Scheduled                | 50 µg |            |
|                     | 1-06       | In-process                     | 25 µg  |            | To Be Scheduled                | 50 µg |            |

\* - assessments completed; data subject to availability (e.g., data entry, processing, QC, etc.)

† - positive objective response above baseline at any timepoint

## KIO-301: ABACUS Completed Subjects – Responder Matrix\*

| Subject ID | Intensity & Contrast | Visual Field | MLOM | fMRI       | VFQ-25 (QoL) | Subject Feedback | Dose Response |
|------------|----------------------|--------------|------|------------|--------------|------------------|---------------|
| 1-01       |                      |              |      |            |              |                  |               |
| 1-02       |                      |              | N/A  |            |              |                  | In-process    |
| 1-04       | N/A                  |              |      | In-process |              |                  | In-process    |
| 1-05       | N/A                  |              |      | In-process | In-process   |                  | In-process    |

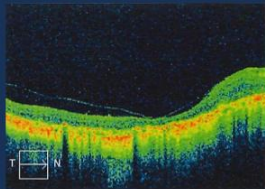
\*+ve objective response above baseline at any timepoint; N/A – testing not appropriate for level of vision or baseline  $\geq 90\%$



## KIO-301: ABACUS Safety & Tolerability

### Safe & Well Tolerated<sup>‡</sup>

#### Systemic & Ocular Safety Assessments



Slit-lamp – abnormal baseline corneal keratopathy;  
no changes observed compared to baseline.



IOP – all patients normal and no change to baseline, except 1  
patient had bilateral increase in IOP (21→27 mmHg).  
Rapidly responded to pharmacological intervention (timolol).



Dilated Fundus w/ photography\* - abnormal at baseline  
(consistent with RP); no change to baseline.



OCT - no macula edema, no change in thickness.

<sup>‡</sup> Mild; IOP increase coded as possibly related; eye soreness after injection coded as unrelated  
\* Performed within 6 hours of injection & Day 29

## PT 1-02: Intensity & Contrast Assessment\*

Visit 1 (Baseline)

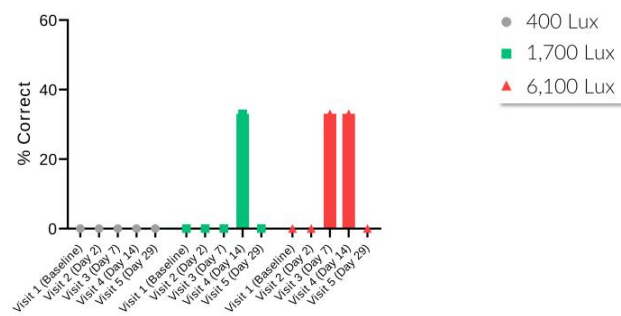


Visit 3 (Day 7)



\* - A series of six (6) visual stimuli ("X" at logMAR 2.0) presented to the patient at 3 light intensities

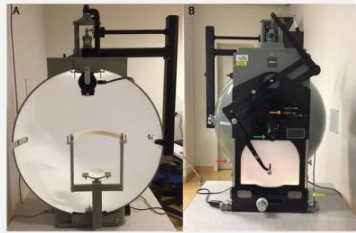
## Pt 1-02: Intensity & Contrast Assessment



### Key Takeaways:

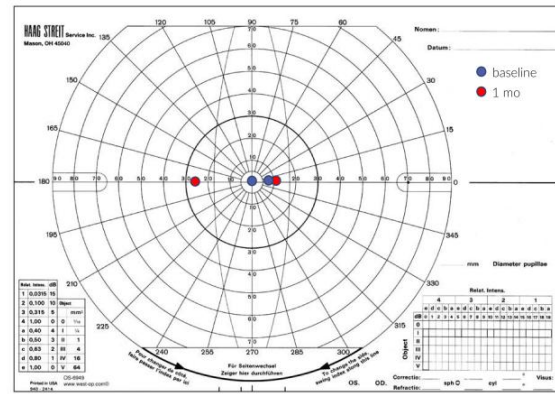
- Light perception increases over first 2 weeks following injection
- Return to baseline expected

## Kinetic Visual Field Goldmann Haag-Streit



- Assessment by orthoptists
- Measure total horizontal and vertical degrees

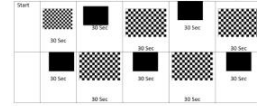
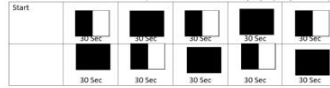
Pt1-01



|          | Total Horizontal<br>Degrees-OD | Total Vertical<br>Degrees-OD |
|----------|--------------------------------|------------------------------|
| Baseline | 8                              | 9                            |
| 1-month  | 28                             | 14                           |

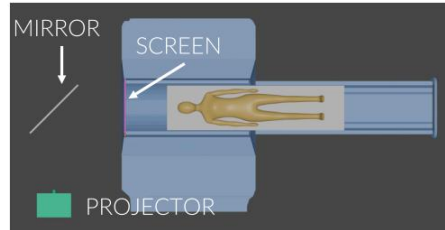
## Functional MRI – Setup & Analysis

Cohort 1 Paradigms (BLP/NLP)



### Processing and Analysis

- BOLD signal acquired
- Preprocessing (e.g., spatial normalization)
- Quantitative analysis using FSL (GLM) - ongoing



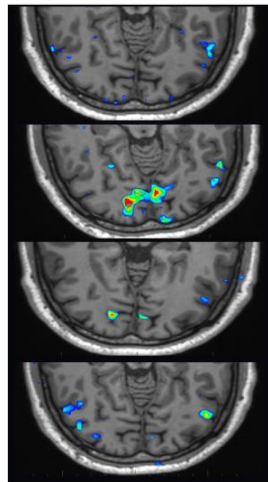
## Pt 1-02: fMRI – Qualitative Overlap of 3 Paradigms

Visit 1  
(Baseline)

Visit 2  
(Day 3)

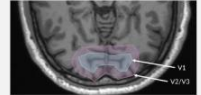
Visit 4  
(Day 15)

Visit 5  
(Day 30)

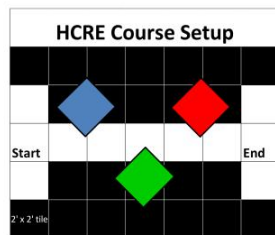


### Key Takeaways

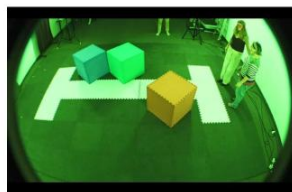
- Clear striate (V1) increase in activity at Visits 2 & 4 compared to Visit 1
- Visit 5 returns to similar activity as baseline



## MLOM Example (High Contrast Room Exit - HCRE)



Baseline

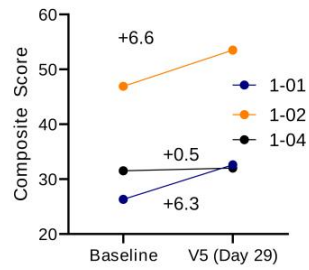


Visit #5 (Day 28)

A test of functional vision  
 Suited best for CF/HM Patients (Cohort 2)

| #1-05 | Visit #       | Pass/Fail |
|-------|---------------|-----------|
|       | V1 (Baseline) | fail      |
|       | V2 (Day 2)    | fail      |
|       | V3 (Day 7)    | pass      |
|       | V4 (Day 14)   | pass      |
|       | V5 (Day 28)   | pass      |

## KIO-301: ABACUS Quality of Life Survey (VFQ-25)



NEI-VFQ-25  
administered at  
Visits 1 & 5

2 - 4 point  
increase is  
accepted by  
payers as clinically  
meaningful\*

\* - HMSA Medical Policy - Luxturna - 2022

## PROs: Direct Feedback Interviews

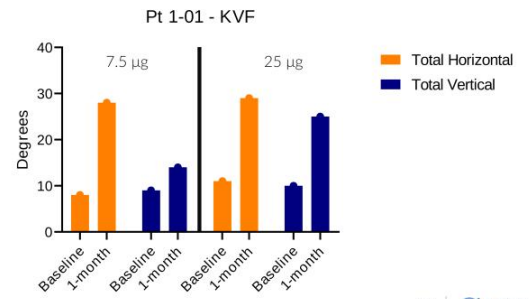
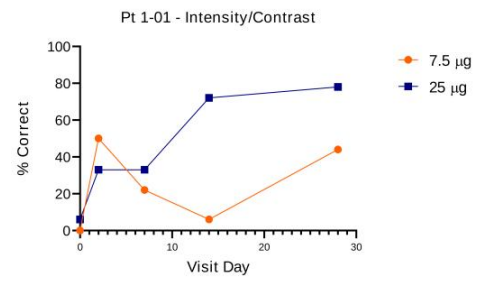
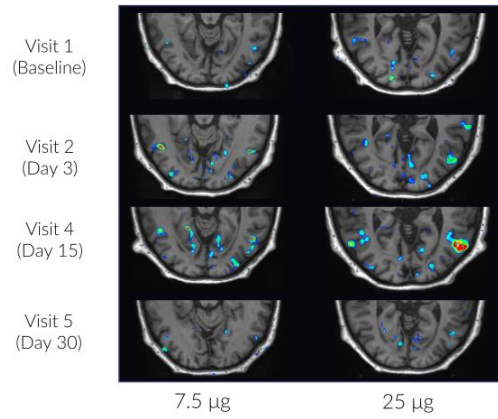


Subject 1-02



Subject 1-05

## Dose Response Observed



## — KIO-301: ABACUS Key Takeaways

- ✓ Intravitreal KIO-301 is safe and tolerable, to date
- ✓ All patients treated demonstrate objective and subjective responses
- ✓ Dose response
  - ✓ Appears to have more robust response & longer duration of effect
- ✓ Enrollment complete, dosing ongoing with full data expected in Q4 2023



## Beyond RP for Photoswitch Platform

### Indications

- Other inherited retinal diseases (rod-cone dystrophies, choroideremia, ...)
- Age-related macular degenerative diseases
  - > Geographic atrophy
  - > Late-stage wAMD
- In combination with any and all gene-replacement therapies
- Screening for optogenetics

### Expanding Exclusivity

- Protected through at least 2041 with combination of formulation, methods, and CoM patents
- Orphan Drug Designation regulatory protection

## Retinal Disease Therapies Experiencing Strong Adoption

| Indication                              | Therapies & Pricing                                                                                                                        | Patients        |
|-----------------------------------------|--------------------------------------------------------------------------------------------------------------------------------------------|-----------------|
| Wet AMD                                 | Vabysmo (Roche): \$13,140 year 1, \$6570 after<br>Eylea (Regeneron): Wet AMD & DME, \$16,000                                               | 8+ million (US) |
| Geographic atrophy                      | Syfovre (Apellis): \$26,500 per year (\$18.4 MM 1 <sup>st</sup> Qtr of sales)<br>Zimura (Astellas via \$5.8 BB Iveric buyout): pricing TBD | 1+ million (US) |
| LCA<br>(RPE65 gene defect)              | Luxturna (Roche via \$4.3 BB Spark buyout): \$850K per patient                                                                             | 180K (WW)       |
| Retinitis Pigmentosa<br>(gene agnostic) | KIO-301                                                                                                                                    | 100K (US)       |

# KIO-101

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Ocular Presentation of Rheumatoid Arthritis & Other  
Autoimmune Diseases (OPRA+)

# KIO-101: Selectively Targets T-Cell Mediated Inflammation in the Eye

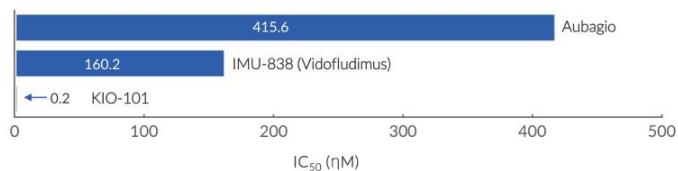
Disease-Modifying Antirheumatic Drugs are validated systemic therapeutics for patients with autoimmune diseases

- DHODH\* is a validated target clinically and commercially
- \$2B+ global sales in 2022†

Systemic approaches do not deliver sufficient drug to ocular surface to drive:

- Decreases T<sub>H</sub> cell function & proliferation locally
- Overcomes systemic delivery shortcomings

KIO-101 has Demonstrated Greater Specificity & Potency



\*Dihydroorotate Dehydrogenase  
† Sanofi 10-K 2022



## Novel Approach to Address Major Need Among RA Patients & Beyond

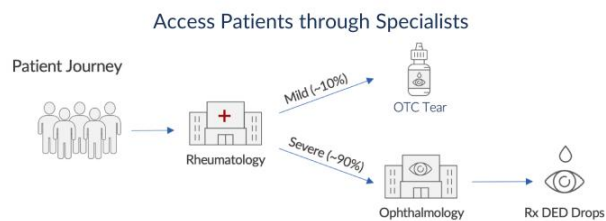
Ocular Surface Discomfort is the Most Common Non-Articular Complaint

### DHODH Inhibition Ideally Suited for OPRA+

- Immune system attack of synovial joints manifests similarly on the ocular surface

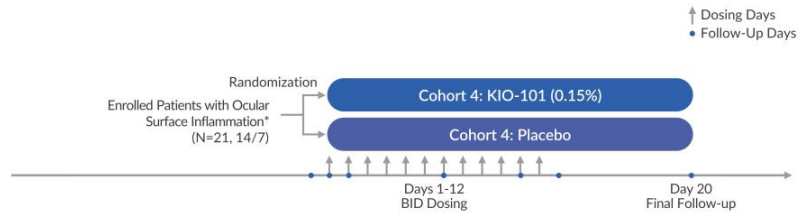
### Large Addressable Population

- US prevalence ~3.43M (ocular presentation of RA, psoriatic disease, SLE, or fibromyalgia)





## KIO-101: Exploratory Phase 1b Ocular Surface Inflammation Trial



### Study Design

- Two cohorts, randomized, double masked, placebo controlled
- OSDI  $\geq 22$ , conjunctival hyperemia  $\leq$  grade 2 (Efron Scale)

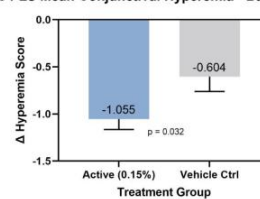
### Outcome Measures

- Safety, AEs & PK
- OSDI, Conjunctival Hyperemia, Corneal Staining, Tear Break-Up Time

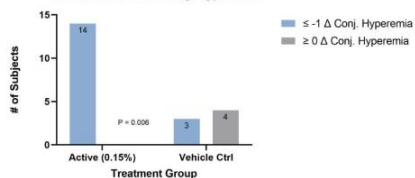
### Safety & Tolerability

- 0.15% was well tolerated in patients with ocular surface inflammation

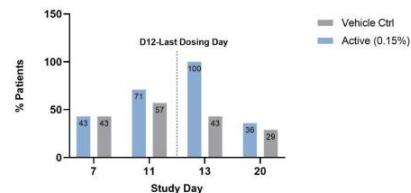
C4-LS Mean Conjunctival Hyperemia - Baseline:D13



C4-Baseline:D13 Δ Conj. Hyperemia



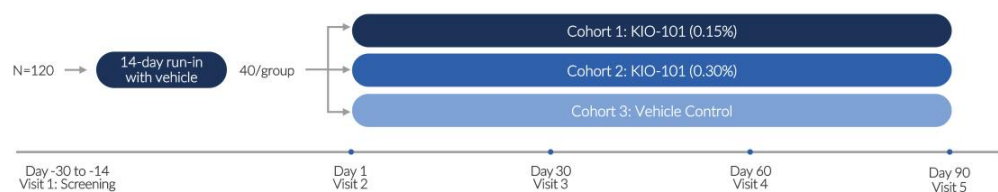
% Patients w/ <-1 Hyperemia Reduction





## KIO-101: Phase 2 OPRA+

Randomized, Multicenter, Double Masked, Multiple Ascending Dose Trial



### Study Design

- Three-arm, randomized (1:1:1), controlled, double masked, 90-day BID topical dosing
- Dx of RA (or other AI disease), conjunctival hyperemia, ODS-VAS
- Up to 120 patients

### Outcome Measures

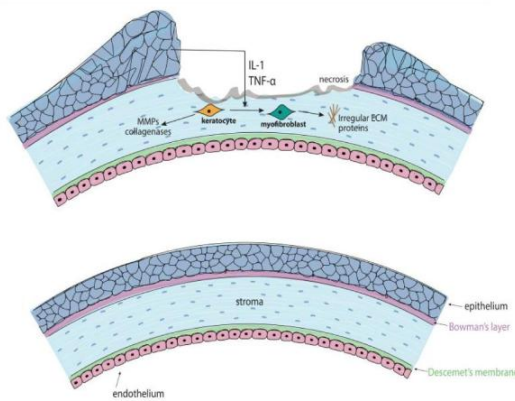
- ODS-VAS, Schirmer's, conjunctival hyperemia, corneal staining, other
- AEs & safety labs

## KIO-201

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Proprietary, Modified Form of Hyaluronic Acid (HA) to Heal  
Challenging Ocular Surface Wounds

## Persistent Corneal Epithelial Defects (PCED)

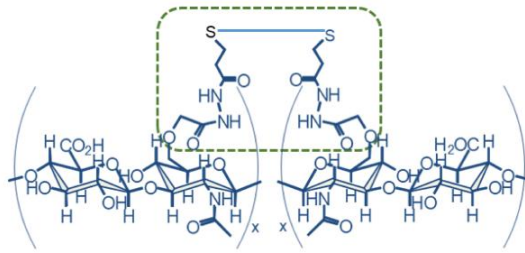


### Unmet Medical Need (Orphan Indication)

- Failure of normal closure of a corneal injury in 10–14 days, despite standard of care
- SoC is sub-optimal and usually treated with Bandage Contact Lenses
  - High recurrence rates
  - High patient discomfort
- Only a few pipeline products in development
- Ideal therapeutics would:
  - Promote epithelium healing
  - Provide physical barrier to protect surface
  - Enables greater epithelial cell migration

\* American Academy of Ophthalmology, Ocular Surgery News; April 10, 2019, Med. Hypothesis Discov. Innov. Ophthalmol. 8 (2019): 163-176.

## KIO-201: Proprietary, Cross-Linked Form of HA



### Ideally Suited to Promote Healing

- HAs known to promote epithelium healing
- Provides physical barrier to protect surface
- Enables greater epithelial cell migration
- "Normal" HAs limited by residence time & blurring

### Clinical Experience Across Multiple Trials

- 3 PRK surgical recovery (2 pilot, 1 pivotal\*)
- 2 dry eye disease

\* Regulated as a medical device until 2020  
American Academy of Ophthalmology, Ocular Surgery News: April 10, 2019; Med. Hypothesis Discov. Innov. Ophthalmol. 8 (2019): 163-176.

# KIO-201: Phase 2 PCED Study

## Single-Arm, Open Label Trial



### Study Design

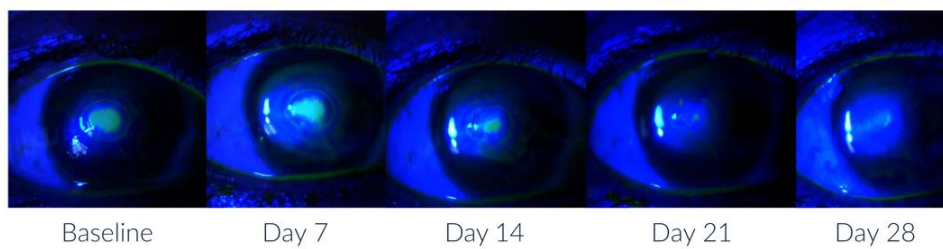
- Single-arm, open label, 28 days 6x daily topical dosing
- Dx of Stage 1 or 2 PCED
- Up to 10 patients

### Outcome Measures

- Safety & tolerability
- % of patients healed (<0.5 mm lesion size) at week 4, time to complete healing

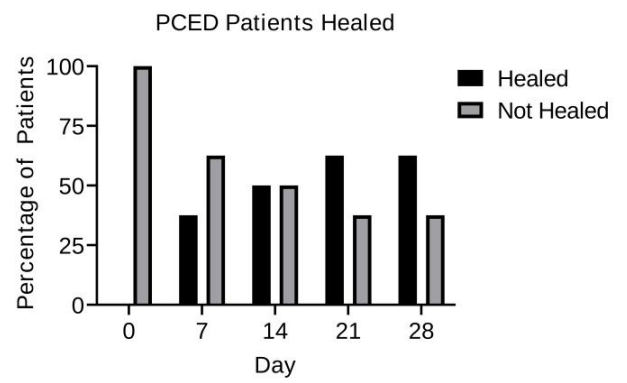
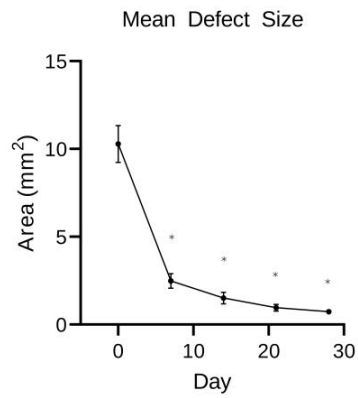
Data presented at ARVO 2023

## — KIO-201: PCED Study Results



Fluorescein staining of a representative patient in the study

## KIO-201: PCED Study Results



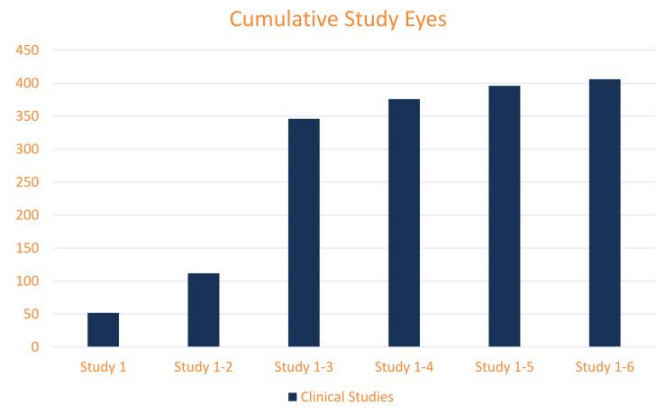
\* p<0.003 versus baseline

## Substantial Clinical History

### 6 clinical studies to date

- PRK Recovery 1
  - N=39 subjects; 78 eyes
- PRK Recovery 2
  - N=45 subjects; 90 eyes
- PRK Recovery 3
  - N=234 subjects; 468 eyes
- Punctate Epitheliopathies 1
  - N=30 subjects; 60 eyes
- Punctate Epitheliopathies 2
  - N=20 subjects; 40 eyes
- Persistent Epithelial Defects
  - N=10 subjects; 11 eyes

Patients Receiving KIO-201: 218  
Eyes Receiving KIO-201: 406





# CORPORATE OVERVIEW

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Financials, Management & Milestones

## Financials & Capitalization

As of March 31, 2023

|                            |         |
|----------------------------|---------|
| Cash & Equivalents         | \$3.4M  |
| ELOC Available*            | ~\$9.6M |
| R&D Credit Tax Receivables | \$1.7M  |

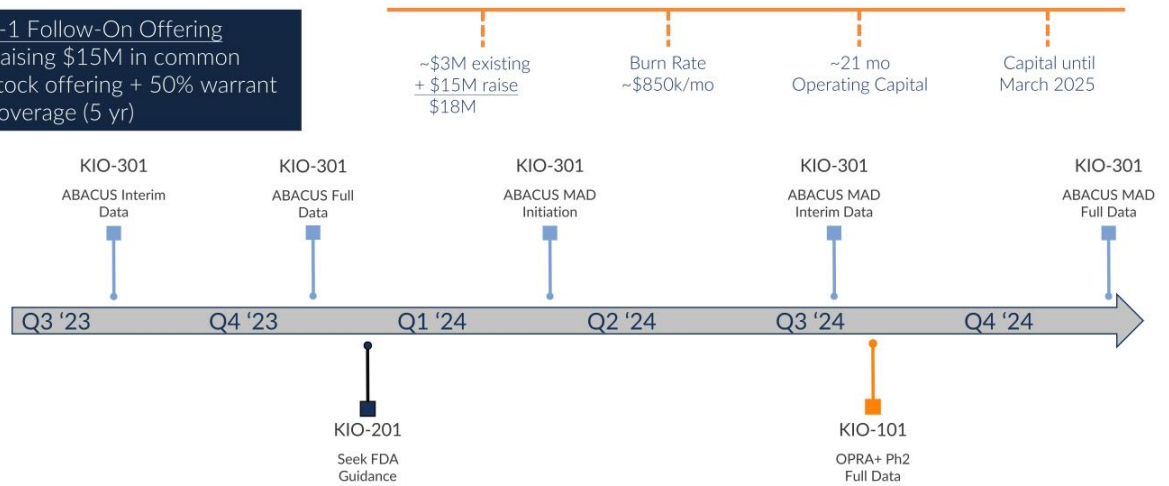
Clean cap table – no ratchets/resets/ACEs;  
No debt

| Capitalization as of May 11, 2023                              | Common Stock Equivalents |
|----------------------------------------------------------------|--------------------------|
| Common Stock                                                   | 2,024,270                |
| Series D Convertible Preferred (convertible @ \$141.28/ share) | 52                       |
| Warrants (WAEP \$16.33)                                        | 1,613,483                |
| Options (WAEP \$17.01)                                         | 211,578                  |
| RSAs                                                           | 70,550                   |
| ESPP                                                           | 191                      |
| Available Option Pool                                          | 11,175                   |
| Total Fully Diluted                                            | 3,931,299                |

\*As of March 31, 2023, \$9.9M ELOC available. Additional draws of \$0.3M completed in April 2023 reported as subsequent event in Q1 2023 10Q. 4,575 common shares pending settlement.

## Capital Raise and Milestones

S-1 Follow-On Offering  
Raising \$15M in common  
stock offering + 50% warrant  
coverage (5 yr)



## 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



## Board of Directors



Paul Chaney  
Chairman



Ken Gayron



David Hollander, MD, MBA



Erin Parsons



Aron Shapiro



Praveen Tyle



Brian M Strem, PhD  
President & CEO

## Scientific Advisory Board

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Christine Kay, MD, PhD



Mark Pennesi, MD, PhD



Russel Van Gelder, MD, PhD



Charlie Wyckoff, MD, PhD



Daniel Durrie, MD



Paul Karpecki, OD, FAAO



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