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's 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.
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

<table>
<thead>
<tr>
<th>Product Route of Delivery</th>
<th>Indication</th>
<th>Development Stage</th>
<th>Prevalence*</th>
</tr>
</thead>
<tbody>
<tr>
<td>KIO-301 Intravitreal</td>
<td>Retinitis Pigmentosa (Mutation Agnostic)</td>
<td>曾获得孤儿药认定（美国）- 2022年3月</td>
<td>250,000</td>
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<td></td>
<td>Choroideremia</td>
<td></td>
<td>16,000</td>
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<td></td>
<td>Stargardt's Disease</td>
<td></td>
<td>99,000</td>
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<tr>
<td>KIO-104 Intravitreal</td>
<td>Posterior Non-Infectious Uveitis</td>
<td>曾获得孤儿药认定（欧盟）- 2015年5月</td>
<td>180,000</td>
</tr>
</tbody>
</table>

*Approximate 2023 populations. Orpha.net, NORD, Ophthalmo Ther. 2021 Sep; 10(3).
Upcoming Clinical/Regulatory Milestones

- **KIO-301**
  - FDA pIND RP
  - ABACUS Top Line Data
  - Initiate Choroideremia Ph2 Trial
  - ODD Choroideremia

- **KIO-104**
  - ODD Posterior Non-Infectious Uveitis
  - Choroideremia Ph1b PoC Data

- **KIO-301**
  - ABACUS-2 Ph2 Initiation
  - Top Line Data*

- **KIO-301**
  - Initiate Stargardt’s Ph2 Trial
  - Stargardt’s Ph2 Top Line Data*

- **KIO-301**
  - FDA pIND RP

* Excludes open label extension
RP - Retinitis Pigmentosa, ODD - Orphan Drug Designation
KIO-301

Small Molecule Targeting Vision Restoration

Retinitis Pigmentosa, Choroideremia, Stargardt's Disease
Many Inherited Retinal Diseases, including Retinitis Pigmentosa (RP), result in death of photoreceptors. Bipolar Cells and Retinal Ganglion Cells (RGCs) remain intact and retain ability to send signals to the brain.
KIO-301: Turns RGCs “ON” in the Presence of Light

- When 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*

* Visual light causes reversible isomeric shift, blocking ion efflux through $K_v$/HCN channels
† P2X7 is solely expressed on RGCs and amacrine cells in the retina
Retinitis Pigmentosa
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
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

NLP – No Light Perception, BLP – Bare Light Perception, HM – Hand Motion, CF – Counting Fingers
Patient Reported Outcomes

Pt1-02
Baseline VA: NLP

Later that week, I went home back home

Pt2-05
Baseline VA: CF

I could see better.

Pt1-03
Baseline VA: HM

VA – Visual Acuity, NLP – No Light Perception, CF – Counting Fingers, HM – Hand Motion
Pt 1-02 Case Study: Baseline NLP

Key Takeaways

- Improvements in Light Perception and QoL
- Clear striate (V1) increase in activity at Visits 2 & 4 compared to Visit 1
ABACUS

KIO-301 IVT is safe & tolerable

KIO-301 reanimates the retina in the blind

Next Steps:

Enrollment complete → full data expected in Q4 2023

Increasing enrollment, based on initial findings, with 3 additional NLP subjects

pIND with FDA planned for Q4 2023

Initiate Sham-controlled, Masked, Randomized, Multiple Dose, Multi-Center Phase 2 Trial in Q1 2024

Expand into other IRDs with similar pathology
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.

### Presentation Usually Between 5-16 Years Old
- Known family history
- Night blindness

### Progression Occurs Between 16-40 Years Old
- Loss of peripheral vision

### End Stage of Disease around 40 Years Old+
- Retinal degeneration
- Central vision loss
- Total blindness

*Clinicaltrials.gov as of 1 July 2023*
The Choroideremia Research Foundation (CRF) is the largest global not-for-profit organization focused on the search for a cure for Choroideremia (CHM).

- Education and Awareness of CHM and KIO-301
- CHM KOL Network to Assist in Clinical Protocol Design
- CHM Patient Identification to Assist in Trial Enrollment
Stargardt’s Disease: No Approved Therapeutics

- **Orphan Disease**: prevalence of 1:10,000 ~30,000 patients in US

- **Autosomal** recessive disease inherited from parent carriers, typical onset in 2nd decade of life, vision loss in 4th-5th decade.

- **Cause**: Mutation in the ABCA4 or ELOVL4 gene

- ** Accumulation** of lipofusion 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-301-2101: Phase 2 Study Design
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

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-γ

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 IC₅₀)*
- KIO-104 is first-in-class in ophthalmology

*1,000x more potent than Teriflunomide (Aubagio© - 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)
CORPORATE OVERVIEW
Leadership Team

Brian M. Strem, PhD
President & CEO

Eric J. Daniels, MD
Chief Development Officer

Melissa Tosca, CPA
EVP – Finance

Stefan Sperl, PhD
EVP – CMC & Operations
Scientific Advisory Board

Allen Ho, MD, PhD  
WillsEye Hospital

Christine Kay, MD, PhD  
Vitreo Retinal ASSOCIATES

Mark Pennesi, MD, PhD  
OREGON HEALTH&SCIENCE UNIVERSITY

Russel Van Gelder, MD, PhD  
UW Medicine

Charlie Wykoff, MD, PhD  
Retina Consultants of Texas™
Contact:
info@kiorapharma.com