at a glance™

KIORA PHARMACEUTICALS

Of Note: ***Patient dosing is underway in the Phase 1b clinical trial of KIO-301, Kiora's novel small molecule designed to restore sight to people afflicted with a genetic form of blindness for which there is no cure. Interim data is expected before year-end. The company is developing two additional ophthalmic candidates. One aims to address dry eye-like signs and symptoms in rheumatoid arthritis (RA) patients; the other is enrolling a Phase 2 trial in patients with non-healing eye surface wounds. New management has streamlined operations and focused the company on high value programs. Near-term catalysts are expected.***

Key Considerations

- **Development stage company focused on rare & underserved ophthalmic diseases**
- **KIO-301 is a first-in-class, small molecule in development to restore vision in people living with retinitis pigmentosa (RP), a progressive inherited eye disease that leads to irreversible blindness.**
- **A Phase 1b trial of KIO-301 is dosing patients and expects to complete enrollment and report initial interim results by the end of this year.**
- **There is no approved therapy for RP. It affects about one in 3,500, or roughly 100,000, people in the US alone. KIO-301 has been designated an Orphan Drug by the FDA.**
- **KIO-101 is a potential treatment for dry eye-like symptoms in RA patients. It is expected to enter a Phase 2 clinical trial in 4Q22. The small molecule is formulated as an eye drop to target an enzyme called DHODH to reduce inflammation. DHODH inhibition is a validated target in autoimmune diseases.**
- **KIO-201 is a liquid ocular bandage, delivered as an eyedrop, and designed to accelerate healing of the ocular surface after surgery or traumatic injury. It is being developed to treat a rare condition, Persistent Corneal Epithelial Defect (PCED), characterized by non-healing eye surface wounds.**

Kiora Pharmaceuticals, Inc.
(Nasdaq: KPRX)

Recent Price:	**$6.45**
Shares O/S:	**1.1 Million**
Approx. Mkt Cap:	**$7.0 Million**
Fiscal Year Ends:	**Dec. 31**

Published: November 2022

About KIO-301

KIO-301 is being developed for the treatment of RP. It has the potential to restore light perception in patients with inherited and/or age-related retinal degeneration.

RP is the number one cause of inherited blindness in the world. It is progressive, often starting in the second decade of life. Gradual progression is increasingly debilitating until it results in total blindness, typically by the fourth or fifth decade.

The disease is caused by one of multiple genetic mutations, each distinct, but all characterized by the same symptoms: initially deteriorating vision in low light conditions such as at dusk or nighttime, followed by peripheral vision loss and finally central vision loss.

RP progresses due to the death of light sensitive photoreceptors (located in the in the back of the eye, or retina). This prevents the retina from performing its normal function of converting light into electrical signals that are transmitted to the brain where they are processed into visual images for interpretation.

KIO-301 is designed to restore the eyes' ability to see or perceive light in the regions that have degenerated photoreceptors (rods and cones), regardless of the mutation and in contrast to several gene therapies being developed for RP. As a small molecule, it will be delivered through a routine injection into the eye like many age degeneration products.

KIO-301 works by converting downstream neural cells into light sensing receptors, thereby renewing the retina's ability to send electrical signals to the brain for processing.

This mechanism has been validated in numerous non-clinical studies, which also showed that visual signals generated by KIO-301 enable blind mice to alter a trained behavior due to the presence of light.

Recent & Anticipated Milestones

-2Q22 – Initiated Phase 2 study for KIO-201 in patients with PCED

-3Q22 – KIO-301 received regulatory authority clearance to start a Phase 1b trial in patients with later-stage RP

-4Q22 – Patient dosing initiated in a Phase 1b study of KIO-301 in RP

-4Q22 – Initial interim data from the Phase 1b study of KIO-301 in RP

-4Q22 – Initiate a Phase 2 trial for KIO-101 to treat the Ocular Presentation of Rheumatoid Arthritis (OPRA)

-1Q23 – Report topline data from KIO-301 and KIO-201 studies

Kiora has initiated patient dosing in ABACUS, a Phase 1b open label trial in patients living with retinitis pigmentosa being conducted at the Royal Adelaide Hospital in Adelaide, South Australia. Interim data is expected to be released before year-end 2022.

The study will enroll six patients and evaluate 12 eyes. The first cohort of three patients will include individuals with no or low light perception due to the progression of retinitis pigmentosa. The second cohort will include patients able to detect hand motion and count fingers.

The primary endpoints are safety and tolerability. The secondary efficacy endpoints include object identification, intensity, and contrast assessment, navigation, functional MRI and other ophthalmic and quality of life assessments.

About KIO-101

KIO-101 is being developed as a treatment of dry eye-like symptoms in patients with RA. Of the approximately 1.8 million Americans with RA, one in three have a form of ocular surface disease (dry eye), referred to as Ocular Presentation of RA (OPRA).

Dry eye is the second most prevalent complaint of RA patients, second only to underlying rheumatic disease in the joints. It represents an opportunity to address a major unmet need, as no product is approved specifically for the approximately 500,000 patients living with this condition the US.

KIO-101 inhibits a key enzyme called DHODH that drives disease progression in RA patients through the release of unwanted immune and inflammatory cells. DHODH is a validated drug target.

Multiple DHODH inhibitors have been approved to treat diseases such as multiple sclerosis (Aubagio® and Arava®) and combine for billions of dollars in annual sales.

Kiora's eye drop formulation is designed to provide localized delivery of the drug to the ocular surface where it is expected to have a more direct effect on inflammation by reducing the number and activity of immune cells that are driving the disease. Delivering the drug locally is important because studies show that orally dosed DHODH inhibitors don't reach the ocular surface in a sufficient concentration.

Kiora completed a Phase 1/1b dose escalation clinical trial in both healthy volunteers and in patients with Ocular Surface Inflammation in 4Q21.

KIO-101 was generally well tolerated and topline data showed successful improvements in reducing several symptoms, including hyperemia. Based on these results, the company expects to initiate a Phase 2 clinical trial in 4Q22, with an anticipated readout in 1Q24.

About KIO-201

KIO-201 is being developed to accelerate the healing of the ocular surface after ocular surgery, or other wound causing incidents. This can include PCEDs, a rare ocular surface condition characterized by non-healing eye surface wounds.

PCEDs can be caused by a variety of factors including physical trauma, surgical injury, infections or ocular manifestation of other diseases such as rheumatoid arthritis or diabetes.

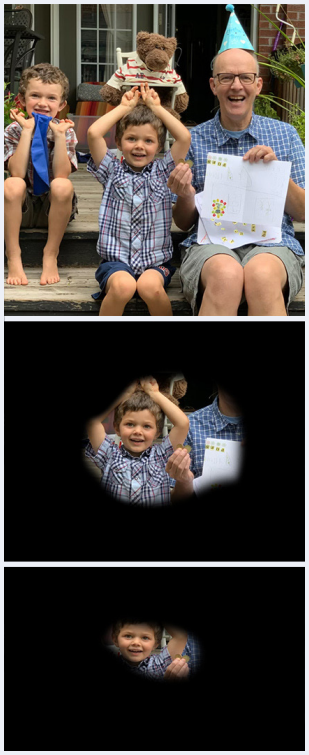
As an eye drop, KIO-201 provides a thin coating of a modified form of the naturally occurring hyaluronic acid to the surface of the eye, to protect it and to speed corneal re-epithelization.

It is intended to function as a mechanical barrier, like a liquid bandage for the ocular surface, designed to be a scaffolding for epithelium to migrate onto and to protect the underlying wound from the constant shear forces of blinking.

Kiora initiated a phase 2 study evaluating KIO-201 topical eye drops in patients in 2Q22. Promising results from this study will support efforts to potentially advance KIO-201 to a registration study next year.

KIO-301

Simulated images below depict the progressive loss of central vision in patients with RP, an inherited orphan disease that leads to irreversible blindness. There are no current therapies. Kiora expects to begin enrollment in 4Q22 of a Phase 1b study of KIO-301 in patients with later-state RP. Initial interim results are expected in 4Q22. The disease afflicts about 100,000 in the US alone.



Summary

- **KIO-301 is a revolutionary small molecule with the potential to restore vision in patients with inherited or age-related blindness. Interim data from a Phase 1b trial is expected to be released by the end of this year.**
- **KIO-101 is being developed for the treatment of the Ocular Presentation of Rheumatoid Arthritis (OPRA). This drug has the potential to become the first treatment specifically for the approximately 500,000 patients suffering from dry eye-like symptoms due to rheumatoid arthritis. A Phase 2 trial is expected to start 4Q22.**
- **In addition, Kiora is developing KIO-201 for Persistent Corneal Epithelial Defect (PCED), a rare disease that addresses an unmet need for ocular wound healing. A Phase 2 clinical trial is underway to support a potential registration study.**
- **New leadership has refocused the company to high value programs under its CEO, Brian M. Strem, PhD, who specialized in ophthalmology at Allergan and Shire. He received degrees in bioengineering from Cornell University and UCLA, where he earned his PhD.**

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