Kiora's Investigational Treatment for Retinitis Pigmentosa, KIO-301, Demonstrates Visual Function Restoration in Patients Who Are Blind

Encinitas, California–(April 27, 2023) – Kiora Pharmaceuticals (NASDAQ: KPRX) presented preliminary results from an ongoing clinical trial showing its investigational drug, KIO-301, has the potential to restore light perception in patients who are blind or living with ultra-low vision due to retinitis pigmentosa. KIO-301 is a light-sensing small molecule designed to reactivate visual function of the eye in response to light. The results were presented today at the Association for Research in Vision and Ophthalmology (ARVO) annual meeting in New Orleans. To view a full multimedia version of this announcement, including downloadable images, videos, graphics, presentation slides, and more, click here.



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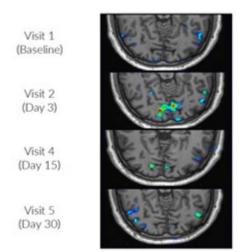
"These preliminary results suggest that the initial low dose of KIO-301 is capable of improving light perception in patients who have ultra-low vision or are completely blind," Dr. Robert Casson, Principal Investigator of the study from the Royal Adelaide Hospital. "We saw a marked improvement in functional measures, which correlate to neural imaging results that demonstrate increased visual cortex activity in the brain from baseline to two weeks after treatment."

Quantitative and qualitative measures from early patients in the study were reported. The highlighted case study presented is a patient with no light perception and received a low dose injection of KIO-301 in one eye. The following observations were reported:

- Patient-reported improvement in the ability to perceive a contrast between light and dark at days seven 14 and 29.
- Improvement in object identification.

- Positive impact in overall functional vision as it relates to the use of vision in everyday activities.
- Substantial improvement in quality of life, as measured by the National Eye Institute's visual functional questionnaire. There was an increase of six points (an increase of two to four points is considered clinically meaningful¹).
- Imaging data, using functional MRI, showed significantly increased activity in areas of the brain (striate V1 region of the visual cortex) at days three and 15 post-injection compared to baseline (See Figure 1).
- The initial dose was safe and well-tolerated at day 29, with no adverse events reported throughout the study.
- The duration of effect is consistent with preclinical pharmacokinetic data.

Functional MRI – Qualitative Overlap of 3 Paradigms



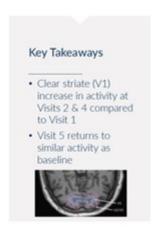


Figure 1. Functional MRI images of the brain visual cortex of patient treated with KIO-301 (from visit one to visit five), and reference image showing striate V1, V2 and V3 regions.

To view an enhanced version of this graphic, please visit: https://images.newsfilecorp.com/files/8481/163965_50bf9e85b43d0b80_002full.jpg

"The early results demonstrate profound improvements in light perception and functional vision," said Eric Daniels, M.D., MBA, Chief Development Officer of Kiora Pharmaceuticals. "As a confirmatory measure, and being an open label study, we reviewed preliminary observations from the first patients treated and felt it was important to share these early findings. The collective body of data supports our plan to escalate doses, complete enrollment, and report full results to the medical and patient communities later this year."

Retinitis pigmentosa is a group of rare, inherited diseases that involve a breakdown and loss of the photoreceptor cells in the retina, the light-sensing tissue that lines the back of the eye. This progressive disease usually initially presents with difficulty seeing at night and a loss of side (peripheral) vision, extending to tunnel vision and eventually, full blindness.

"Unfortunately, there are no approved therapies to help patients with retinitis pigmentosa, which is estimated to affect 100,000 people in the U.S. alone," added Brian M Strem, Ph.D., President and CEO of Kiora Pharmaceuticals. "We see KIO-301 as a potential treatment option across all genetic causes of retinitis pigmentosa as an easy-to-deliver small molecule. Based on how KIO-301 works as a light sensing photoswitch, we also see it potentially addressing additional eye diseases, including other inherited and age-related retinal degenerative diseases, namely geographic atrophy and late-stage wet AMD."

The trial, called the ABACUS study, is a Phase 1b open-label, single ascending dose clinical trial for people living with retinitis pigmentosa. The study comprises the enrollment of six patients and the evaluation of 12 eyes, following monitoring for 29 days. The first cohort of three patients includes individuals with no or bare light perception due to the progression of RP. The second cohort includes three patients able to detect hand motion and count fingers. The primary endpoints are safety and tolerability, with secondary efficacy endpoints including object identification and contrast assessment, navigation, fMRI and other ophthalmic and quality-of-life assessments. This study is being conducted at multiple sites in Adelaide, South Australia, including The Royal Adelaide Hospital.

KIO-301 is a visible light-sensitive small molecule that acts as a reversible 'photoswitch', specifically designed to restore the eyes' ability to perceive and interpret light in visually impaired patients. KIO-301 selectively enters retinal ganglion cells (those downstream of degenerated rods and cones) and 'switches' them into light sensing cells, capable of signaling the brain as to the presence or absence of visible light.

The presentation (5444), titled, "An Intravitreal 'Photoswitch' Molecule (KIO-301) for Reanimation in Retinitis Pigmentosa: a first-in-human trial," was presented by Eric Daniels, MD, Chief Development Officer of Kiora.

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About Kiora Pharmaceuticals

Kiora Pharmaceuticals is a clinical-stage biotechnology company developing and commercializing products for the treatment of ophthalmic diseases. KIO-301 is being developed for the treatment of retinitis pigmentosa. It is a molecular photoswitch that has the potential to restore vision in patients with inherited and/or age-related retinal degeneration. KIO-101 is being developed for the treatment of the Ocular Presentation of Rheumatoid Arthritis ("OPRA"). It is a next-generation, non-steroidal, immuno-modulatory and small molecule inhibitor of Dihydroorotate Dehydrogenase ("DHODH") with what Kiora believes is best-in-class picomolar potency and a validated immune modulating mechanism (blocks T cell proliferation and proinflammatory cytokine release) designed to overcome the off-target side effects and safety issues associated with commercially available DHODH inhibitors. In addition, Kiora is developing KIO-201, a modified form of the natural polymer

hyaluronic acid, designed to accelerate corneal wound healing.

In addition to news releases and SEC filings, we expect to post information on our website, 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.

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, the ability of KIO-301 to restore visual function in patients with retinitis pigmentosa, the potential for KIO-301 to address other eye diseases, Kiora's ability to enroll patients and report results from its Phase 1b trial of KIO-301 on a timely basis, the development and commercialization efforts and other regulatory or marketing approval efforts pertaining to Kiora's developmentstage products, including 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 press release, including, among other things, the ability to conduct clinical trials on a timely basis, the risk that the full results of the Phase 1b trial of KIO-301 may not be consistent with preliminary data, 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 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.

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