

May 6, 2024



Kiora Pharmaceuticals Reports Additional Clinical Data for KIO-301 Showing a Statistically Significant Increase in Brain Activity in the Visual Cortex in Patients with Retinitis Pigmentosa

- Previously released ABACUS-1 clinical trial results reported KIO-301, a small molecule photoswitch, is safe, tolerable, and improved vision in patients with late-stage retinitis pigmentosa.
- Additional analysis of functional MRI data demonstrated a statistically significant increase in neural activity over baseline within the brain's visual processing center.
- The increase in observed brain activity was time-dependent and demonstrated concordance with previously reported improvements in visual field, visual acuity, and functional vision.

Encinitas, California--(Newsfile Corp. - May 6, 2024) -[Kiora Pharmaceuticals, Inc.](#) (NASDAQ: KPRX), ("Kiora" or the "Company") announced additional data from ABACUS-1, the Phase I/II clinical trial in patients with retinitis pigmentosa showing KIO-301 significantly increased brain activity, specifically in the visual cortex, relative to baseline, as assessed by functional MRI (fMRI). This standard method of measuring visual cortex activity, in response to visual stimuli, is performed by assessing the quantitative change in voxels, a three-dimensional equivalent of a pixel at each visit. KIO-301 is a small molecule photoswitch providing light sensitivity to retinal cells capable of transmitting neural signals to the brain following the loss of native photoreceptors (rods and cones).

The results were presented May 5, 2024 by Professor Robert James Casson, DPhil, Head of the Ophthalmic Research Lab at The University of Adelaide, at the Association for Research in Vision and Ophthalmology (ARVO) annual meeting in Seattle, WA. Additional key findings include the following:

- A statistically significant increase in visual cortex activity from baseline at all timepoints assessed (1574.0 ± 689.7 voxels at d2, 1061.8 ± 632.1 voxels at d14, 1110.8 ± 478.4 voxels at d28, $p < 0.05$ for all timepoints, $n=12$).
- A statistically significant increase in visual cortex activity was measured in both cohorts, those with baseline vision of counting fingers or hand motion range and those with baseline vision of bare light perception or no light perception.
- A more pronounced increase in visual cortex activity was found in patients with better

baseline vision.

- The increase was time-dependent following initial administration of KIO-301, consistent with improvements in visual acuity, visual field, and functional improvements that mimic performing everyday activities.

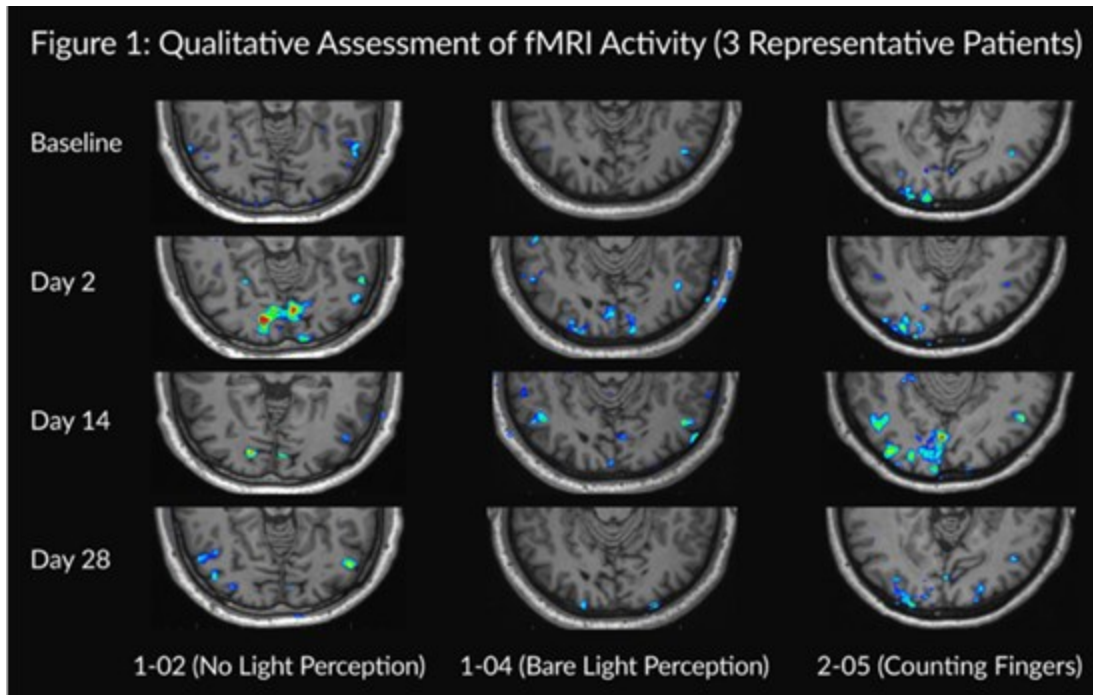


Figure 1

To view an enhanced version of this graphic, please visit:

https://images.newsfilecorp.com/files/8481/208051_e7fb33683455a9ff_002full.jpg

Figure 2: Quantitative Assessment of fMRI Activity in the Visual Cortex (Mean +/- SEM)

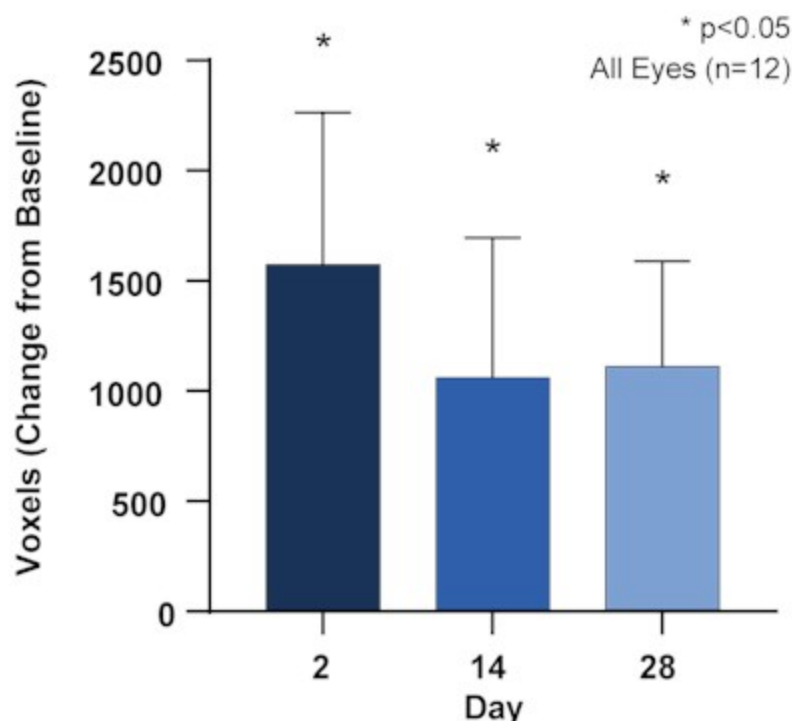


Figure 2

To view an enhanced version of this graphic, please visit:

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"These data further support KIO-301's underlying potential to restore meaningful vision, both clinically and mechanistically," explained Professor Casson. "fMRI has allowed us to visually capture and quantify the changes happening in the vision processing center of the brain, helping us connect the dots between the observed functional outcomes and the drugs' mechanism of action."

"The fMRI results reinforce our understanding of KIO-301's mechanism of action, as well as the assertion that conferring light sensitivity to cells other than the native rods and cones, can positively impact vision," added Eric Daniels, MD, Chief Development Officer of Kiora. "Importantly, this data is consistent with improvements in functional endpoints assessing change in patients' everyday activities that we and regulatory bodies believe are essential for drug approval. Our next step is to complete ongoing validation of functional endpoints and initiate a double-masked, multi-center Phase II clinical trial (ABACUS-2) in cooperation with our development and commercialization partner, Théa Open Innovation."

About KIO-301

KIO-301 is a small molecule photoswitch. It is designed to selectively confer light-sensing capabilities to retinal ganglion cells (RGCs). In healthy eyes, light is first converted to

electrical signals via rods and cones (photoreceptors) and transmitted through RGCs to the vision center of the brain (visual cortex). In many inherited retinal diseases (IRDs), genetic mutations cause photoreceptors to degenerate and die, affecting an individual's ability to perceive light. However, while photoreceptors degenerate in IRDs, RGCs remain viable. They therefore represent a target cell to bypass degenerated photoreceptors, perceive light, and signal the brain. It has been shown KIO-301 selectively enters RGCs downstream of degenerated photoreceptors. In the presence of light, KIO-301 turns to an "on" position, triggering RGCs to signal the brain. In the absence of light, KIO-301 turns to an "off" position. In this way, the molecule acts as a light switch within the eye.

In January 2024, Kiora, along with Théa Open Innovation (TOI), a sister company of the global ophthalmic specialty company Laboratoires Théa (Théa), agreed to an exclusive worldwide co-development and commercialization agreement, excluding Asia, to KIO-301 for the treatment of retinal diseases. As part of this agreement, the companies are jointly planning a phase II multi-center, randomized, controlled clinical trial (ABACUS-2).

About Retinitis Pigmentosa

Retinitis pigmentosa (RP) is a hereditary degenerative disorder affecting the retina's photoreceptors with no approved therapies. Typically characterized by progressive loss of side (peripheral) vision and night vision, it results from mutations in one or more than 150 genes. This disease affects approximately 1 in 4,000 individuals globally and about 100,000 patients in the United States alone. The prevalence, combined with the fact that 50% of patients are not qualified to drive by age 37 and are often considered legally blind by 55, underscores the need for treatment options that address as many or all of the gene mutations implicated in the disease. Kiora's development of KIO-301 as a mutation-agnostic treatment for RP could meet this need. This drug is being developed as a standalone therapy or in combination with a potential future gene therapy.

About Kiora Pharmaceuticals

Kiora Pharmaceuticals is a clinical-stage biotechnology company developing and commercializing products for the treatment of orphan 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 posterior non-infectious uveitis. 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) 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 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 ability of KIO-301 to improve visual function, the potential to expand KIO-301 to other indications including choroideremia and Stargardt disease, and the planned timing and design of the Phase II clinical trial for KIO-301. 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 ability to obtain any required regulatory approvals, whether future trials of KIO-301 will yield similar results for participants, 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 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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