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Kiora Pharmaceuticals Receives Investigational New Drug Approval to Initiate ABACUS-2, a Phase 2 Clinical Trial of KIO-301 for the Treatment of Retinitis Pigmentosa

Encinitas, California--(Newsfile Corp. - October 29, 2024) - Kiora Pharmaceuticals, Inc. (NASDAQ: KPRX) ("Kiora" or the "Company") today announced it received regulatory approval to initiate a Phase 2 clinical trial to investigate KIO-301 for vision restoration in patients with retinitis pigmentosa. The ABACUS-2 trial will be a 36 patient, multi-center, double-masked, randomized, controlled, multiple dose study enrolling patients with ultra-low vision or no light perception regardless of their underlying gene mutation associated with retinitis pigmentosa. Dosing of the first patient with KIO-301 is expected to begin next year following validation of novel functional vision endpoints. These functional assessments may serve as approvable primary endpoints in subsequent registration studies in the United States, Europe and other major regions.

"There are unfortunately no approved therapies for patients with retinitis pigmentosa," said Eric Daniels, M.D., Chief Development Officer at Kiora. "This study represents a significant step toward addressing this challenge. Prior to submission for approval, we engaged with European and US regulators to incorporate their expectations and guidance for approvable endpoints. Consistent with historical approval in other inherited retinal disease, both regulatory bodies emphasized the need to measure a therapy's effect on everyday functional vision. For this reason, we are investing time upfront to validate the functional endpoints for ABACUS-2, increasing our likelihood of success in a potential single Phase 3 trial for market approval in the US and Europe. This validation work is being performed, in collaboration with our partner Théa Open Innovation, with the support of the Choroideremia Research Foundation as part of a grant to design a standard endpoint for investigational therapies of inherited retinal diseases."

Kiora has identified and is now contracting with trial sites and clinical investigators at major inherited retinal disease reference centers across Australia. Patients will be randomized 2:1 to receive KIO-301 intravitreally or control. Treatment will be delivered to both eyes. Participants will be randomized to receive either a high dose (100 micrograms) or low dose (50 micrograms) of KIO-301 and after trial participants receive three consecutive doses (6 weeks apart), they will be followed for three months. Following this phase of the study, patients in the control arm may elect to cross-over into the active arm. Primary endpoints will consist of safety and tolerability, and key efficacy assessments include: functional vision; visual acuity as measured by the Berkeley Rudimentary Vision Test; visual fields as measured by perimetry, and a validated ultra-low vision quality-of-life questionnaire. The trial will be conducted across five centers within Australia.

"In a short window since entering our partnership with Kiora, tremendous progress has been made on advancing KIO-301 toward ABACUS-2," said Dr. Céline Olmiere, Head of Théa Open Innovation. "What makes KIO-301 compelling is that it appears, based on the Phase 1b data, to have potential for meaningful vision restoration. Further, because of its unique mechanism of action, it has the potential to work across all 150-plus underlying gene mutations associated with retinitis pigmentosa and other inherited retinal diseases."

KIO-301 is a small molecule that acts as a light-sensitive photoswitch that has the potential to return vision to patients living with reduced sight due to inherited retinal diseases. The novel compound is activated in the presence of light and deactivated in the absence of light. Because hundreds of gene mutations underlie inherited retinal diseases like retinitis pigmentosa, there is an important need for therapies like KIO-301 that have the potential to act in a gene mutation agnostic manner.

KIO-301 targets and enters specialized cells of the retina (retinal ganglion cells or RGCs) that are located 'downstream' of degenerated rods and cones, the cells normally responsible for converting light to vision. KIO-301, when inside RGCs, is activated by visible light and confers light-sensing capabilities by altering the flow of ions in and out of the cell, thus giving RGCs the ability to facilitate visual processing. This is a reversible process allowing turning on and off the RGCs in the presence and absence of light.

About Kiora Pharmaceuticals

Kiora Pharmaceuticals is a clinical-stage biotechnology company developing and commercializing products for the treatment of 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 retinal inflammation. 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.

About Théa Open Innovation

Théa Open Innovation (TOI) is a sister company of Théa, the leading independent European pharmaceutical company specialized in the research, development, and commercialization of eye care products. TOI's mission is to establish partnerships with startups, biotech companies, and universities to help bring the most cutting-edge eye care products to the market.

For more information, visit www.theaopeninnovation.com

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, Kiora's ability to execute on development and commercialization efforts and other regulatory or marketing approval efforts pertaining to Kiora's development-stage products, including KIO-

104 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, the sufficiency of existing cash on hand to fund operations for specific periods, the projected cash runway, and Kiora's plans to further fund development of KIO-104. 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 satisfy the closing conditions related to the offering ,the ability to conduct clinical trials on a timely basis, 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 including on Form 10-Q filed with the SEC on August 9, 2024. 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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