

October 17, 2022



Kiora Pharmaceuticals Announces Publication of Phase 1 Trial Results for KIO-100 as a Treatment for Ocular Inflammatory Disease

Salt Lake City, Utah--(Newsfile Corp. - October 17, 2022) -[Kiora Pharmaceuticals, Inc.](#) (NASDAQ: KPRX), ("Kiora" or the "Company") announced that results from a Phase 1 study of KIO-100 (formerly PP-001) demonstrate its potential as a treatment for non-infectious uveitis, an ophthalmic inflammatory disease. The results were published today in a paper titled "[A new small molecule DHODH-inhibitor \[KIO-100 \(PP-001\)\] targeting activated T cells for intraocular treatment of uveitis - a phase I clinical trial](#)," in the journal *Frontiers in Medicine*.

This first-in-man, open-label, phase-1 clinical trial investigated the use of KIO-100 for treating uveitis, a T cell-mediated, intraocular inflammatory disease. Results showed that a single intravitreal injection of KIO-100 decreased intraocular inflammation in a dose dependent fashion, and improved visual acuity significantly during the duration of the study. The drug was well tolerated, with no serious side effects on intraocular tissues or other adverse events observed.

KIO-100 is a dihydroorotate dehydrogenase (DHODH) inhibitor, a validated drug target in the treatment of systemic autoimmune conditions. As a third generation DHODH inhibitor with an intravitreal formulation and a high potency for suppressing T and B cells, KIO-100 is a promising non-steroidal anti-inflammatory agent.

"The publication of this data is clinical proof-of-concept for using KIO-100 as a safe and effective non-steroidal immune modulator for a wide range of inflammatory eye diseases," said Eric Daniels, M.D., Chief Development Officer of Kiora. "We look forward to building off of these promising results in future clinical studies."

Study Design and Results

Twelve adult patients with bilateral chronic, non-infectious intermediate, posterior or pan-uveitis were enrolled and divided into three groups (n=4/group) to receive a single intravitreal injection in the more severely affected eye of 100 µl containing 0.3 µg, 0.6 µg or 1.2 µg KIO-100. All patients were tested at baseline and on days 2, 7, 14, 21 and 28 post-injection. The drug was safe and well tolerated, with one transient increase in intraocular pressure reported in one eye. Consistent with intravitreal injections, mild conjunctival hemorrhages or conjunctival hyperemia were reported at the injection site in all treated eyes. All reported adverse events were considered to be unrelated to the drug.

Visual acuity was assessed using Early Treatment Diabetic Retinopathy Study (ETDRS) charts, and intraocular inflammation was assayed by measuring cells in the anterior chamber (AC) and vitreal haze. The results reported demonstrated a dose-dependent improvement in visual acuity, with the highest dose (1.2 µg) reaching three lines of improvement (equal to doubling in resolution) on day 14, and remaining stable until day 28. This improvement was statistically significant ($p < 0.05$) relative to baseline at day 14, 21, and 28. One patient in this group had trace (0.5+) haze at baseline, which disappeared after two weeks. In addition to a regression of intraocular inflammation, a decrease of retinal thickness was found in 3 of 4 eyes 2 weeks after receiving the 1.2 µg dose.

According to Stephan Thureau, M.D., lead investigator and Professor of Ophthalmology, University Hospital, LMU München, Germany, "Given the known and very real limitations of using chronic intraocular steroids, KIO-100 represents the future of controlling inflammation in the eye. This data is both encouraging and exciting for patients affected with uveitis."

Kiora previously tested the same inhibitor formulated as eye drops, KIO-101, in a phase 1 clinical trial in healthy subjects and patients diagnosed with ocular surface inflammation. The results also demonstrated favorable safety and tolerability of KIO-101 as well as statistically significant improvements in conjunctival hyperemia. The favorable results on KIO-100 further validate the development of KIO-101 as the Company pursues a program for the treatment of patients with Ocular Presentations of Rheumatoid Arthritis (OPRA).

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 chemically cross-linked 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 development and commercialization efforts and other regulatory or marketing approval efforts pertaining to Kiora's development-stage products, including KIO-101, KIO-201 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. 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, market and other conditions and certain risk factors described under the heading "Risk Factors" contained in Kiora's Amendment No. 1 to Annual Report on Form 10-K/A filed with the SEC on July 7, 2022 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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