November 10, 2023-

Notice of IND Clearance by U.S. FDA Enabling Phase 2 Initiation of KUS121 for the treatment of Central Retinal Artery Occlusion (CRAO)

Kyoto Drug Discovery and Development Co., Ltd., a clinical stage pharmaceutical company developing novel neuroprotective therapies, today announced that the U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application of KUS 121, a potential first-in-class treatment for Central Retinal Artery Occlusion (CRAO). CRAO is an ophthalmologic emergency that leads to severe and permanent vision loss for which no evidence-based therapies currently exist.
The GION study, the phase 2 clinical trial assessing the efficacy and safety of KUS121 in CRAO patients, will launch in the U.S. in early 2024.

▼About the GION study

This is a Phase II, double-masked, sham-controlled, multi-center, parallel-group study to evaluate the efficacy and safety of KUS121 intravitreal (IVT) injection in patients with non-arteritic Central Retinal Artery Occlusion (CRAO) diagnosed and treated within 3-48 hours of disease onset. Participants will be randomized to high dose KUS121, low dose KUS121, or sham in a 1:1:1 ratio. Over the course of three days, patients will receive a single daily intravitreal injection of KUS 121 or sham, which mimics an injection. The primary efficacy endpoint is the proportion of participants who gain 15 letters or more in best-corrected visual acuity (BCVA) from baseline and will be assessed at Week 12. The GION study will be conducted at multiple research facilities in the U.S. Details will be published on ClinicalTrials.gov shortly.

▼About KUS121

KUS121, an ATPase inhibitor of valosin-containing protein, is a novel neuroprotective agent that can control intracellular ATP levels and prevent cell death. KUS 121 is one of Kyoto University Substances (KUS) created by Kyoto University Researchers. KUS121 is currently being investigated to treat patients with Central Retinal Artery Occlusion (CRAO). CRAO is an ophthalmologic emergency that leads to severe and permanent vision loss for which no evidence-based therapies currently exist. The previously conducted Phase 1/2 investigator-initiated trial demonstrated that intravitreal injections of KUS121 are not only safe but also effective in improving vision of affected patients. The Phase 2 GION study (named after the famed Gion district in Kyoto) will launch in the U.S. in early 2024. KUS121 holds a promise to become a first-in-class treatment for CRAO. This program has received Orphan Drug Designation from the FDA.

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Kyoto Drug Discovery & Development

▼ABOUT KDDD (Kyoto Drug Discovery and Development Co., Ltd.)

KDDD is a clinical stage pharmaceutical company developing novel neuroprotective therapies discovered by Kyoto University Researchers. KDDD was founded to deliver innovative drugs that can save patients from the effects of intractable eye diseases, especially CRAO, Retinal Pigmentosa, Age-Related Macular Degeneration (AMD) and Glaucoma.
See KDDD website for more information.
https://www.kyoto-drug.com/en/

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