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KUS121 Receives Fast Track Designation from the U.S. FDA for Retinal Artery Occlusion

Kyoto Drug Discovery and Development Co., Ltd. (KDDD) today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to its investigational drug, KUS121, for the treatment of Retinal Artery Occlusion (RAO).

Fast Track is a designation intended to facilitate the development and expedite the review of drugs that treat serious or life-threatening conditions and address unmet medical needs.

This designation allows for more frequent interactions with the FDA, eligibility for Rolling Review*, and the potential for Priority Review, thus accelerating both development and regulatory approval.

KUS121 is currently being developed as a treatment for retinal artery occlusion. With the Fast Track Designation, KDDD aims to make this treatment available to patients more quickly. KUS121 was previously granted Orphan Drug Designation by the FDA in May 2022.

Currently, KUS121 is undergoing a Phase II clinical trial in the United States for the treatment of RAO. Preparations are also underway to initiate a Phase III trial in Japan.

Commenting on the designation, Dr. Musashi, CEO of KDDD, said:

“Retinal artery occlusion is a serious condition for which no established treatment currently exists. By suppressing ischemia-reperfusion injury, KUS121 has the potential to preserve retinal function in the acute phase of RAO. Our mission is to deliver meaningful treatment options to patients with high unmet medical needs, and we are committed to advancing the development of KUS121 to fulfill that goal.”

*Rolling Review

Under this system, a drug sponsor may submit portions of a New Drug Application (NDA) or Biologics License Application (BLA) as they are completed, rather than waiting until the entire application is ready for submission.

▼About KUS121

KUS121 (Kyoto University Substances) is a novel neuroprotective agent that inhibits the ATPase activity of valosin-containing protein (VCP), thereby regulating intracellular ATP levels and preventing cell death. Created by researchers at Kyoto University, KUS121 is currently being investigated as a potential treatment for Central Retinal Artery Occlusion (CRAO)—an ophthalmic emergency that causes sudden and often irreversible vision loss.

In a physician-led Phase I/II trial conducted in Japan, intravitreal administration of KUS121 was shown to be safe and potentially effective in improving vision. The Phase II “GION Study” (named after Kyoto’s historic district) was initiated in the U.S. in early 2024.

KUS121 is expected to become a first-in-class therapy for CRAO.

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

KDDD is a Kyoto University spin-out biotechnology company developing novel neuroprotective therapies, including KUS121. The company is focused on creating innovative treatments for severe ophthalmic diseases, especially retinal artery occlusion (RAO). KUS121 also holds promise in other therapeutic areas beyond ophthalmology.

For more information, please visit:

<https://www.kyoto-drug.com>

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