

Press Release

November 27, 2025

Designation of KUS121 as an Orphan Drug in Japan

Kyoto Drug Discovery & Development Co., Ltd. (KDDD) is pleased to announce that its investigational compound KUS121 has been designated as an Orphan Drug by the Ministry of Health, Labour and Welfare (MHLW) of Japan on November 27, 2025, for the treatment of Central Retinal Artery Occlusion (CRAO).

This designation provides various forms of governmental support, including development guidance and consultation, priority review, and financial incentives such as research grants and tax relief measures. Upon marketing approval, the product will also be eligible for an extended re-examination period of 10 years, offering further regulatory advantages.

CRAO is a vision-threatening condition caused by the occlusion of the central retinal artery, leading to severe visual impairment and visual field loss. KUS121 acts as an ATPase inhibitor of valosin-containing protein (VCP) and is expected to suppress intracellular ATP depletion, reduce endoplasmic reticulum stress, and prevent retinal cell death.

“The designation of KUS121 as an Orphan Drug by the MHLW represents a major milestone and a significant advantage in our mission to develop a treatment for CRAO. We intend to leverage this designation to accelerate development efforts in Japan.”

— Kunihiro Musashi, M.D., Ph.D., Chief Executive Officer, Kyoto Drug Discovery & Development Co., Ltd.

Notes:

(1) Orphan Drug: Under Article 77-2 of Japan’s Pharmaceuticals and Medical Devices Act, Orphan Drugs, Orphan Medical Devices, and Orphan Regenerative Medical Products are designated by the MHLW for diseases affecting fewer than 50,000 patients in Japan and for which there is a particularly high unmet medical need.

(2) KUS121: KUS121 is a novel compound discovered at Kyoto University that inhibits the ATPase activity of valosin-containing protein (VCP), a member of the AAA (ATPases Associated with diverse cellular Activities) family. VCP plays a key role in cellular stress responses to misfolded protein accumulation and oxidative stress and is ubiquitously expressed, including in retinal cells. KDDD is actively advancing the development of KUS121 as a first-in-class cytoprotective agent for diseases with no established therapies.

(3) Central Retinal Artery Occlusion (CRAO): CRAO is a rare ophthalmic disorder in which embolic or thrombotic occlusion of the central retinal artery blocks oxygen supply to retinal neurons, leading to irreversible vision loss due to retinal ganglion cell death. Currently, there is no proven effective therapy for CRAO.