

May 6, 2022-

KUS121 receives Orphan Drug Designation^{*1} from the US FDA

We are pleased to announce that we have received Orphan Drug Designation to KUS121^{*2} for the treatment of retinal artery occlusion (RAO)^{*3} from the US Food and Drug Administration (FDA) on May 3, 2022. The orphan drug designation enables us to receive benefits such as 7-year marketing exclusivity, tax credit for clinical trials costs and waiver of a partial marketing application user fees in the US.

RAO is a serious disease that causes visual impairment and visual field impairment due to occlusion of the retinal artery. As an inhibitor for the ATPase of VCP, KUS121 is expected to suppress intracellular ATP depletion, reduce endoplasmic reticulum stress, and prevent cell death of retinal cells.

Dr. Kunihiro Musashi, an ophthalmologist and our CEO, mentioned that being designated as an orphan drug granted by the FDA is a huge advantage in the development of RAO therapeutic agents. We would like to receive this support to accelerate the KUS121 development for the patients with RAO.

*1: Orphan Drug Designation:

Orphan Drug Designation (ODD) is the program for accelerate development of drugs including biologicals that has been developed specifically to treat a rare medical condition, the condition itself being referred to as an orphan disease. The condition of designation and incentives by the program is different from country to country and drugs would qualify for orphan status if they were intended to treat a disease affecting less than 200,000 American citizens in the US.

*2: KUS121:

A novel compound discovered at Kyoto University as an inhibitor of ATP hydrolase (ATPase) activity of valosin-containing protein (VCP). VCP is one of the ATPases belonging to the ATPase associated with diverse cellular activities (AAA) family, and plays an important role in the process of inducing a stress response to abnormal protein accumulation and oxidative stress when cells encounter them. It is expressed in all cells in the body, including the retina. We are enthusiastically developing KUS121 as a novel cytoprotectant with a new mechanism of action for diseases which have no therapeutic method.

*3: Retinal artery occlusion (RAO):

Retinal artery occlusion is a severe retinal disease that occurs when the retinal artery, which carries

oxygen to the nerve details in the retina, is embolized. When the oxygen supply to the retina is insufficient, visual impairment and visual impairment occur due to dysfunction of retinal ganglion cells that consume a large amount of oxygen, and the impairment persists due to cell death of retinal ganglion cells. RAO is classified into central retinal artery occlusion (CRAO) and branch retinal artery occlusion (BRAO) according to the site of occlusion of the retinal artery. Currently there is no effective treatment for this disease.