Choroideremia
RESEARCH FOUNDATION

April 4, 2024

Mr. Yasuhiro Fujiwara
Chairman of the Board of Directors
Pharmaceuticals and Medical Devices Agency

Request for Early Approval of Emixustat Hydrochloride for the Treatment of Stargardt's Disease

Dear PMDA,

We would like to express our sincere respect for your ongoing efforts for the health of the people of Japan. On behalf of the Choroideremia Research Foundation, a US-based, international nonprofit organization which supports research, education and advocacy for choroideremia (CHM) patients and family members, we respectfully request the PMDA's consideration of early approval of Emixustat hydrochloride, a new drug developed by Kubota Pharmaceutical Holdings Corporation for the treatment of Stargardt's disease.

Similar to CHM, Stargardt's disease causes progressive loss of vision, leading to blindness, which usually begins in childhood or adolescence. The financial burden over the lifetime of a patient is significant, impacting schooling, employment, daily life, and mental health.

Emixustat hydrochloride is a drug with potential for maintaining retinal health. It is expected to slow the progression of Stargardt's disease by suppressing RPE65, an enzyme essential for the visual cycle, and reducing toxic metabolites accumulated by genetic abnormalities involved in the development of Stargardt's disease. It is designed to be of benefit in Stargardt's disease with different mutations and is not limited to work in any specific genetic abnormality. Therefore, it may also be beneficial in other inherited retinal diseases, such as choroideremia and RDH12 retinal degeneration, as it has been shown to be neuroprotective in preclinical models. Emixustat has been demonstrated to be of clinical benefit in reducing the growth rate of smaller atrophic lesions in Stargardt's disease patients. Therefore, early approval is desperately needed because administration at a stage when the lesion area is small can be expected to maintain visual function for a longer time.

We sincerely hope that the Pharmaceuticals and Medical Devices Agency will consider Emixustat hydrochloride for early approval to provide a new treatment option for patients with this blinding disease. Approving this therapy for Stargardt's disease will encourage investigation into other retinal degeneration conditions where additional patient communities could benefit.

Sincerely,

Kathi Wagner
Executive Director