June 15, 2020

Start of a Phase 3 Clinical Trial of MT-7117 for the Ultra-Rare Disease, Erythropoietic Protoporphyria (EPP) and X-Linked Protoporphyria (XLP)

Mitsubishi Tanabe Pharma Corporation (MTPC, Head Office: Chuo-ku, Osaka; President & Representative Director, CEO: Hiroaki Ueno) announced today that its research and development subsidiary in U.S., Mitsubishi Tanabe Pharma Development America, Inc. (MTDA, President: Hideki Kuki), has started a global Phase 3 clinical trial of MT-7117 (dersimelagon – selective melanocortin 1 receptor (MC1R) agonist), an investigational oral treatment for the increased pain free light exposure in adult and adolescent patients with a history of phototoxicity (including severe pain on exposure to sunlight) from erythropoietic protoporphyria (EPP) [1]and X-Linked Protoporphyria (XLP).
MTPC received Fast Track Designation for dersimelagon by the U.S. Food and Drug Administration (FDA) in June 2018 and was granted Orphan Drug Designation by FDA on June 8th 2020[2].

The global phase 3 trial leveraged the experience and knowledge obtained from the successfully completed ENDEAVOR study. ENDEAVOR was a phase 2 proof of concept study for dersimelagon. Analysis of the primary endpoint showed a significant improvement in average daily time to first prodromal symptom associated with sunlight exposure between 1 hour post sunrise and 1 hour pre-sunset in dersimelagon subjects compared to placebo subjects for the 100 mg (53.8 min/day, $p<0.008$) and 300 mg (62.5 min/day, $p<0.003$) cohorts at Week 16. Dersimelagon was generally well tolerated with an acceptable safety profile. These results were presented in the late-breaking clinical session at the annual AAD 2020 conference.

MTPC believes that further development and potential regulatory approval of MT-7117, an oral treatment, could provide another option for healthcare providers treating EPP.

Mitsubishi Tanabe Pharma Group will continue to advance R&D activities so that we can deliver new therapy options that address the needs of patients around the world fighting serious diseases as quickly as possible.

[1]NIH website: https://rarediseases.info.nih.gov/diseases/4527/erythropoietic-protoporphyria
[2]https://www.accessdata.fda.gov/scripts/opdlisting/oopd/detailedIndex.cfm?cfgridkey=645518

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About Dersimelagon (MT-7117):

Dersimelagon is a novel synthetic, orally-administered, non-peptide small molecule, which acts as a selective agonist of melanocortin-1 receptor (MC1R) with a potential for being effective to increase pain free light exposure in patients with a history of phototoxicity from erythropoietic protoporphyria (EPP) and X-Linked Protoporphyria (XLP). Mitsubishi Tanabe Pharma Corporation (MTPC) is developing dersimelagon for the treatment of EPP or XLP.

Dersimelagon is an investigational medication and not approved by FDA or any other regulatory authority. Mitsubishi Tanabe Pharma Corporation received Fast Track Designation for dersimelagon by the U.S. Food and Drug Administration in June 2018.

About Erythropoietic Protoporphyria and X-Linked Protoporphyria

Erythropoietic Protoporphyria (EPP) is an inherited disorder of the heme biosynthetic pathway that results from mutations of the ferrochelatase (FECH) gene or, less commonly X-Linked Protoporphyria (XLP) that results from mutations in the aminolevulinic acid synthase-2 (ALAS2) gene. Both EPP and XLP are characterized by accumulation of protoporphyrin in blood, erythrocytes and tissues and cutaneous photosensitivity. EPP and XLP usually present early in childhood with extremely painful phototoxic reactions which are preceded by a “prodrome” of tingling, stinging, and/or burning of sun-exposed skin. The onset of prodromal symptoms after direct sun exposure varies but may occur in less than 10 minutes. Importantly, continued exposure to sunlight following the onset of prodromal symptoms will lead to phototoxicity-induced pain.

About Mitsubishi Tanabe Pharma Development America, Inc.

The U.S. headquarters of Mitsubishi Tanabe Pharma Development America, Inc. (MTDA) is located in Jersey City, New Jersey. MTDA is a wholly-owned subsidiary of Mitsubishi Tanabe Pharma Corporation’s 100 percent-owned U.S. holding company, Mitsubishi Tanabe Pharma Holdings America, Inc. MTDA has obtained the approval of Radicava® the new treatment option for ALS in more than 20 years in the ALS therapeutic area in the United States. MTDA is dedicated to research and develop innovative pharmaceutical products that address the unmet medical needs of patients.

https://mt-pharma-development-america.com/