



July 27, 2018

**Mitsubishi Tanabe Pharma's MT-7117 Receives
U.S. FDA Fast Track Designation
for the Investigational Treatment of Patients with the
Ultra-Rare Disease, Erythropoietic Protoporphyrin**

Mitsubishi Tanabe Pharma Corporation (MTPC) (Head Office: Osaka; President & Representative Director, CEO: Masayuki Mitsuka), announces that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for its investigational drug coded MT-7117 for the prevention of phototoxicity in patients with erythropoietic protoporphyria (EPP¹). Mitsubishi Tanabe Pharma Development America, Inc. (MTDA) and MTPC Group companies have initiated the enrollment of the Phase 2 "ENDEAVOR" trial for MT-7117 in the United States.

"The FDA has established significant challenging criteria in order to meet the standard required for the Fast Track designation². This designation allows the MT-7117 Development Team to have increased dialogue with the FDA regarding the clinical program at key points in time, which could lead to a priority review of the New Drug Application depending on the outcome of our clinical trials," said Douglas N. Dobak, Vice President and Head of Regulatory Affairs, MTDA.

Erythropoietic Protophyria (EPP) is an inherited disorder of the heme metabolic pathway that results from mutations of the ferrochelatase (FECH) gene or, less commonly, the aminolevulinic acid synthase-2 (ALAS2) gene. EPP is characterized by accumulation of protoporphyrin in blood, erythrocytes and tissues and cutaneous photosensitivity that usually manifests in early infancy during initial exposure to sun. Photosensitivity is the main symptom of EPP and exposure of skin to sunlight results in acute cutaneous symptoms such as stinging, burning and severe pain leading to the development of erythema, cutaneous edema and petechiae with prolonged exposure to sunlight. Photosensitivity has a significant deleterious impact on the quality of life of EPP patients, leading to chronic avoidance of both long-wave radiation and visible light and a resultant profound decrease of social activity. EPP is a lifelong disorder whose prognosis depends on the evolution of the hepatic disease; in addition, approximately 3 percent of all patients with EPP develop liver pathology. MTDA has started the "ENDEAVOR" trial in the United State. The ENDEAVOR trial is a Phase 2, multicenter, randomized, double-blind, placebo-controlled study to

evaluate efficacy, safety, and tolerability of MT-7117 in subjects with EPP; The study will enroll approximately 100 subjects and consists of a 2-week screening period, a 16-week double-blind treatment period, and a 6-week follow up period.

MTPC Group will work hard to ensure that our research will substantially contribute to delivering the best possible pharmaceutical products as quickly as possible to many patients fighting against severe diseases all over the world.

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1. NIH website: <https://rarediseases.info.nih.gov/diseases/4527/erythropoietic-protoporphyrria>.
2. US Food and Drug Administration. Fast Track Information Page:
<https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm>

■ **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. <http://mt-pharma-development-america.com/>