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Mitsubishi Tanabe Pharma Corporation Announces Results of the MT-7117 ENDEAVOR Study for the Ultra-Rare Disease, Erythropoietic Protoporphyria (EPP)

Mitsubishi Tanabe Pharma Corporation (MTPC) announces successful completion of the ENDEAVOR study for MT-7117 (dersimelagon – selective MC1R agonist), an investigational oral treatment under development by Mitsubishi Tanabe Pharma Development America, Inc. (MTDA) for the prevention of phototoxicity (including severe pain on exposure to sunlight) in patients with erythropoietic protoporphyria (EPP).[1] ENDEAVOR is a phase 2 proof of concept study for MT-7117 as a treatment option for EPP. ENDEAVOR met its primary endpoint of change from baseline in average daily time (minutes) to first prodromal symptom associated with sunlight exposure between 1 hour post sunrise and 1 hour pre-sunset at Week 16. MT-7117 was generally well tolerated with an acceptable safety profile.

The Company plans to present the topline results characterizing the primary analysis of safety and efficacy at a scientific congress in early 2020.

"*The results of ENDEAVOR are very encouraging and will pave the way for a pivotal trial to evaluate the safety, efficacy and effectiveness of MT-7117 as an oral once a day treatment option for EPP" said Robert Desnick, MD PhD, Dean for Genetic and Genomic Medicine, Icahn School of Medicine at Mount Sinai, NY and Lead Investigator on the study. "If confirmed and approved by regulatory agencies, this could be the first oral treatment option for EPP and we believe could be a clinically meaningful alternative for patients."*

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 in the prevention of phototoxicity in erythropoietic protoporphyria (EPP) patients. Mitsubishi Tanabe Pharma Corporation (MTPC) is developing dersimelagon for the treatment of EPP.

Dersimelagon is an investigational medication and not approved by FDA or any other global 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

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. The primary objective of the ENDEAVOR study was to demonstrate a treatment-related increase of the time to occurrence of the "prodrome"; thus avoiding the onset of phototoxicity induced pain, which can be incapacitating for several days. Photosensitivity and subsequent pain episodes have a significant deleterious impact on the quality of life of EPP and XLP patients, leading to chronic avoidance of both long-wave radiation and visible light and a resultant profound decrease of work opportunities and daily and social activities.[2] EPP is a lifelong disorder and complications in some patients may include hepatic disease, of which, approximately 2-5% develop liver failure requiring transplantation.[2,3]

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/

Overview of Mitsubishi Tanabe Pharma Corporation

Mitsubishi Tanabe Pharma, which was founded in 1678, has its headquarters in Doshomachi, Osaka, which is the birthplace of Japan's pharmaceutical industry. With business centered on ethical pharmaceuticals, Mitsubishi Tanabe Pharma is a well-established company and has the longest history of any listed pharmaceutical company in Japan.[4] In accordance with the corporate philosophy of "contributing to the healthier lives of people around the world through the creation of pharmaceuticals," the Company formulated the key concept of Open Up the Future under the Medium-Term Management Plan 16-20. Through the discovery of drugs that address unmet medical needs, centered on its priority disease areas — immuno-inflammation, diabetes and kidney, central nervous system, and vaccines — Mitsubishi Tanabe Pharma will strive to contribute to the health of patients around the world.

For more information, go to https://www.mt-pharma.co.jp/e/

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References

1. NIH website: https://rarediseases.info.nih.gov/diseases/4527/erythropoietic-protoporphyria
2. Todd DJ. Erythropoietic Protoporphyria. Br J Dermatol 1994; 131: 751-66.
3. Balwani et al. Clinical, Biochemical, and Genetic Characterization of North American Patients With Erythropoietic Protoporphyria and X-linked Protoporphyria JAMA dermatology 2017: 153 (8) 789-796
4. Research by TOKYO SHOKO RESEARCH, LTD.: https://www.mt-pharma.co.jp/e/

Forward-looking Statements

This press release includes certain disclosures that contain “forward-looking statements,” including, without limitation, statements regarding progress, timing, scope and results of clinical trials, the timing of clinical data, and the FDA Fast Track process leading to faster drug approval and patient access. You can identify forward-looking statements because they contain words such as “will,” “believes” and “expects.” Forward-looking statements are based on current expectations and assumptions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that may differ materially from those contemplated by the forward-looking statements, which are neither statements of historical fact nor guarantees or assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements are set forth in regulatory filings with the Securities and Exchange Commission,

MTPC and MTDA assume no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.