March 31, 2021

Phase -2 Clinical Trial of MT-7117 Initiated in the U.S., Canada, and Europe for the Rare Disease, Diffuse Cutaneous Systemic Sclerosis (dcSSc)

Mitsubishi Tanabe Pharma Corporation (MTPC; Head Office: Chuo-ku, Osaka, Japan; President & Representative Director, CEO: Hiroaki Ueno) announced today that its research and development subsidiary in the US, Mitsubishi Tanabe Pharma Development America, Inc. (MTDA; President: Hideki Kuki), has initiated enrollment of Phase 2 clinical trial "DECODE" of MT-7117 (dersimelagon), a selective melanocortin 1 receptor (MC1R) agonist being studied as an investigational oral therapy for the treatment of diffuse cutaneous systemic sclerosis (dcSSc)[1] in the US, Canada, and Europe.

MTPC was granted Fast Track Designation[2] for MT-7117 for the treatment of dcSSc in April 2020 by the US Food and Drug Administration (FDA).

Systemic sclerosis (SSc, scleroderma) is a rare, chronic, and systemic disease characterized by autoimmunity, vasculopathy, and fibrosis of the skin and internal organs (e.g., heart, lungs, gastrointestinal tract, blood vessels, and kidneys). Diffuse cutaneous SSc, one of the two clinical subsets of SSc, is characterized by skin thickening (fibrosis) and is associated with severe organ damage, which accounts for the high observed morbidity and mortality.[3] Current disease management is focused on mitigating organ-specific complications.

The DECODE trial is a Phase 2, multicenter, randomized, double-blind, placebo-controlled, parallel-group study to evaluate the efficacy, safety, and tolerability of MT-7117 in subjects with dcSSc. The study will enroll approximately 72 patients and consists of a screening period, 52-week double-blind treatment period, and safety follow-up period after the last dose.

MTDA has also been conducting a global Phase 3 clinical trial of MT-7117 to evaluate efficacy, safety, and tolerability of MT-7117, an investigational oral treatment, in adult and adolescent patients with a history of phototoxicity (including severe pain on exposure to sunlight) from erythropoietic protoporphyria (EPP) and X-Linked Protoporphyria (XLP).

Mitsubishi Tanabe Pharma Group will continue to advance research and development activities to deliver new treatment options addressing the needs of patients fighting serious diseases around the world.

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About Dersimelagon (MT-7117)
Dersimelagon is a synthetic, orally-administered, non-peptide small molecule being developed for multiple indications that acts as a selective agonist of MC1R.
Dersimelagon is an investigational drug and is not approved by the FDA or any other regulatory authority. MTPC received Fast Track Designation for dersimelagon for the treatment of dcSSc by the FDA in April 2020.

About Diffuse Cutaneous Systemic Sclerosis
Diffuse cutaneous SSc is a subtype of systemic sclerosis (scleroderma) characterized by skin thickening (fibrosis) and associated with severe organ damage. The disease can occur at any age but mainly affects people between 40 and 50 years of age.[1] SSc is a complex, polygenetic, autoimmune, and chronic medical condition characterized by excessive production of collagen (fibrosis) and microvascular damage in the skin, joints, lungs, esophagus, gastrointestinal tract, kidneys, heart, and other internal organs. SSc has an estimated prevalence in the US of 276 to 300 cases per million and an incidence of about 20 cases per million per year.

About Mitsubishi Tanabe Pharma Corporation (MTPC)
Mitsubishi Tanabe Pharma Corporation (MTPC) was founded in 1678 and has its headquarters in Doshomachi, Osaka, Japan, which is the birthplace of Japan's pharmaceutical industry. With business centered on ethical pharmaceuticals, MTPC is a well-established company and has the longest history of any listed company in Japan[4]. Through the discovery of vaccines and drugs that address unmet medical needs, centered on its priority disease areas — autoimmune diseases and central nervous system diseases —MTPC strives to contribute to the health of patients around the world. MTPC is the parent company of MTDA.
For more information, go to https://www.mt-pharma.co.jp/e/

About Mitsubishi Tanabe Pharma Development America, Inc.
The headquarter of Mitsubishi Tanabe Pharma Development America, Inc. (MTDA) is located in Jersey City, New Jersey. MTDA is a wholly-owned subsidiary of MTPC's 100 percent-owned US holding company, Mitsubishi Tanabe Pharma Holdings America, Inc. MTDA is dedicated to researching and developing innovative pharmaceutical products that address the unmet medical needs of patients.
For more information, go to https://mt-pharma-development-america.com/

Reference
[1] NIH website: https://rarediseases.info.nih.gov/diseases/9751/diffuse-cutaneous-systemic-sclerosis
[2] US Food and Drug Administration. Fast Track Information Page: https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track
[3] Denton CP, Khanna D. Systemic sclerosis. Lancet 2017; 390: 1685–99.
[4] Research by TOKYO SHOKO RESEARCH, LTD.