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Phase 2 Trial of MT-3921 Initiated for Treatment of Spinal Cord Injury

Mitsubishi Tanabe Pharma Corporation (MTPC, Head Office: Chuo-ku, Osaka; President & Representative Director, CEO: Hiroaki Ueno) and Professor Toshihide Yamashita at the Department of Molecular Neuroscience and Department of Neuro-Medical Science, Osaka University Graduate School of Medicine, announced today that MTPC's research and development subsidiary in the US, Mitsubishi Tanabe Pharma Development America, Inc. (MTDA) has initiated enrollment of a Phase 2 clinical trial of jointly developed MT-3921 for the treatment of spinal cord injury.

With no effective therapy available, spinal cord injury is a primary disease target for regenerative medicines. MT-3921 is a novel humanized immunoglobulin G1 (IgG1) monoclonal antibody that binds to repulsive guidance molecule A (RGMA) for the treatment of spinal cord injury. It is a potentially significant addition to the treatment of neurological disorders, one of the focus areas of MTPC's development pipeline. MTDA was granted Fast Track Designation¹ for MT-3921 for the treatment of spinal cord injury in July 2021 by the U.S. Food and Drug Administration (FDA).

MTDA has initiated a Phase 2 Proof of Concept (POC) study (NCT04683848) to enroll 72 patients with spinal cord injury in the US, Canada, and Japan. It is a multicenter, randomized, double-blind, placebo-controlled, parallel-group study to evaluate the safety, efficacy, and tolerability of MT-3921. The primary endpoint of the study is a change in Upper Extremity Motor Score (UEMS) after 6 months of treatment. Professor Yamashita's research team supports the Phase 2 study with preclinical research and scientific communication with the study sites.

Mitsubishi Tanabe Pharma Group and Osaka University Graduate School of Medicine are committed to advance research and development activities to satisfy unmet medical needs and to deliver innovative treatments to patients.

¹ US Food and Drug Administration. Fast Track Information Page:
<https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm>

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About MT-3921

Based on the results of fundamental research by Professor Yamashita's research team, MT-3921 is a humanized anti-RGMA antibody jointly developed by MTPC and Osaka University since 2005. Current research concluded that RGMA is associated with inhibition of neuronal survival and neuroregeneration, involved in the progression of inflammation, and may play a role in neurological diseases including spinal cord injury, stroke, and multiple sclerosis. The data from the preclinical animal studies conducted by Professor Yamashita's research team and MTPC indicate that treatment with MT-3921 improves locomotor function and promotes neuroregeneration.

Pre-clinical studies conducted by Professor Yamashita's team were supported by a grant from the Japan Agency for Medical Research and Development (AMED).

About Spinal Cord Injury

Spinal cord injury (SCI) typically occurs due to a traumatic, sudden onset of external force to the spinal cord, resulting in neurological impairment or loss of motor, sensory, autonomic, and/or reflex functions below the neurological level of injury. SCI is a serious medical condition due to automobile accidents, falls, sports-related accidents, violence, and other causes. The injuries vary in severity and can cause motor paralysis, loss of sensation, and disturbance of bladder and rectal function. Spinal cord injuries affect the health of the patients and place a major burden on the patients' families and caregivers. Epidemiological studies estimate there are approximately 18,000 acute SCI incidents in the United States annually. There is no FDA-approved treatment for acute spinal cord injury. The current treatment of spinal cord injury mainly focuses on surgical spine stabilization, intensive neurological rehabilitation, and prevention and treatment of acute and chronic complications. No effective treatment for traumatic paraplegia or quadriplegia is available to date.

About Mitsubishi Tanabe Pharma Corporation (MTPC)

Mitsubishi Tanabe Pharma Corporation (MTPC), founded in 1678, is one of the oldest pharmaceutical companies in the world. Focused on ethical pharmaceuticals, MTPC is headquartered in Doshomachi, Osaka, the birthplace of Japan's pharmaceutical industry. In light of the anticipated changes in healthcare needs in the future, MTPC is advancing business activities under MISSION of "Creating hope for all facing illness," and has set the corporate vision for 2030 (VISION 30) to "Be a healthcare company that delivers optimal therapy to each individual." As part of the vision for 2030, MTPC is prioritizing work on "precision medicine" to create effective therapies and preventive methods by identifying patient populations with high potential for efficacy and safety, focusing on the disease areas of central nervous system and immuno-inflammation. In addition, MTPC is working to develop "around the pill solutions" to address specific patient concerns based on therapeutic medicine, including prevention of diseases, pre-symptomatic disease care, prevention of aggravation and prognosis. MTPC is a member of Mitsubishi Chemical Holdings Group.

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

About Mitsubishi Tanabe Pharma Development America, Inc. (MTDA)

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 is dedicated to the research and development of innovative pharmaceutical products, satisfying unmet medical needs.

For more information, please go to <https://mt-pharma-development-america.com/>

About Osaka University

Originating from Kaitokudo and Tekijuku, centuries-old institutions established during Japan's Edo period, Osaka University was founded in 1931 as the sixth imperial university of Japan to satisfy the strong demand from the people, the businesses, and the government in Osaka. With 11 faculties, 16 graduate schools, and 6 research institutes, Osaka University continues to develop as a leading comprehensive research university. With its motto "Orchestration and co-creation of knowledge", Osaka University conducts creative activities in cooperation with society, driving towards its goal of becoming a "World-Leading Innovative University Contributing to Social Changes" by its 100th anniversary in 2031.

For more information, please go to <https://www.osaka-u.ac.jp/en/>