December 10, 2021

# Global Phase 3 Safety Study Results of Investigational Oral Edaravone MT-1186 in the treatment of ALS

Mitsubishi Tanabe Pharma Corporation (MTPC, Head Office: Chuo-ku, Osaka; President & Representative Director: Hiroaki Ueno) announced 24-week results from the global Phase 3 study evaluating the safety and tolerability of investigational oral edaravone in the treatment of amyotrophic lateral sclerosis (ALS). Findings from the Phase 3 study, which is conducted by Mitsubishi Tanabe Pharma Development America, Inc. (MTDA), were presented during the Motor Neurone Disease Association (MNDA) virtual 32nd International Symposium on ALS/MND (December 7-10).

The 24-week treatment results showed important data on the safety and tolerability for investigational oral edaravone, as follows.

【Summary of Studies】
The global multi-center, open-label study (MT-1186-A01), enrolled 185 ALS patients (aged ≥18 years to 75 years) across 50 sites in the U.S., Canada, Europe and Japan. The primary objective is to evaluate the 24 and 48 weeks safety and tolerability of oral edaravone in patients with ALS.

【Results】
The 24-week results presented at MNDA include findings from a total of 185 subjects included in the Safety Analysis Set who received at least one dose of study drug. Treatment emergent adverse events (TEAEs) reported by ≥5% of subjects were muscular weakness (16.2%), fall (15.7%), fatigue (7.6%), back pain (7.0%), constipation (7.0%), headache (5.9%) and dyspnea (5.4%). No serious TEAEs considered to be treatment-related by investigators were reported.
Eleven subjects (5.9%) discontinued the study due to TEAEs, two of them related to the study drug. The most common TEAEs were respiratory failure and muscular weakness, consistent with the disease state. There were six deaths during the 24-week study period, and none of the deaths were related to the study drug (respiratory failure (3), ALS (1), pneumonia (1) and suicide (1)).

In addition, the loss of physical function was evaluated as an Exploratory Endpoint and measured by the ALS Functional Rating Scale–Revised (ALSFRS-R), a validated rating instrument for monitoring the progression of disability in patients with ALS. The changes from baseline to all post-baseline visits until Week 24 in ALSFRS-R score were estimated using a mixed model for repeated measures (MMRM) analysis. At the beginning of the study, patients had an average ALSFRS-R score of 40 (SD 4.5). At Week 24, the average change from baseline in ALSFRS-R score was −5.6 (95% CI −6.5, −4.8).

MTPC Group is working with the regulatory authorities of each country to promptly obtain approval of MT-1186 based on the results of this study.
MTPC Group will continue to make untiring efforts to promptly provide new treatment options for ALS patients.

**Mitsubishi Tanabe Pharma Corporation**
**Communication Crossroads Department**
Media contacts: TEL: +81 6 6205 5119



<Reference>

**About Oral Edaravone (MT-1186)**

MT-1186 is an investigational oral suspension of edaravone being studied in patients with amyotrophic lateral sclerosis (ALS) by Mitsubishi Tanabe Pharma Development America, Inc., a subsidiary of Mitsubishi Tanabe Pharma Corporation (MTPC). MT-1186 received Fast Track designation from the FDA in October 2019. The ongoing MT-1186 A03 study is an extension study of up to 96 weeks of treatment in patients who have completed MT-1186 A01. MT-1186-A01 and MT-1186 A03 studies provide data for up to 144 weeks.

**About Edaravone**

Edaravone is a free radical scavenger discovered by MTPC. It was approved by the Ministry of Health, Labour and Welfare in April 2001 for the treatment of patients with acute cerebral infarction and is marketed in Japan under the product name of Radicut®. The indication of ALS has been approved in 8 countries including Japan

in June, South Korea in December 2015, the United States in May 2017, Canada in October 2018, and Switzerland in January 2019.

**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 achieved the approval of Radicava for the first time in more than 20 years in the U.S in the ALS treatment. 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/