Mitsubishi Tanabe Pharma America Press Release

MITSUBISHI TANABE PHARMA AMERICA TO PRESENT ON ALS CLINICAL PROGRAMS AT 2019 MUSCLE STUDY GROUP ANNUAL SCIENTIFIC MEETING

Company to Share Details on Collaboration with ALS Patients & Caregivers to Inform Clinical Study Design

JERSEY CITY, N.J., September 20, 2019 – Mitsubishi Tanabe Pharma America, Inc. (MTPA) today announced three presentations on amyotrophic lateral sclerosis (ALS) at the 2019 Muscle Study Group Annual Scientific Meeting, including a podium presentation highlighting collaborative work with a group of ALS patients and caregivers to help inform the design of the REFINE-ALS biomarker study. The meeting will be held September 20 – 22 in Snowbird, Utah.

MTPA will present details about the approach taken to seek study input from the ALS Research Ambassadors, a group of patients and caregivers with specialized training from the ALS Clinical Research and Learning Institute.

"Our collaboration with the ALS Research Ambassadors was instrumental to ensuring the REFINE-ALS study is truly patient-centered," said Stephen Apple, M.D., Senior Medical Director, Medical Affairs, MTPA. "The substantive feedback we received from ALS patients and their caregivers during the study design process allowed us to modify the study protocol to enhance patient convenience while maintaining scientific rigor."

In addition, MTPA will present a poster on the use of a novel machine-learning risk-based analysis tool to determine if results from the phase 3 studies of edaravone may be generalizable to a broader patient population.

A second poster presentation will highlight an analysis of 10 clinical studies which indicated that certain ALS clinical trial inclusion criteria may influence study participants' baseline characteristics.

Presentation details:

Friday, September 20 from 7:30 PM – 9:00 PM MDT

- Evidence for Generalizability of Edaravone Efficacy Using a Novel Machine-Learning (ML) Risk-Based Analysis Tool *(Poster presentation)*

- The Influence of Clinical Study Inclusion Criteria on Baseline Characteristics and Disease Progression in Amyotrophic Lateral Sclerosis *(Poster presentation)*

Sunday, September 22 from 8:45 AM – 8:55 AM MDT

- Engaging ALS Research Ambassadors to Help Design the REFINE-ALS Biomarker Study *(Platform presentation)*

About REFINE-ALS
REFINE-ALS is a trial designed to identify and measure specific biomarkers in up to 300 people with ALS who are starting treatment with RADICAVA® (edaravone). The study is sponsored by Mitsubishi Tanabe Pharma America, Inc. and led by Massachusetts General Hospital Neurological Clinical Research Institute. The REFINE-ALS study includes approximately 40 sites across the country and will utilize the expertise of multiple specialty laboratories to assess biomarker samples. The trial includes assessments of biomarkers for oxidative stress, inflammation, neuronal and muscle injury. Biomarkers will be measured prior to initiating treatment with RADICAVA, at the start of treatment, and at pre-specified time points throughout the 24-week study period (six cycles of treatment).

About Mitsubishi Tanabe Pharma America, Inc.
Based in Jersey City, N.J., Mitsubishi Tanabe Pharma America, Inc. (MTPA) is a wholly-owned subsidiary of Mitsubishi Tanabe Pharma Corporation's (MTPC) 100 percent owned U.S. holding company, Mitsubishi Tanabe Pharma Holdings America, Inc. MTPA is dedicated to delivering innovative products that address the unmet medical needs of patients in North America. It was established by MTPC to commercialize approved pharmaceutical products in North America with plans to expand its product line through collaborations with partners. For more information, please visit www.mt-pharma-america.com or follow us on Twitter and Facebook.

Overview of Mitsubishi Tanabe Pharma Corporation (MTPC)
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 company in Japan.[1] 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 2016-2020. Through the discovery of drugs that address unmet medical needs, centered on its priority disease areas — autoimmune diseases, diabetes and kidney diseases, central nervous system diseases, and vaccines — Mitsubishi Tanabe Pharma will strive to contribute to the health of patients around the world. MTPC is the parent company of MTPA and the license holder of RADICAVA. For more information, go to http://www.mt-pharma.co.jp/.

About RADICAVA® (edaravone)
The U.S. Food and Drug Administration approved RADICAVA® (edaravone) on May 5, 2017, as a treatment for amyotrophic lateral sclerosis (ALS).[2] In a pivotal trial, people given RADICAVA experienced a 33 percent slower rate of decline in the loss of physical function, compared to placebo

as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R), a validated rating instrument for monitoring the progression of disability in people with ALS.[3,4]

Edaravone was discovered and developed for ALS by Mitsubishi Tanabe Pharma Corporation (MTPC) and commercialized in the U.S. by Mitsubishi Tanabe Pharma America, Inc. MTPC group companies began researching ALS in 2001 through an iterative clinical platform over a 13-year period. In 2015, edaravone was approved for use as a treatment for ALS in Japan and South Korea. Marketing authorization was granted in Canada in October 2018 and Switzerland in January 2019.

IMPORTANT SAFETY INFORMATION

Before you receive RADICAVA, tell your healthcare provider about all of your medical conditions, including if you:

- have asthma.
- are allergic to other medicines.
- are pregnant or plan to become pregnant. It is not known if RADICAVA will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if RADICAVA passes into your breast milk. You and your healthcare provider should decide if you will receive RADICAVA or breastfeed.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

What are the possible side effects of RADICAVA?

- RADICAVA may cause serious side effects including hypersensitivity (allergic) reactions and sulfite allergic reactions.

- Hypersensitivity reactions have happened in people receiving RADICAVA and can happen after your infusion is finished.

- RADICAVA contains sodium bisulfite, a sulfite that may cause a type of allergic reaction that can be serious and life-threatening. Sodium bisulfite can also cause less severe asthma episodes in certain people. Sulfite sensitivity can happen more often in people who have asthma than in people who do not have asthma.

- Tell your healthcare provider right away or go to the nearest emergency room if you have any of the following symptoms: hives; swelling of the lips, tongue, or face; fainting; breathing problems; wheezing; trouble swallowing; dizziness; itching; or an asthma attack (in people with asthma).

- Your healthcare provider will monitor you during treatment to watch for signs and symptoms of all the serious side effects.

The most common side effects of RADICAVA include bruising (contusion), problems walking (gait disturbance), and headache.

These are not all the possible side effects of RADICAVA. Call your healthcare provider for medical advice about side effects. You may report side effects to Mitsubishi Tanabe Pharma America, Inc. at 1-888-292-0058 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

For more information, including full Prescribing Information and Patient Information, please visit **www.RADICAVA.com**.

Media inquiries:
Mitsubishi Tanabe Pharma America
Debbie Etchison
908-340-8578
Media_MTPA@mt-pharma-us.com

---

[1] Research by TOKYO SHOKO RESEARCH, LTD.

[2] RADICAVA® U.S. Prescribing Information. August 2018.

[3] Simon, N. G., Turner, M. R., Vucic, S., Al-Chalabi, A., Shefner, J., Lomen-Hoerth, C., & Kiernan, M. C. (2014). Quantifying Disease Progression in Amyotrophic Lateral Sclerosis. *Annals of Neurology*, 76(5), 643-657. http://dx.doi.org/10.1002/ana.24273.

[4] The Writing Group on behalf of the Edaravone (MCI-186) ALS 19 Study Group (2017). Safety and efficacy of edaravone in well defined patients with amyotrophic lateral sclerosis: a randomised, double-blind, placebo-controlled trial. *Lancet Neurology*. 16(7), 505-512.