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**MT Pharma America Announces FDA Approval of RADICAVA™ (Edaravone),  
the First New Treatment Option for ALS in More Than 20 Years**

- First FDA-approved ALS treatment option in more than 20 years<sup>1</sup>
- RADICAVA has been demonstrated to slow decline of physical function by 33 percent<sup>2</sup>
- Comprehensive clinical development program in ALS conducted over a 13-year period<sup>2</sup>

**JERSEY CITY, N.J., May 5, 2017** – MT Pharma America, Inc. today announced the U.S. Food and Drug Administration (FDA) has granted approval of RADICAVA™ (edaravone), as an intravenous infusion treatment for amyotrophic lateral sclerosis (ALS), a rapidly progressive neurodegenerative disease in which the majority of patients die within two to five years of diagnosis.<sup>3,4</sup> People given RADICAVA showed significantly less decline in physical function compared to placebo as measured by the ALS Functional Rating Scale-Revised (ALSFRRS-R), a validated rating instrument for monitoring the progression of disability in patients with ALS.<sup>2,5</sup>

To view the multimedia assets associated with this release, please click:

<https://www.multivu.com/players/English/8047051-mt-pharma-america-radicava-fda-approval/>

“We believe RADICAVA offers new hope for people with ALS and exemplifies MT Pharma America’s commitment to innovative therapies for patients in the United States battling life-threatening diseases,” said Atsushi Fujimoto, President, MT Pharma America. “We recognize how important this therapy may be to people with ALS and are committed to helping provide access to this important treatment option, with the goal of keeping out-of-pocket costs at a minimum for eligible patients.”

An estimated 5,000-6,000 Americans are diagnosed each year with ALS, an incurable disease that affects the nerve cells in the brain and spinal cord.<sup>3,6,7</sup> Initial symptoms can be subtle at first, and it can take 12 to 14 months to be accurately diagnosed with ALS.<sup>8,9</sup>

“For people with ALS and their families, having a new therapy which slows the decline of physical ability is incredibly significant,” said Jonathan S. Katz, M.D., ALS Clinic Director, Forbes Norris MDA/ALS Research and Treatment Center at California Pacific Medical Center. “The approval of RADICAVA brings us into a new era of treatment by evolving how we manage this complex disease. This is an uplifting milestone for the ALS community especially since it’s been so long since we had anything new.”

The comprehensive clinical development program for RADICAVA in ALS spanned 13 years and included multiple Phase 3 trials. The pivotal Phase 3 study (MCI186-19), which evaluated 137 people with ALS, formed the basis for the FDA approval of RADICAVA. Data demonstrated patients who received RADICAVA for six months experienced significantly less decline in physical function — by 33 percent or 2.49 ALSFRRS-R points ( $p=0.0013$ ).<sup>2</sup>

The most common adverse reactions that occurred in greater than 10 percent of patients and greater than placebo were bruising (contusion), problems walking (gait disturbance) and

headache.<sup>2</sup>

MT Pharma America is helping to make RADICAVA accessible to all patients who need it and has created Searchlight Support, a patient access program for people with ALS who are prescribed RADICAVA. As soon as a person receives a prescription, he or she can access Searchlight Support, including personal case management, reimbursement support and 24/7 clinical support. Some people with ALS who have received prescriptions for RADICAVA may be eligible to receive additional assistance from MT Pharma America. For more information on Searchlight Support, contact 844-SRCHLGT (844-772-4548).

“This is an important time for people living with ALS. The approval of RADICAVA gives great promise for what we hope will be the first of many new treatments,” said Barbara Newhouse, President and CEO, ALS Association. “We applaud the work MT Pharma America and the FDA are doing as they have taken unprecedented steps to get this treatment into the hands of patients as quickly as possible.”

### **About the MCI186-19 Study**

Study MCI186-19 was a pivotal Phase 3 study that evaluated the efficacy and safety of RADICAVA compared with placebo in 137 people with ALS. In the study, after a 12-week pre-observation period, eligible patients were randomized 1:1 to receive RADICAVA 60 mg intravenously for 60 minutes or placebo during a six-month double-blind placebo-controlled phase. The primary endpoint for the study was change in the ALS Functional Rating Scale-Revised (ALSFRS-R) score from baseline to six months.<sup>2</sup>

### **About RADICAVA™ (edaravone)**

RADICAVA is administered in 28-day cycles by intravenous infusion. It takes 60 minutes to receive each 60 mg dose. For the initial cycle, the treatment is infused daily for 14 consecutive days, followed by a two-week drug-free period. All cycles thereafter are infused daily for 10 days within a 14-day period, followed by a two-week drug-free period.<sup>2</sup>

Edaravone was discovered and developed for ALS by Mitsubishi Tanabe Pharma Corporation (MTPC) and will be commercialized in the U.S. by MT Pharma America. MTPC group companies began researching ALS in 2001 through a comprehensive clinical platform over a 13-year period. In 2015, edaravone was approved for use as a treatment for ALS in Japan and South Korea.

## **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 breastmilk. 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 MT Pharma America, Inc. at 1-888-292-0058 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

For more information, including full Prescribing Information and Patient Information, please visit [www.RADICAVA.com](http://www.RADICAVA.com).

### **About MT Pharma America**

Based in Jersey City, N.J., MT Pharma America 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 the U.S. It was established by MTPC to commercialize approved pharmaceutical products in the U.S. with plans to expand its product line through collaborations with partners. For more information, please visit [www.mt-pharma-america.com](http://www.mt-pharma-america.com) or follow us on Twitter at <https://twitter.com/MTPharmaUS>.

## 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 company in Japan.<sup>10</sup> 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 — 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/>.

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<sup>1</sup> National Institute of Neurological Disorders and Stroke. Amyotrophic Lateral Sclerosis (ALS) Fact Sheet.

<https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Amyotrophic-Lateral-Sclerosis-ALS-Fact-Sheet>. Accessed April 17, 2017.

<sup>2</sup> RADICAVA™ U.S. Prescribing Information. May 2017.

<sup>3</sup> National Institute of Neurological Disorders and Stroke. Amyotrophic Lateral Sclerosis (ALS) Information Page.

<https://www.ninds.nih.gov/disorders/all-disorders/amyotrophic-lateral-sclerosis-als-information-page>. Accessed April 17, 2017.

<sup>4</sup> Mehta P, Kaye W, Bryan L, et al. (2016). Prevalence of Amyotrophic Lateral Sclerosis — United States, 2012–2013. *MMWR Surveill Summ*; 65(No. SS-8):1–12.

<sup>5</sup> 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.

<sup>6</sup> Marin B, Boumediene F, Logroscino G, et al. (2016). Variation in worldwide incidence of amyotrophic lateral sclerosis: a meta-analysis. *Int J Epidemiol*, 00:1-18.

<sup>7</sup> ALS Association. Quick Facts about ALS. <http://www.alsa.org/news/media/quick-facts.html>. Accessed April 17, 2017.

<sup>8</sup> ALS Therapy Development Institute. What is ALS. <http://www.als.net/what-is-als/>. Accessed April 17, 2017.

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<sup>9</sup> Brooks BR. (2000). Risk factors in the early diagnosis of ALS: North American epidemiological studies. *Amyotrophic Lateral Sclerosis and Other Motor Neuron Disorders*, 1:sup1, S19-S26.

<sup>10</sup> Research by TOKYO SHOKO RESEARCH, LTD.