Radicava ORS® (edaravone) – New orphan drug approval

- On May 12, 2022, the FDA announced the approval of Mitsubishi Tanabe Pharma's Radicava ORS (edaravone) oral suspension, for the treatment of amyotrophic lateral sclerosis (ALS).

- ALS is a rare disease that attacks and kills the nerve cells that control voluntary muscles. ALS causes the nerves to lose the ability to activate specific muscles, which causes the muscles to become weak and leads to paralysis. Most cases will result in death from respiratory failure, usually within 3 to 5 years from when the symptoms first appear.

    — According to the latest data from the Centers for Disease Control and Prevention (CDC), at least 16,000 Americans have ALS.

- Radicava ORS is an orally administered version of Radicava, which was originally approved in 2017 as an intravenous (IV) infusion to treat ALS.

- The effectiveness of Radicava ORS is based on a study that showed comparable levels of Radicava ORS in the bloodstream to the levels from the IV formulation of Radicava.

    — The efficacy of Radicava for the treatment of ALS was previously demonstrated in a 6-month clinical study that served as the basis for approval in 2017. In that study, 137 participants were randomized to receive Radicava or placebo. At week 24, individuals receiving Radicava declined less on a clinical assessment of daily functioning compared to those receiving placebo.

- Warnings and precautions for Radicava ORS include hypersensitivity reactions and sulfite allergic reactions.

- The most common adverse reactions (≥ 10% of patients treated with Radicava and greater than placebo) with Radicava ORS use were contusion, gait disturbance, and headache.

- The recommended dose of Radicava ORS is 105 mg (5 mL) taken orally or via feeding tube in the morning after overnight fasting. Radicava ORS should be administered according to the following schedule:

    — An initial treatment cycle with daily dosing for 14 days, followed by a 14-day drug-free period
    — Subsequent treatment cycles with daily dosing for 10 days out of 14-day periods, followed by 14-day drug-free periods.

- Patients treated with 60 mg of Radicava IV infusion may be switched to 105 mg (5 mL) Radicava ORS using the same dosing frequency. Upon switching to Radicava ORS, patients should follow Radicava ORS dosing recommendations with regards to food consumption.

- Mitsubishi Tanabe Pharma's launch plans for Radicava ORS are pending. Radicava ORS will be available as a 105 mg/5 mL oral suspension.