Radicava™ (edaravone) – New orphan drug approval

- On May 5, 2017, the FDA announced the approval of Mitsubishi Tanabe Pharma Corporation's Radicava (edaravone) injection for the treatment of amyotrophic lateral sclerosis (ALS).
    - MT Pharma America is a subsidiary of Mitsubishi Tanabe Pharma Corporation.
- ALS, commonly referred to as Lou Gehrig's disease, is a rare disease that attacks and kills the nerve cells of muscles involved in movements such as chewing, walking, breathing, and talking. ALS is progressive and leads to paralysis.
- The Centers for Disease Control and Prevention estimates that approximately 12,000 – 15,000 Americans have ALS. Most people with ALS die from respiratory failure, usually within three to five years from when the symptoms first appear.
- The mechanism by which Radicava exerts its therapeutic effect in patients with ALS is unknown.
- The efficacy of Radicava was based on data from a 6 month, placebo-controlled clinical study of 137 patients with ALS. The primary efficacy endpoint was a comparison of change between treatment arms in the ALS functional rating scale – revised (ALSFRS-R) from baseline to week 24. Higher ALSFRS-R scores represent greater functional ability.
    - The decline in ALSFRS-R scores from baseline was significantly less in Radicava-treated patients vs. placebo-treated patients (-5.01 ± 0.64 vs. -7.50 ± 0.66, respectively, p = 0.0013; treatment difference: 2.49, 95% CI: 0.99, 3.98).
- Warnings and precautions of Radicava include hypersensitivity reactions and sulfite allergic reactions.
- The most common adverse reactions (≥ 10% and greater than placebo) with Radicava use were contusion, gait disturbance, and headache.
- The recommended dosage of Radicava is 60 mg administered as an intravenous (IV) infusion over 60 minutes as follows:
    - Initial treatment cycle: daily dosing for 14 days, followed by a 14-day drug free period
    - Subsequent treatment cycles: daily dosing for 10 days out of 14-day periods, followed by 14-day drug-free periods
- MT Pharma America created Searchlight Support, a patient access program for people with ALS who are prescribed Radicava. Searchlight Support will provide personal case management, reimbursement support, and 24/7 clinical support.

Continued . . .

- The cost of Radicava is $1,086 per infusion ($145,524 per year).
- MT Pharma America plans to launch Radicava in August 2017. Radicava will be available as a 30 mg/100 mL (0.3 mg/mL) solution for IV infusion.