Revcovi™ (elapegademase-lvlr) – New orphan drug approval

- On October 5, 2018, Leadiant Biosciences announced the FDA approval of Revcovi (elapegademase-lvlr) for the treatment of adenosine deaminase severe combined immune deficiency (ADA-SCID) in pediatric and adult patients.

- ADA-SCID is an ultra-rare, inherited genetic disorder, caused by a deficiency in the ADA enzyme that is fatal if left untreated. Patients affected by ADA-SCID have compromised immune systems that leave them unprotected from infection-producing bacteria, viruses, and fungi. ADA-SCID is estimated to occur in approximately one in 200,000 to one in 1,000,000 newborns around the world.

- ADA-SCID primarily affects infants and young children and is typically diagnosed within the first few months of life. SCID newborn screening in most states has allowed detection of ADA-SCID in newborns and has led to early initiation of ADA enzyme therapy and improved outcomes.

- Revcovi is a PEGylated recombinant adenosine deaminase (rADA) enzyme that works by supplementing levels of an essential enzyme called ADA. The recombinant technology eliminates the need to source the enzyme from animals.

    - Adagen® (pegademase bovine) is an enzyme replacement therapy also approved for the treatment ADA-SCID. However, the ADA used in the manufacture of Adagen is derived from bovine intestine.

- The safety and efficacy of Revcovi is based on data from two open-label clinical studies of a total of ten patients with ADA-SCID. The study endpoints evaluated ADA activity, levels of toxic metabolites that are the hallmark of ADA-SCID, and changes in lymphocyte counts.

    - Overall, results demonstrated that Revcovi increases ADA activity, reduces concentrations of toxic metabolites and improves total lymphocyte counts.

- Warnings and precautions of Revcovi include injection site bleeding in patients with thrombocytopenia and delay in improvement of immune function.

- The most common adverse reactions reported with Revcovi use were cough (50%) and vomiting (33%).

- The recommended starting dose of Revcovi is determined by whether or not the patient has had prior treatment with Adagen.

    - Transitioning from Adagen to Revcovi: 0.2 mg/kg weekly, intramuscularly (dose may also be calculated using a conversion formula provided in the drug label).
    - Adagen-naïve patients: 0.4 mg/kg weekly based on ideal body weight, divided into two doses (0.2 mg/kg twice a week), intramuscularly.
    - Refer to the Revcovi drug label for complete dosing instructions and therapeutic monitoring information.

Continued . . .

- Leadiant Biosciences' launch plans for Revcovi are pending. Revcovi will be available as a 2.4 mg/1.5 mL (1.6 mg/mL) single dose vial**.**