Mepsevii™ (vestronidase alfa-vjbk) – New orphan drug approval

- On November 15, 2017, the FDA announced the approval of Ultragenyx's Mepsevii (vestronidase alfa-vjbk), in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome).
    - The effect of Mepsevii on the central nervous system manifestations of MPS VII has not been determined.
- MPS is an inherited, lysosomal storage disorder caused by the deficiency of an enzyme called beta-glucuronidase, which results in abnormal build-up of toxic materials in the body's cells. MPS impacts < 150 patients worldwide.
    - Most MPS patients have various skeletal abnormalities that become more pronounced with age, including short stature. Patients may also develop heart valve abnormalities, enlarged liver and spleen, and narrowed airways which can lead to lung infections and difficulty breathing.
    - Affected individuals may have developmental delay and progressive intellectual disability.
    - Heart disease and airway obstruction are major causes of death in people with MPS VII.
- Mepsevii is an enzyme replacement treatment that works by replacing the deficient enzyme in MPS VII.
- The safety and efficacy of Mepsevii were established in clinical trial and expanded access protocols involving a total of 23 patients (5 months – 25 years old). Efficacy was primarily assessed via the six-minute walk distance test in 10 patients who could perform the test.
    - After 24 weeks of treatment, the mean difference in distance walked relative to placebo was 18 meters.
    - Additional follow-up for up to 120 weeks suggested continued improvement in three patients and stabilization in the others. Two patients in the Mepsevii development program experienced marked improvement in pulmonary function. Overall, the results observed would not have been anticipated in the absence of treatment.
- Mepsevii carries a boxed warning regarding the risk of anaphylaxis.
- Warnings and precautions of Mepsevii include anaphylaxis.
- The most common adverse reactions (≥ 1 patient) with Mepsevii use were infusion site extravasation, diarrhea, rash, anaphylaxis, infusion site swelling, peripheral swelling and pruritus.
- The recommended dose of Mepsevii is 4 mg/kg administered every 2 weeks as an intravenous infusion.
    - Mepsevii should be given under the supervision of a healthcare professional with the capability to manage anaphylaxis.
    - Premedication with a non-sedating antihistamine with or without an antipyretic medication is recommended 30 – 60 minutes prior to the start of infusion for patient comfort.
- In order to support patients, Ultragenyx has launched UltraCare™, a comprehensive support service that will provide ongoing support to patients and caregivers. UltraCare will help patients obtain coverage and assist with financial support for both medication and administration of medication.

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- Ultragenyx plans to launch Mepsevii later this month. Mepsevii will be available as a 10 mg/5 mL single-dose vial for injection.