Voxzogo™ (vosoritide) – New orphan drug approval

- On November 19, 2021, the FDA announced the approval of BioMarin's Voxzogo (vosoritide), to increase linear growth in pediatric patients with achondroplasia who are 5 years of age and older with open epiphyses.
    - This indication is approved under accelerated approval based on an improvement in annualized growth velocity. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).
- Achondroplasia is a genetic condition that causes severely short stature and disproportionate growth. The average height of an adult with achondroplasia is approximately four feet. People with achondroplasia have a genetic mutation that causes a certain growth regulation gene called fibroblast growth factor receptor 3 to be overly active, which prevents normal bone growth.
    - The worldwide incidence rate of achondroplasia is about one in 25,000 live births.
- Voxzogo works by binding to a specific receptor called natriuretic peptide receptor-B that reduces the growth regulation gene's activity and stimulates bone growth.
- The efficacy of Voxzogo was established in a 52-week, randomized, double-blind, and placebo-controlled study in 121 patients with achondroplasia. Patients were randomized to either Voxzogo or placebo. The primary endpoint was the change from baseline in annualized growth velocity (AGV) at week 52 compared with placebo.
    - The change from baseline in AGV was -0.17 cm/year with placebo vs. 1.40 cm/year with Voxzogo (difference of 1.57 cm/year, 95% CI: 1.22, 1.93; $p < 0.0001$).
- A warning and precaution for Voxzogo is risk of low blood pressure.
- The most common adverse reactions (> 10%) with Voxzogo use were injection site erythema, injection site swelling, vomiting, injection site urticaria, arthralgia, decreased blood pressure, and gastroenteritis.
- The recommended dosage of Voxzogo is based on the patient's actual body weight. Voxzogo is administered by subcutaneous injection once daily. Refer to the drug label for complete dosing information.
    - Caregivers may inject Voxzogo subcutaneously after proper training by a healthcare professional on the preparation and administration of Voxzogo.
    - Patient body weight, growth, and physical development should be monitored and assessed regularly every 3 to 6 months. The dosage should be adjusted according to the patient's actual body weight. Voxzogo should be discontinued permanently upon confirmation of no further growth potential, indicated by closure of epiphyses.

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- BioMarin plans to launch Voxzogo mid- to late-December. Voxzogo will be available as a 0.4 mg, 0.56 mg, or 1.2 mg lyophilized powder in single-dose vials for reconstitution

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