Study to Evaluate the Efficacy and Safety of BMN 333 Versus Vosoritide in Children With Achondroplasia

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 2 years to 17 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

1. Participants must be aged ≥ 2 to < 11 years (Phase 2) or ≥ 2 to < 18 years (Phase 3), at the time of signing the informed consent
2. Participants must have ACH (confirmed by documented genetic testing) and open epiphyses
3. Are Tanner Stage I (Phase 2) or any Tanner stage (Phase 3)
4. Are ambulatory and able to stand without assistance

Exclusion Criteria:

1. Have any short stature condition other than ACH (eg, hypochondroplasia, trisomy 21, pseudoachondroplasia, GH deficiency)
2. Have any of the following disorders: Hypothyroidism or hyperthyroidism, unless treated with evidence of normalized thyroid-stimulating hormone (TSH) levels, diabetes mellitus, unless considered well-controlled, autoimmune inflammatory disease, inflammatory bowel disease, autonomic neuropathy, anemia defined as hemoglobin < 10 g/dL, vitamin D deficiency, significant hip pathology.
3. Have history of any renal insufficiency or cardiac/ cardiovascular disease that places the participant at increased risk of an adverse cardiac outcome in the setting of hypotension.
4. Have had bone fractures of the long bones or spine within 6 months prior to screening.
5. Have used vosoritide, any other approved product (except GH, as detailed below), investigational product, or investigational medical device for the treatment of ACH or short stature at any time
6. Have been treated with GH, insulin-like growth factor 1, or anabolic steroids in the 6 months prior to treatment start

Conditions & Interventions

Interventions:
DRUG: BMN 333, DRUG: Vosoritide Injection [Voxzogo]
Conditions:
Achondroplasia
Keywords:
Achondroplasia, ACH, Bone Diseases, Developmental Dwarfism, Bone Diseases, Genetic Diseases, Inborn, Musculoskeletal Diseases, Natriuretic Peptide, C-type, Osteochondrodysplasias, Physiological Effects of Drugs, Skeletal Dysplasias

More Information

Description:
This is a multicenter, multinational, randomized, active-controlled, operationally seamless Phase 2/3 study of BMN 333 in treatment-naïve pediatric participants with achondroplasia (ACH). The study consists of a Phase 2 part and a Phase 3 part.

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