# A Study of Vosoritide in Children With Noonan Syndrome With Inadequate Growth During or After Human Growth Hormone Treatment

Status: RECRUITING

## Eligibility Criteria

Sex: ALL
Age: 3 years to 11 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

1. Participants must be ≥ 3 years old, and < 11 years old (females) or < 12 years old (males), at the time of signing the informed consent form
2. A clinical diagnosis of Noonan syndrome.
3. A height assessment corresponding to a height Z-score of ≤ -1.28 SDs (below the 10th percentile for height) in reference to the general population of the same age and sex.
4. Tanner Stage 1, at time of signing the ICF.
5. Previous or current hGH treatment for short stature associated with their condition.
6. Inadequate growth confirmed with an AGV that is less than age- and sex-matched average stature AGV determined using median heights from CDC growth charts

Exclusion Criteria:

1. Diagnosis of systemic disease or condition that may cause short stature other than Noonan syndrome, eg, renal, neoplastic, pulmonary, cardiac, gastrointestinal, immunologic and metabolic disease.
2. Bone age advanced beyond chronological age by more than 2 years.
3. Uncorrected congenital heart disease which places the participant at increased risk of an adverse cardiac outcome in the setting of hypotension,
4. Have an unstable condition likely to require surgical intervention during the study.
5. Evidence of decreased growth velocity (AGV < 1.5 cm/year) as assessed over a period of at least 6 months and growth plate closure assessed using bilateral lower extremity X-rays.
6. Previous limb-lengthening surgery, or planned or expected to have limb lengthening surgery during the study period.
7. Planned or expected bone-related surgery (ie, surgery involving disruption of bone cortex, excluding tooth extraction), during the study period.

## Conditions & Interventions

Interventions:
DRUG: Vosoritide Injection
Conditions:
Noonan Syndrome
Keywords:
Short Stature, Musculoskeletal Diseases, Bone Diseases, Developmental Endocrine System Diseases Natriuretic Peptide, C-Type

## More Information

Description:
The purpose of this study in children with Noonan syndrome is to evaluate the effect of 3 doses of vosoritide on growth as measured by AGV after 6 months of treatment. The long-term efficacy and safety of vosoritide at the therapeutic dose will be evaluated up to FAH.

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