

# A Basket Study of Vosoritide in Children With Turner Syndrome, Short Stature Homeobox-Containing Gene Deficiency, and Noonan Syndrome With Inadequate Growth During or After Human Growth Hormone Treatment

NCT06668805

---

Status	RECRUITING
Phase	Phase 2
Sponsor	BioMarin Pharmaceutical
Enrollment	54 participants

## Key Eligibility Criteria

---

### Inclusion (6)

- Participants must be  $\geq 3$  years old, and  $< 11$  years old (females) or  $< 12$  years old (males), at the time of signing the informed consent form
- A genetically confirmed diagnosis of Turner syndrome, SHOX deficiency or Noonan syndrome.
- A height assessment corresponding to a height Z-score of  $\leq -1.28$  SDs (below the 10th percentile for height) in reference to the general population of the same age and sex.
- Tanner Stage 1, at time of signing the ICF.
- Previous or current hGH treatment for short stature associated with their condition.

... and 1 more (see full listing online)

### Exclusion (8)

- Participants with Turner syndrome known to have Y-chromosome material unless they have undergone gonadectomy and have fully external female genitalia.
- Diagnosis of systemic disease or condition that may cause short stature other than Turner syndrome, SHOX deficiency, or Noonan syndrome, eg, renal, neoplastic, pulmonary, cardiac, gastrointestinal, immunologic and metabolic disease.
- Bone age advanced beyond chronological age by more than 2 years.
- Uncorrected congenital heart disease which places the participant at increased risk of an adverse cardiac outcome in the setting of hypotension,
- Have an unstable condition likely to require surgical intervention during the study.

... and 3 more (see full listing online)

## Locations (24 total)

---

Children's Hospital of Orange County Main Campus - Orange, Orange, California, United States

Children's Hospital Colorado, Aurora, Colorado, United States

Nemours Children's Hospital, Delaware (Alfred I. Dupont Hospital for Children), Wilmington, Delaware, United States

... and 21 more locations