A Phase 2, Randomized, Human Growth Hormone-Controlled, Multicenter, Basket Study of Vosoritide in Children with Turner Syndrome, Short Stature Homeobox-Containing Gene Deficiency, and Noonan Syndrome with an Inadequate Response to Human Growth Hormone

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Up to 18 years old
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- Males >= 3 years old to < 11 years old - Females >= 3 years old to < 10 years old - Genetically confirmed diagnosis of Turner syndrome, SHOX deficiency or Noonan Sydrome - Have been receiving continuous human growth hormone treatment of short stature associated with their condition for a minimum of 1 year

Exclusion Criteria:

- Diagnosis of another systemic disease or condition that may cause short stature

Conditions & Interventions

Conditions:
Bone, Joint & Muscle, Rare Diseases
Keywords:
Noonan Syndrome, short stature, Turner Syndrome

More Information

Description: This study is enrolling children with Turner syndrome, SHOX deficiency, or Noonan syndrome to evaluate the effect of 3 doses of a study drug, vosoritide, versus the standard of care human Growth Hormone (hGH). The study will look at growth over a 6 month period of time. The study will also look at how well the the study drug works (efficacy) and its safety at the therapeutic dose up until the child reaches their final adult height.
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