

Prospective Clinical Assessment Study in Children With Hypochondroplasia

Status: RECRUITING

Eligibility Criteria

Sex: ALL

Age: 30 months to 16 years old

Healthy Volunteers: This study is NOT accepting healthy volunteers

Inclusion Criteria:

Signed informed consent.

Aged 2.5 to <17 years at study entry.

Diagnosis of HCH documented clinically by the presence of disproportionate short stature and confirmed with a molecular test.

Participants are ambulatory and able to stand without assistance.

Study participants and parent(s), guardian(s), or caregiver(s) are willing and able to comply with study visits and study procedures.

Exclusion Criteria:

Have ACH or short stature condition other than HCH.

In females, having had their menarche. Annualized height growth velocity ≤ 1.5 cm/year over a period ≥ 6 months prior to screening.

Having a clinically significant disease or condition that in view of the investigator or Sponsor will interfere with the evaluation of growth, with study participation or not be in the best interest of the participant.

Clinically significant abnormality in any laboratory test result at screening

Current evidence of corneal or retinal disorders.

Have used any other investigational or approved product or medical device for the treatment of HCH or short stature for ≥ 30 days or with the last dose <6 months before screening.

Have had regular long-term treatment (>1 month) with oral corticosteroids (low-dose ongoing inhaled steroid for asthma is acceptable).

Previous limb-lengthening surgery or guided growth surgery with plates still in place or removed within the 6 months prior to screening.

Having had a fracture of the long bones or spine within 12 months of screening.

History and/or current evidence of extensive ectopic tissue calcification.

History of malignancy.

Concurrent circumstance, disease, or condition that, in the view of the investigator and/or sponsor, would interfere with study participation, and/or would place the participant at high risk for poor compliance with study activities or for not completing the study.

Current participation in any other ongoing clinical study with another sponsor.

Conditions & Interventions

Conditions:

Hypochondroplasia

Keywords:

skeletal dysplasia, endochondral ossification, hypochondroplasia, HCH, shortened proximal limbs, fibroblast growth factor receptor 3, FGFR3, endochondral bone formation, disproportionate short stature, quality of life, dwarfism, bone diseases, musculoskeletal diseases, osteochondrodysplasia, functional abilities, annualized growth velocity, annualized height velocity, growth, genetic diseases, congenital, AHV, AGV

More Information

Description:

This is a long-term, multicenter, non-interventional study of children ages 2.5 to <17 years with hypochondroplasia (HCH).

Contact(s): Laurie Laurie Bailey - Laurie.Bailey@cchmc.org

Principal Investigator:

Principal Investigator ID:

Phase:

IRB Number:

System ID: NCT06410976

