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Recruiting ⓘ

A Clinical Trial to Evaluate Efficacy and Safety of TransCon CNP Compared With Placebo in Infants (0 to <2 Years of Age) With Achondroplasia

ClinicalTrials.gov ID ⓘ NCT06079398

Sponsor ⓘ Ascendis Pharma A/S

Information provided by ⓘ Ascendis Pharma A/S (Responsible Party)

Last Update Posted ⓘ 2025-11-26

Study Details Tab

Study Overview

Brief Summary

This trial is a Phase 2, multicenter, double-blind, randomized (ratio 2:1 TransCon CNP vs. placebo), placebo-controlled trial, designed to evaluate the safety, tolerability, and efficacy of 100 µg CNP/kg of Navepegritide (TransCon CNP) administered SC once-weekly for 52 weeks in infants with genetically verified heterozygous ACH, aged 0 to < 2 years at the time of randomization.

Official Title

A Phase 2, Multicenter, Double-Blind, Randomized, Placebo-controlled Trial, Evaluating Safety, Tolerability, and Efficacy of Subcutaneous Doses of TransCon CNP Administered Once Weekly for 52 Weeks in Infants (0 to <2 Years of Age) With Achondroplasia Followed by an Open Label Extension (OLE) Period

Conditions ⓘ

Achondroplasia

Study Start (Actual) ⓘ

2024-01-23

Primary Completion (Estimated) ⓘ

2026-03

Study Completion (Estimated) ⓘ

2027-03

Enrollment (Estimated) ⓘ

72

Study Type ⓘ

Interventional

Phase ⓘ

BIOMARIN EXHIBIT 2016

Ascendis Pharma A/S v. BioMarin Pharmaceutical Inc.

PGR2026-00013

- Drug: Navepegritide
- Drug: Placebo for Navepegritide

Other Study ID Numbers ⓘ

- ASND0030

Resource links provided by the National Library of Medicine

[MedlinePlus Genetics](https://medlineplus.gov/genetics/) (<https://medlineplus.gov/genetics/>), related topics:

[Achondroplasia](https://medlineplus.gov/genetics/condition/achondroplasia) (<https://medlineplus.gov/genetics/condition/achondroplasia>)

[Genetic and Rare Diseases Information Center](https://rarediseases.info.nih.gov/gard) (<https://rarediseases.info.nih.gov/gard>), resources:

[Achondroplasia](https://rarediseases.info.nih.gov/?gard_id=0008173) (https://rarediseases.info.nih.gov/?gard_id=0008173)

[FDA Drug and Device Resources](https://clinicaltrials.gov/fda-links) (<https://clinicaltrials.gov/fda-links>)

Contacts and Locations

This section provides contact details for people who can answer questions about joining this study, and information on where this study is taking place.

To learn more, please see the [Contacts and Locations section in How to Read a Study Record](https://clinicaltrials.gov/study-basics/how-to-read-study-record#contacts-and-locations) (<https://clinicaltrials.gov/study-basics/how-to-read-study-record#contacts-and-locations>).

Study Contact ⓘ

Name: Ascendis Registry Inquiries

Phone Number: +45 61242484

Email: asnd_registryinquiries@ascendispharma.com

This study has 17 locations

United States

Minnesota Locations

-  Saint Paul, Minnesota, United States, 55102
Recruiting
Ascendis Investigational Site

Texas Locations

-  Houston, Texas, United States, 77030
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Ascendis Investigational Site

Wisconsin Locations

-  Madison, Wisconsin, United States, 53705
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Ascendis Investigational Site

Australia

 **Parkville, Australia, 3052**
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Ascendis Investigational Site

Austria

 **Linz, Austria, 4020**
Recruiting
Ascendis Investigational Site

Denmark

 **Copenhagen, Denmark, 2100**
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Ascendis Investigational Site

Finland

 **Helsinki, Finland, 00029**
Recruiting
Ascendis Investigational Site

France

 **Paris, France, 75743**
Recruiting
Ascendis Investigational Site

Germany

 **Berlin, Germany, 13353**
Recruiting
Ascendis Investigational Site

 **Cologne, Germany, 50931**
Recruiting
Ascendis Investigational Site

Ireland

 **Dublin, Ireland, D01 YC76**
Recruiting
Ascendis Investigational Site

Italy

 **Milan, Italy, 20132**
Recruiting
Ascendis Investigational Site

New Zealand

 **Auckland, New Zealand, 1023**
Recruiting
Ascendis Investigational Site

Norway

 **Oslo, Norway, 0372**
Recruiting
Ascendis Investigational Site

Portugal

 **Coimbra, Portugal, 3000-602**
Recruiting
Ascendis Investigational Site

Sweden

 **Stockholm, Sweden, 17176**
Recruiting
Ascendis Investigational Site

United Kingdom

 London, United Kingdom, SE1 7EH
Recruiting
Ascendis Investigational Site

Participation Criteria

Researchers look for people who fit a certain description, called [eligibility criteria](#). Some examples of these criteria are a person's general health condition or prior treatments.

For general information about clinical research, read [Learn About Studies \(https://clinicaltrials.gov/study-basics/learn-about-studies\)](https://clinicaltrials.gov/study-basics/learn-about-studies).

Eligibility Criteria

Description

Inclusion Criteria:

- Written, signed informed consent by the parent(s)/caregiver(s) of the participant, and as required by the institutional review board/human research ethics committee/independent ethics committee (IRB/HREC/IEC).
- Male or female younger than 2 years of age at the time of randomization; or for open label sentinel participants, at the time of first administration of IMP.
- Clinical diagnosis of achondroplasia (ACH) with genetic confirmation of heterozygous genotype present during screening.
- Parent(s)/caregiver(s) willing to follow the protocol and instructions provided, including being able to administer weekly subcutaneous injections of trial treatment.
- Compliance to daily Vitamin D supplementation for infants aged 14 days to 1 year. All participants older than 1 year of age with serum 25-hydroxyvitamin D (25OHD) measured below lower limit of reference range at screening should start daily Vitamin D supplementation prior to randomization.
- Considered eligible based on the medical history, physical examination, and the results of vital signs, ECG, imaging, and clinical laboratory tests performed during the screening period.

Exclusion Criteria:

- Known or suspected hypersensitivity to the investigational product or related products (trehalose, tris[hydroxymethyl]aminomethane, succinate, and polyethylene glycol [PEG]).
- Genetic confirmation of ACH homozygous genotype.
- Premature birth with gestational age < 32 weeks.
- Premature birth with gestational age 32 to 37 weeks, unless time from birth is > 6 months at the time of screening and the child is in good nutritional status, defined as gain in body weight expected for age and diagnosis of ACH, as determined by the Investigator and confirmed with the Medical Monitor.
- Anticipated, as assessed by Investigator and confirmed with Medical Monitor, to undergo surgical intervention during trial participation, including cervicomedullary decompression. Evaluation of immediate risk of requiring cervicomedullary decompression surgery will rely on the following assessments:
 - Physical examination (e.g., neurologic findings of clonus, opisthotonus, exaggerated reflexes, dilated facial veins)
 - Evidence of uncontrolled sleep apnea as confirmed by local standard of care assessment (e.g. polysomnography or simple sleep test) performed within 6 months prior to screening.
 - MRI performed at screening indicating presence of severe cervicomedullary compression (CMC) or spinal cord damage. Presence of abnormal MRI T2 signal intensity at and immediately above and below the cervicomedullary junction should be considered high risk for requiring surgery and the participant is not eligible for trial participation.

Common surgeries, such as insertion of grommets, adenoidectomy, tonsillectomy, or myringotomy tube placement are permitted during trial participation.

Ages Eligible for Study ¹

0 Years to 2 Years (Child)

Sexes Eligible for Study ¹

All

Accepts Healthy Volunteers ¹

No

- Have a growth disorder or medical condition, other than ACH, resulting in short stature or abnormal growth as determined by the Investigator and confirmed with the Medical Monitor.
- Have received any dose of prescription medications and/or investigational medicinal product or device intended to affect stature, growth, or body proportionality (including human growth hormone or vosoritide) at any time.
- Requires or anticipated to require chronic (> 4 weeks) or repeated treatment (more than twice/year) with oral corticosteroids, or high-dose inhaled corticosteroids during trial participation.
- History or presence of injury or disease of the growth plate(s), other than ACH, affecting growth potential of long bones, including Salter-Harris fracture and recent bone-related surgery, as determined by Investigator and confirmed with the Medical Monitor.
- Have a clinically significant finding indicating abnormal cardiac function, including but not limited to:
 - Repaired or unrepaired coarctation.
 - Moderate or greater complexity congenital heart disease including tetralogy of Fallot, atrioventricular septal defects, truncus arteriosus, total anomalous pulmonary venous return, double outlet right ventricle, or single ventricle heart disease.
 - QTcF $\geq$ 450 msec on screening 12-lead ECG.
- History or presence of a condition impacting hemodynamic stability (such as autonomic dysfunction and orthostatic intolerance).
- History or presence of the following:
 - Chronic anemia.
 - Chronic renal insufficiency.
 - Chronic or recurrent illness that can affect hydration or volume status, including conditions associated with decreased nutritional intake or increased volume loss.
- History or presence of malignant disease.
- Any disease or condition that, in the opinion of the Investigator, may make the participant unlikely to fully complete the trial, not adhering to trial procedures, may confound interpretation of trial results, or may present undue risk from receiving trial treatment. This could include family situations, comorbid conditions, or medications that might impact safety or be considered confounding.

Study Plan

This section provides details of the study plan, including how the study is designed and what the study is measuring.

How is the study designed?

Design Details

Primary Purpose ⓘ : Treatment
Allocation ⓘ : Randomized
Interventional Model ⓘ : Parallel Assignment
Masking ⓘ : Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor)

Arms and Interventions

Participant Group/Arm ⓘ	Intervention/Treatment ⓘ
Experimental: Navepegritide Once weekly double-blinded treatment with SC injection of 100 µg/kg of Navepegritide for 52 weeks	Drug: Navepegritide • Once-weekly subcutaneous injection of 100 µg/kg Navepegritide
Placebo Comparator: Placebo for Navepegritide Once weekly double-blinded treatment with SC injection of 100 µg/kg of Placebo for Navepegritide for 52 weeks	Drug: Placebo for Navepegritide • Once-weekly subcutaneous injection of 100 µg/kg placebo for Navepegritide

What is the study measuring?

Primary Outcome Measures ⓘ

Outcome Measure	Measure Description	Time Frame
To evaluate the safety and tolerability of Navepegritide	Incidence of treatment emergent adverse events (TEAEs)	52 weeks
To evaluate the effect of Navepegritide on growth	Change from baseline to 52 weeks in length/height Z-score	52 weeks

Collaborators and Investigators

This is where you will find people and organizations involved with this study.

Sponsor ⓘ

Ascendis Pharma A/S

Investigators ⓘ

- Study Director: Medical Director, MD, Ascendis Pharma A/S

Study Record Dates

These dates track the progress of study record and summary results submissions to ClinicalTrials.gov. Study records and reported results are reviewed by the National Library of Medicine (NLM) to make sure they meet specific quality control standards before being posted on the public website.

Study Registration Dates

First Submitted ⓘ

2023-10-06

First Submitted that Met QC Criteria ⓘ

2023-10-06

First Posted ⓘ

2023-10-12

Study Record Updates

Last Update Submitted that Met QC Criteria ⓘ

2025-11-25

Last Update Posted ⓘ

2025-11-26

Last Verified ⓘ

2025-11

More Information

Terms related to this study

Additional Relevant MeSH Terms

Dwarfism
Bone Diseases, Developmental
Bone Diseases
Musculoskeletal Diseases
Osteochondrodysplasias
Genetic Diseases, Inborn
Congenital, Hereditary, and Neonatal Diseases and Abnormalities
Achondroplasia

Plan for Individual Participant Data (IPD)

Plan to Share Individual Participant Data (IPD)?

No

Drug and device information, study documents, and helpful links

Studies a U.S. FDA-Regulated Drug Product

Yes

Studies a U.S. FDA-Regulated Device Product

No