Comparison of VRS-317, a Long-acting Human Growth Hormone, to Daily rhGH in a Phase 3, Randomized, One-year, Open-label, Multi-center, Non-inferiority Trial in Pre-pubertal Children with Growth Hormone Deficiency.

Published: 23-03-2015
Last updated: 14-04-2024

Primary Objective- Compare the safety and efficacy of subcutaneous somavaratan and daily rhGH during 12 months of treatment.Secondary Objective-Evaluate and compare changes in pharmacodynamic responses (IGF-I, IGF binding protein-3 (IGFBP-3), growth...

Ethical review	Approved WMO
Status	Pending
Health condition type	Hypothalamus and pituitary gland disorders
Study type	Interventional

Summary

ID

NL-OMON42218

Source
ToetsingOnline

Brief title
VELOCITY study

Condition

- Hypothalamus and pituitary gland disorders

Synonym
Growth hormone deficiency, small stature

Research involving

Human

Sponsors and support

Primary sponsor: Versartis Inc.
Source(s) of monetary or material Support: sponsor

Intervention

Keyword: child, deficiency, growth hormone

Outcome measures

Primary outcome

Primary efficacy endpoint

- Annual height velocity (cm/yr) after 12 months of treatment with either

somavaratan, or daily rhGH

Secondary outcome

Secondary efficacy endpoints

-IGF-I and IGFBP-3 responses to study drug administration

-changes in height standard deviation score, body weight, body mass index, bone

age

Study description

Background summary

Growth Hormone Deficiency (GHD) in children results from a variety of genetic, neoplastic, inflammatory, post-traumatic and iatrogenic causes. Subjects with untreated childhood onset GHD will have significant growth failure with attainment of adult heights significantly less then five feet in many instances. In addition, there is abnormal body composition with decreased bone mineralizatoin, decreased lean body mass and increased fat mass. Treatment with exogenous rhGH initiates a period of accelerated or "catch-up" growth that when begun at an early age allows attainment of normal adult height and body composition.

Somavaratan is a recombinant human growth hormone analog, designed to maintain active drug levels for longer period of time than current daily therapies.
Daily therapy is currently the only approved treatment for adults and children with GHD. Long-acting GH offers the possibilities of many fewer injections, enhanced compliance and attainment of improved treatment outcomes.

Study objective

Primary Objective
- Compare the safety and efficacy of subcutaneous somavaratan and daily rhGH during 12 months of treatment.

Secondary Objective
-Evaluate and compare changes in pharmacodynamic responses (IGF-I, IGF binding protein-3 (IGFBP-3), growth hormone binding protein (GHBP) and acid labile subunit (ALS)), bone age, weight, body mass index, height standard deviation scores, and anti-drug antibody (ADA) and neutralizing antibody (NAb) responses.

Study design

This is a randomized, multi-center, open label study comparing study drug with commercial available treatment in a parallell group design.

Intervention

Subjects will receive commercial available recombinant human growth hormone daily ór somavaratan every 15 days during 1 year.

Study burden and risks

Burden and risk:
Pain, bruising or infection due to additional bloodsampling compared to standard of care, additional radiation exposure to less then 1 mSv due to an additional X-ray of the hand, requirement to keep a dosing record at home. An ECG twice; ECG patches could cause a transient rash.

Benefit:
Increase in growth that is similar to that of standard of care. If assigned to the somavaratan treatment group less frequent treatment injections (twice per month) as compared to standard of care (daily)

Contacts

Public

Versartis Inc.

4200 Bohannon Drive Suite 250
94025 Menlo Park
US
Scientific
Versartis Inc.

4200 Bohannon Drive Suite 250
94025 Menlo Park
US

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Children (2-11 years)

Inclusion criteria

1. Chronological Age >= 3.0 years and <= 10.0 (girls) and <= 11.0 (boys).;2. Pre-pubertal status: Absent breast development in girls, testicular volume <;4.0 mL in boys.;3. Diagnosis of GHD as documented two or more GH stimulation test results <= 10.0 ng/mL.;4. Height SDS <= -2.0 at screening.;5. Weight for Stature >= 10th percentile.;6. IGF-I SD score <= -1.0 at screening.;7. Delayed bone age (>= 6 months as determined by the central reader). Left hand X-Ray must be obtained within 90 days of screening visit or during screening.;8. Normal thyroid function test results at screening visit (or a minimum of four weeks of thyroxine replacement therapy prior to study drug administration).;9. Available adrenal function test results at screening visit (or in the preceding 6 months) in all subjects without a minimum of four weeks glucocorticoid replacement therapy prior to study drug administration. ;10. Pathology relating to cause of GH deficiency must be stable for at least 6 months prior to screening.;11. Legally authorized representatives must be willing and able to give informed;consent.

Exclusion criteria

1. Prior treatment with any growth promoting agent (e.g., GH, IGF-I, GH releasing hormone (GHRH), gonadotrophins, sex steroids). Up to 10 day exposures to a growth promoting agent for diagnostic purposes are permitted if administered 30 or more days prior to screening. ;2. Documented history of, or current, significant disease.;3. Chromosomal aneuploidy, significant gene mutations (other than those that cause GHD) or confirmed diagnosis of a named syndrome.;4. Birth weight and/or birth length less than 5th percentile for gestational age using gestational age growth charts.;5. A diagnosis of Attention Deficit Hyperactivity Disorder (ADHD), use of ADHD;medications or a likelihood of starting ADHD medications during study participation.;6. Daily use of anti-inflammatory doses of glucocorticoid.;7. Prior history of leukemia, lymphoma, sarcoma or cancer.;8. Treatment with an investigational drug in the 30 days prior to screening.;9. Known allergy to constituents of the study drug formulation.;10. Ocular findings suggestive of increased intracranial pressure and/or retinopathy at screening.;11. Significant spinal abnormalities including scoliosis, kyphosis and spina bifida;variants.;12. Significant abnormality in screening laboratory studies.;13. Current social conditions which would prevent completion of study activities;(e.g., planned family move to a distant location).;14. History of pancreatitis or undiagnosed chronic abdominal pain.;15. History of spinal or total body irradiation.;16. Subjects with other pituitary hormone deficiency who are not treated properly.;17. Unwillingness to provide consent for participation in all trial activities.;18. Unwillingness to accept dose assignments.

Study design

Design

Study phase:	3
Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-04-2015

Enrollment:	4
Type:	Anticipated

Medical products/devices used

Product type:	Medicine
Brand name:	Genotropin Miniquick
Generic name:	Somatropin
Registration:	Yes - NL outside intended use
Product type:	Medicine
Brand name:	somavaratan
Generic name:	VRS-317

Ethics review

Approved WMO Date:	23-03-2015
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	03-09-2015
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	21-10-2015
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	28-10-2015
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	22-01-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO	

Date:	03-06-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	20-06-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2014-004525-41-NL
ClinicalTrials.gov	NCT02339090
CCMO	NL52440.091.15