Santhera GUARDIAN

A Study on Safety and Effectiveness of Long-term Treatment With Vamorolone in Boys With Duchenne Muscular Dystrophy (GUARDIAN)

Hub Summary

This Phase 4 study aims to assess safety and effectivness of long-term treatment with vamoralone in boys with Duchenne Muscular Dystrophy (DMD) who have completed prior studies with vamoralone.

Study Number: NCT06713135

Description by Santhera Pharmaceuticals

All subjects in this study have completed previous studies with vamorolone and continued to receive vamorolone under special programs: Compassionate Use Program [CUP], Named Patient Program [NPP] or Expanded Access Protocol [EAP]. All subjects will continue treatment with vamorolone under Guardian protocol instead. The primary objective of this study is to evaluate the safety of long-term treatment with vamorolone in boys with Duchenne Muscular Dystrophy regarding vertebral fractures. Secondary study objectives will evaluate the safety of long-term treatment with vamorolone on non-vertebral fractures, cataracts, delayed puberty, overall safety as well as ambulatory and non-ambulatory function.

Primary Outcome Measures

- Number of vertebral fractures per 1000 person-years based on X-ray central reading.

Secondary Outcome Measures

- Time to first vertebral fractures (cumulative incidence)
- Number of non-vertebral fractures per 1000 person-years based on investigator reporting
- Time to first non-vertebral fractures (cumulative incidence)
- Number of cataracts per 1000 person-years based on ophthalmologist assessment
- Number of subjects not reaching Tanner stage 2 by 15 years of age
- Frequency of adverse events (AEs) and serious adverse events (SAEs)
- Change from baseline in body weight
- Number of subjects with clinically relevant laboratory abnormalities
- Change from baseline in Time to Stand (TTSTAND) velocity
- Six-minute Walk Test (6MWT)
- Change from baseline in 6MWT distance
- Age at ambulatory and non-ambulatory milestones
- North Star Ambulatory Assessment (NSAA) scores
- Change from baseline in body height
- Change from baseline in Body Mass Index (BMI)

Can I take part?

Inclusion Criteria

- ✔ Subject and/or subject's parent(s) or legal guardian has provided written informed consent
- ✔ Subject has previously completed either the VBP15-LTE or VBP15-004 study, and transitioned through the Compassionate Use Program, Named Patient Program or Expanded Acess Protocol
- ✔ Subject is on vamorolone on day of enrolment
- ✔ Subject and parent / legal guardian are willing and able to comply with the protocol schedule, assessments and requirements

Trial Status
Fully recruited

UK Locations
London - GOSH, Fully recruited, Alder Hey, Fully recruited, Glasgow, Fully recruited, Leeds, Fully recruited, Newcastle, Fully recruited

Trial Sponsor
Santhera Pharmaceuticals

Phase
Phase 4

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Interventional

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

Exclusion Criteria

- ✖ Any medical condition, which in the opinion of the Investigator, would affect study participation, performance or interpretation of study assessments
- ✖ Vamorolone treatment discontinued for 6 months within the year prior to enrolment for a non-safety reason, or vamorolone treatment previously discontinued at any time for a safety reason
- ✖ Severe hepatic impairment

For contact details and to find out more, please refer to dmdhub.org.

Duchenne UK

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