Santhera SIDEROS Open Label Extension [TERMINATED]

Phase III Study with Idebenone in Patients with Duchenne Muscular Dystrophy

Hub Summary

This study is a stage 3 trial of Sanethera's Idebenone drug and it's long term effects in delaying the loss of lung function in patients with DMD, receiving glucocorticoid steroids. This trial is only open to those patients who were part of the SIDEROS trial and are currently taking steroids.

Study Number: NCT03603288

Description by Santhera Pharmaceuticals

The purpose of the study is to assess the long-term safety and efficacy of idebenone in patients with Duchenne muscular dystrophy (DMD) who completed the SIDEROS study.

The study is an open-label, single-group, multi-center extension study in patients with DMD receiving glucocorticoid steroids who participated in the SIDEROS study and who meet all the inclusion criteria and none of the exclusion criteria for this extension study.

The study consists of 4 study visits scheduled every 6 months (Visit 1/Baseline, Visit 2/Week 26, Visit 3/ Week 52 and Visit 4/ Week 78), and a follow-up visit 4 weeks after treatment discontinuation. Visit 8 /Week 78 in SIDEROS study is also SIDEROS-E Visit 1/Baseline.

Primary Outcome Measures

- Incidence and severity of adverse events, as per ICH Topic E2A [Time Frame: From baseline until visit 4 (week 78)].
- Incidence and severity of adverse events, as per ICH Topic E2A [Time Frame: 4 weeks after discontinuation of treatment].
- Number of patients with premature discontinuations of study treatment due to adverse events. [Time Frame: From baseline until visit 4 (week 78)].
- Number of patients with abnormal safety laboratory parameters. [Time Frame: From baseline until visit 4 (week 78)]
- Number of patients with abnormal safety laboratory parameters. [Time Frame: 4 weeks after discontinuation of treatment]
- Number of patients with abnormal vital signs. [Time Frame: From baseline until visit 4 (week 78)]
- Number of patients with abnormal vital signs. [Time Frame: 4 weeks after discontinuation of treatment]
- Number of patients with abnormal ECG. [Time Frame: From baseline until visit 4 (week 78)]

Secondary Outcome Measures

To describe the long-term evolution of respiratory function in idebenone-treated DMD patients who completed the SIDEROS study:

- Change from Baseline in Forced Vital Capacity (FVC) as percent of predicted (FVC%p). [Time Frame: From baseline until visit 4 (week 78)]
- Change from Baseline in Peak Expiratory Flow (PEF) as percent of predicted (PEF%p) [Time Frame: From baseline until visit 4 (week 78)]
- Change from Baseline in Forced Expiratory Volume in 1 second (FEV1) as percent of predicted (FEV1%p) [Time Frame: From baseline until visit 4 (week 78)]

Can I take part?

Inclusion Criteria

- ✔ Completion of the SIDEROS study at Visit 8/ Week 78
- ✔ Signed and dated Informed Consent Form for SIDEROS-E

Exclusion Criteria

- ✖ Patients who discontinued SIDEROS study prematurely (i.e. did not attend all visits from V1 to V8)

Trial Status
Trial terminated

UK Locations
London - GOSH, Trial complete/terminated, Leeds, Trial complete/terminated, Newcastle, Trial complete /terminated, Oswestry, Trial complete/terminated

Trial Sponsor
Santhera Pharmaceuticals

Phase
3

Length Of Participation
18 months

Recruitment Target
266

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Respiratory/mitochondrial

Age
11 +

Mutation Specific
All treatment types

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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- ✖ Safety, tolerability or other issues arising during the course of the SIDEROS study which in the opinion of the Investigator may put the patient at significant risk or may interfere significantly with the patient's participation in the SIDEROS-E study
- ✖ Use of any investigational drug other than the study medication

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

Duchenne UK

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