A Phase IIIb Double-Blind, Randomised, Placebo-Controlled Study of Patient Reported Outcomes in Friedreichâ¤*s Ataxia Patients after withdrawal from Treatment with Idebenone (PROTI Study)

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Other condition
Study type	Interventional

Summary

ID

NL-OMON36407

Source
ToetsingOnline

Brief title
PROTI

Condition

- Other condition

Synonym
hereditary ataxia; neuromuscular disease

Health condition

neuromuscular disorder, genetic, degenerative diseases affecting nerve and muscle tissue.

Research involving
Human

Sponsors and support

Primary sponsor: Santhera
Source(s) of monetary or material Support: Santhera Pharmaceuticals (Switzerland) Ltd.

Intervention

Keyword: Friedreich□s Ataxia, Idebenone

Outcome measures

Primary outcome

Patient assessment of treatment assignment:

Comparison of the proportions of patients randomised to idebenone and placebo

who assessed that they received idebenone

Secondary outcome

The key secondary objective is to compare the rate of withdrawal from the PROTI

study of patients who have been withdrawn from treatment with high-dose

idebenone with the rate of withdrawal of patients continuing to receive high

dose idebenone.

Other secondary endpoints:

• Change in fatigue level as assessed by the MFIS

• Change in 9-HPT time

• Change in speech capability

• CGI-C

• Investigator assessment of treatment assignment

• Change in ICARS score

• PROs collected via Daily Patient Diary and Questionnaires

• Safety

Study description

Background summary

A Phase IIIb Double-Blind, Randomised, Placebo-Controlled Study of Patient Reported Outcomes in Friedreich*s Ataxia Patients after withdrawal from Treatment with Idebenone will be achieved by randomising patients receiving high dose idebenone in MICONOS-Extension study.
Therefore, patients participating in the MICONOS-Extension study will be invited to suspend their participation in MES to participate in a randomised withdrawal study in which they will be randomised either to placebo or to continue on high dose idebenone treatment for a period of 2 months. During this period, important additional information not otherwise available from the MES will be collected, including Patient Reported Outcomes and performance measures.

Study objective

The primary objective of the PROTI study is to establish whether patients can correctly determine which treatment assignment they received during the randomised phase of the trial.

The key secondary objective is to compare the rate of withdrawal from the PROTI study of patients who have been withdrawn from treatment with high-dose idebenone with the rate of withdrawal of patients continuing to receive high dose idebenone.

Secondary objectives are to compare Patient Reported Outcomes (PROs) and performance measures in FRDA patients who have been withdrawn from treatment with high-dose idebenone and to compare these outcomes and performance measures with those from patients continuing to receive high dose idebenone.

Study design

Double-blind, randomised, placebo-controlled, parallel-group multi-centre withdrawal study

Intervention

Group A will receive Idebenone: Patients <=45 kg: 1350 mg/day - 3 tablets 3 times a day with food
Patients >45 kg: 2250

mg/day - 5 tablets 3 times a day with food
Group B will receive placebo: Placebo <=45 kg - 3 tablets 3 times a day with food
Placebo >45 kg - 5
tablets 3 times a day with food

Study burden and risks

Burden associated with participation as required by study procedures is:
- 2 study visits in period of 2 months per cycle. At each visit following test
will be performed:
Pregnancy test for women of childbearing potential
Routine ECG
Safety blood and urine sample
Performance measures and neurological assessments

- Patients will also complete a Patient Report Outcomes (PRO) Status
Questionnaire at first visit and Daily Patient Diary throughout the study.
- At second visit patient will complet PRO Change Questionnaire.

The risks of treatmant are considered to be acceptable.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

1. Completion of Visit 5 (Month 12), Visit 6 (Month 18), or Visit 7 (Month 24) in MICONOS-extension study
2. Body weight >= 25 kg
3. Patients who in the opinion of the investigator are able to comply with the requirements of the study
4. Negative urine pregnancy test (women of childbearing potential)

Exclusion criteria

1. Adverse event during the course of MES which in the opinion of the investigator is attributable to idebenone and precludes further treatment with idebenone
2. Clinically significant abnormalities of clinical haematology or biochemistry including, but not limited to, elevations greater than 1.5 times the upper limit of normal of SGOT, SGPT, or creatinine
3. Parallel participation in another clinical drug trial
4. Pregnancy or breast-feeding
5. Abuse of drugs or alcohol
6. Any change of concomitant medication within the last 30 days that in the opinion of the investigator the intake could negatively impact the study

Study design

Design

Study phase:	3
Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial

Masking:	Double blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	02-08-2011
Enrollment:	6
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	N/A
Generic name:	idebenone

Ethics review

Approved WMO Date:	15-03-2011
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	08-07-2011
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)

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	EUCTR2010-023388-16-NL
ClinicalTrials.gov	NCT01303406
CCMO	NL35243.042.11

Study results

Date completed:	25-10-2011
Actual enrolment:	3