A Non-interventional Study of Clinical Experience in Patients Prescribed Raxone® for the Treatment of Leber’s Hereditary Optic Neuropathy (LHON) (PAROS)

First published: 23/05/2016

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Study Finalised

Administrative details

EU PAS number

EUPAS13438

Study ID

48523

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ France
- ☐ Germany

- [ ] Greece
- [ ] Italy
- [ ] Netherlands
- [ ] Norway

Study description

This study is a multicentre, prospective, non-interventional post-authorisation safety study (PASS) to collect additional information on the use of Raxone® when used under conditions of routine clinical practice.

Study status

Finalised

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Valerio Carelli

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/10/2015

Actual: 23/09/2016

Study start date
Planned: 03/10/2016

Actual: 23/09/2016

Date of final study report
Planned: 13/12/2021

Actual: 18/05/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Santhera Pharmaceuticals (Switzerland) Ltd

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

To further evaluate the long-term safety profile of Raxone® in the treatment of patients with LHON when used under conditions of routine clinical care

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Multicentre, prospective, non-interventional post-authorisation safety study (PASS)

Study drug and medical condition

Name of medicine

RAXONE

Medical condition to be studied

Hereditary optic atrophy

Population studied

Short description of the study population

Patients with Leber’s hereditary optic neuropathy prescribed treatment with Raxone® under routine clinical practice.

Age groups

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Special population of interest

Renal impaired

Hepatic impaired

Pregnant women

Other

Special population of interest, other

Patients with Leber’s hereditary optic neuropathy

Estimated number of subjects

250

Study design details

Outcomes

• Frequency of adverse events of special interest (AESIs) • Frequency and nature of AEs and serious adverse events (SAEs), • Frequency and nature of adverse drug reactions (ADRs) and serious adverse drug reactions (SADRs), • Assessment of long term outcomes when Raxone® is used according to the SmPC

Data analysis plan

Collection of safety data, responder analysis

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Drug registry

Other

Data sources (types), other

Prospective patient-based data collection

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No