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

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# 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**

Julien Gaudias julien.gaudias@santhera.com

Study contact

julien.gaudias@santhera.com

### **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

# 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

# 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 sources (types)

### **Data characterisation conducted**

No