European Post-Authorization Registry for RAVICTI® (glycerol phenylbutyrate) Oral Liquid in Partnership with the European Registry and Network for Intoxication Type Metabolic Diseases (E-IMD) (HZNP-RAV-401)

First published: 16/01/2017

Last updated: 02/07/2024

Study Finalised

Administrative details

EU PAS number

EUPAS17267

Study ID

30377

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Denmark

- [ ] France
- [ ] Germany
- [ ] Poland

Study description

This is a multi-center, prospective, non-interventional registry conducted by E-IMD in collaboration with Immedica Pharma designed to collect data on safety and outcomes in patients with urea cycle disorders (UCDs) on treatment with RAVICTI or patients with UCDs treated with alternative nitrogen scavenging medication other than RAVICTI.

Study status

Finalised

Research institutions and networks

Institutions

Multiple centres: 5 centres are involved in the study

Networks

E-IMD

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Åsa Jansson

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 10/10/2016

Study start date

Actual: 07/11/2017

Data analysis start date

Planned: 31/01/2030

Actual: 27/06/2022

Date of final study report

Planned: 31/07/2030

Actual: 31/10/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Immedica Pharma AB

Study protocol

V3 0_RAVICTI-EU-Registry_protocol__20161129_final-clean_incl app with redaction_Redacted_sk_Redacted_sk.pdf(2.7 MB)

V5 0_RAVICTI-EU-Registry_protocol__26 Apr 2018_Final Signed_Complete_Redacted (2).pdf(9.18 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

Evaluation and characterization of the safety profile of RAVICTI and long-term outcomes in UCD patients treated with RAVICTI. The registry includes a comparator group treated with alternative nitrogen scavenging medication. In all other relevant characteristics (age group, severity of the UCD and gender for OTC patients), the comparator group will be adequately matched to the study group.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Multi-centre, prospective, PASS study

Study drug and medical condition

Name of medicine

RAVICTI

Study drug International non-proprietary name (INN) or common name

GLYCEROL PHENYLBUTYRATE

Anatomical Therapeutic Chemical (ATC) code

(A16AX09) glycerol phenylbutyrate

glycerol phenylbutyrate

Medical condition to be studied

Urea cycle disorder

Population studied

Short description of the study population

The study population included adult and pediatric patients diagnosed with urea cycle disorder received treatment with RAVICTI or alternative nitrogen scavenging medication.

Age groups

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

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

Other

Pregnant women

Renal impaired

Special population of interest, other

Patients with urea cycle disorder

Estimated number of subjects

200

Study design details

Outcomes

Collect relevant long-term safety data in patients with UCDs treated with RAVICTI and also collect and compare this data to a comparator group treated with alternative nitrogen scavenging medication., For the RAVICTI and comparator group, collect and compare information on: Incidence rate and type of cancer Occurrence of potential PAA (phenylacetate) toxicity Safety information in patients with concurrent renal impairment Pregnancy outcomes in children born to female patients

Data analysis plan

Descriptive statistics including number of observations, mean, standard deviation (SD), median, minimum, and maximum for continuous variables; and n and percent for categorical variables. Data will be presented for patients in the RAVICTI and the comparator group. Data of comparator group patients will

only be presented if consent is given. Additional subgroups may be examined, as appropriate (pediatric versus adult, UCD type, etc.). Disposition data will be summarized with descriptive statistics. Demographic and clinical data will be summarized with descriptive statistics. Post-baseline values and/or change from baseline in the outcome variables will be summarized with descriptive statistics, and, where appropriate, graphical presentations. Differences in outcome variables between the groups will be evaluated using generalized linear mixed models (continuous and dichotomous endpoints) as well as hazard models for dichotomous and, when adequate, multinomial endpoints.

Documents

Study results

HZNP-RAV-401_Synopsis.pdf(256.93 KB)

Data management

Data sources

Data sources (types)

Disease 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