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)

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Study | Finalised

Administrative details

Contact details

Study institution contact
Åsa Jansson
Study contact

asa.jansson@immedica.com
Primary lead investigator
Åsa Jansson
Primary lead investigator

PURI
https://redirect.ema.europa.eu/resource/30377

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 institution and networks

Institutions

Multiple centres: 5 centres are involved in the study

Networks

E-IMD

Study timelines

Date when funding contract was signed
Actual:
10/10/2016

Data collection
Actual:

07/11/2017

Start date of data analysis
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 and secondary data collection

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 ge

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
100000144759
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

Results tables
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