Orphacol® patient surveillance database (Orphabase)

First published: 16/09/2020

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Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/49962

EU PAS number

EUPAS37229

Study ID

49962

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany
- ☐ Italy

☐ Spain

☐ Switzerland

☐ United Kingdom

Study description

Multi-centre, multi-national, retrospective and prospective observational study to collect patients' clinical information (treatment, safety and efficacy data) of patients receiving Orphacol® for the treatment of inborn errors in primary bile acid synthesis due to 3β-hydroxy-Δ5-C27-steroid oxidoreductase (3β-HSD) deficiency or Δ4-3-oxosteroid 5β-reductase (Δ4-3-oxoR) deficiency.

Study status

Ongoing

Research institutions and networks

Institutions

Laboratoires CTRS (Cell Therapies Research & Services)

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Heidelberg University Hospital

First published: 01/02/2024

Last updated: 01/02/2024

Institution

University Medical Centre Hamburg-Eppendorf

Germany

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Educational Institution | Hospital/Clinic/Other health care facility

Hannover Medical School (MHH)

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Educational Institution | Hospital/Clinic/Other health care facility

Kremlin Bicêtre Hospital, France France, Hôpital La Timone, Marseille France, Hôpital Beaujon, Paris France, Westfällische Wilhelms-Universität Münster Germany, Universitätsklinikum Heidelberg Germany, University Hospital Hamburg-Eppendorf, Hamburg Germany,

Medizinische Hochschule Hannover Germany, Ospedale Infantile Regina Margherita, Torino Italy, Fondazione I.R.C.C.S. Policlinico S.Matteo, Pavia Italy, Azienda Ospedaliera Padova Italy

Contact details

Study institution contact

Theravia Medical Affairs Direction

Study contact

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Primary lead investigator

Theravia Medical Affairs Direction

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 17/11/2006

Actual: 17/11/2006

Study start date

Planned: 20/11/2006

Actual: 20/11/2006

Date of final study report
Planned: 30/09/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Theravia

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)

Regulatory procedure number

Initial marketing authorisation procedure (EU/1/13/870/001-006)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

Increase the amount of available data on the treatment of inborn errors in primary bile acid synthesis due to 3β-HSD deficiency or Δ4-3-OxoR deficiency with Orphacol® in infants, children, adolescents and adults, and especially data on initial efficacy and safety of treatment with cholic acid.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Retrospective and prospective observational study

Study drug and medical condition

Name of medicine

ORPHACOL

Medical condition to be studied

Bile acid synthesis disorder

Additional medical condition(s)

inborn errors in primary bile acid synthesis due to 3β-Hydroxy-Δ5-C27-steroid oxidoreductase deficiency or Δ4-3-Oxosteroid-5β-reductase deficiency

Population studied

Age groups

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

Renal impaired

Hepatic impaired

Immunocompromised

Pregnant women

Estimated number of subjects

100

Study design details

Outcomes

The primary criteria for efficacy evaluation are measurement of blood biochemistry parameters, in particular the levels of aspartate aminotransferase (ASAT), alanine aminotransferase (ALAT), gamma-glutamyl transferase (GGT), alkaline phosphatase (ALP), total bilirubin and Vitamin E.

Data analysis plan

Descriptive statistics are used to describe the included patients.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Retrospective and prospective data collected from patient medical records

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