Orphacol® patient surveillance database (Orphabase)

First published: 16/09/2020

Last updated: 23/04/2024

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 3?-hydroxy-?5-C27-steroid oxidoreductase (3?-HSD) deficiency or ?4-3-oxosteroid 5?-reductase (?4-3-oxoR) deficiency.

Study status
Ongoing

Research institution 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 3?-HSD deficiency or ?4-3-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 3?-Hydroxy-?5-C27-steroid oxidoreductase deficiency or ?4-3-Oxosteroid-5?-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