Kuvan® Adult Maternal Paediatric European Registry (KAMPER)

First published: 20/01/2017

Last updated: 07/02/2022

Study Finalised

Administrative details

EU PAS number

EUPAS17360

Study ID

45633

DARWIN EU® study

No

Study countries

- Austria
- France
- Germany
- Italy
- Netherlands
- Portugal

Slovakia

Spain

Sweden

Study description

Kuvan® is a synthetic copy of a body's own substance called tetrahydrobiopterin (BH4). BH4 is required by the body to use an amino acid called phenylalanine in order to build another substance called tyrosine. Kuvan® received marketed authorisation in Europe in December 2008 and is now available in several European countries for the treatment of Hyperphenylalaninemia (HPA). The primary objective is to assess the long-term safety in subjects treated with Kuvan®. Secondary objectives are to provide additional information regarding: • Safety in specific subject groups (elderly, pediatric, pregnant women and subjects with renal or hepatic insufficiency). • Growth and neurocognitive outcomes for subjects with hyperphenylalaninemia (HPA) who are receiving treatment with Kuvan®. • Progress and outcome of pregnancy for women with HPA who become pregnant while receiving treatment with Kuvan® (these women will be enrolled in a dedicated sub-registry). • Assessment of adherence to diet and to Kuvan®. • Assessment of long-term sensitivity to Kuvan®treatment.

Study status

Finalised

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Institution

Multiple centres: 69 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Kucuksayrac Ece

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 30/04/2009

Study start date
Actual: 08/12/2009

Data analysis start date
Actual: 28/02/2011

Date of interim report, if expected
Planned: 29/06/2020

Actual: 29/06/2020

Date of final study report
Planned: 30/11/2021

Actual: 10/12/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin International Limited

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 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

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Main study objective:

The primary objective is to assess the long-term safety in subjects treated with Kuvan

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational, multicentre, drug registry study

Study drug and medical condition

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

SAPROPTERIN DIHYDROCHLORIDE

Medical condition to be studied

Phenylketonuria

Tetrahydrobiopterin deficiency

Population studied

Short description of the study population

Subjects treated with Kuvan®.

The following inclusion and exclusion criteria must have been fulfilled:

Inclusion criteria

• Adult or paediatric patients (no age limit) of either gender with HPA due to PKU or BH4 deficiency,

• Have been shown to be responsive to BH4 or Kuvan,

• Currently being treated with Kuvan at a participating site,

• Patient or parent/legal guardian willing and able to provide written, signed informed consent and to give consent before any data collection. If a child is old enough to read and write, a separate assent form was given.

Exclusion criteria

• Known hypersensitivity to Kuvan,

• Legal incapacity or limited legal capacity without legal guardian representation,

• Breast-feeding.

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

Renal impaired

Hepatic impaired

Pregnant women

Estimated number of subjects

625

Study design details

Outcomes

The primary endpoint: Incidence and description of Adverse events and Serious Adverse Events (AE/SAEs), Secondary endpoints: Incidence of AEs/SAEs in specific populations elderly (65 years), children and subjects with renal or hepatic insufficiency. Description of somatic growth in BH4 deficient children <3 years, Neurocognitive outcomes, Neurological and psychiatric assessment, Diet and Kuvan treatment adherence, Long-term sensitivity to Kuvan treatment, Pregnancy outcomes and delivery outcomes.

Data analysis plan

Interim analyses will be performed annually. Each interim analysis will include cross-sectional analyses of data regarding the safety and other outcomes such as clinical parameters, laboratory parameters or neurocognitive outcomes. For each variable, descriptive statistics from the current and previous annual analysis will be included in the interim report. Analyses will be descriptive. Confidence intervals will be provided for the primary endpoints and for secondary endpoints when relevant.

Documents

Study results

Abstract KAMPER_Final CSR_final v1.0_10Dec2021_Redacted.pdf (167.48 KB)

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

Data sources (types)

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