A Multi-Center, Observational Study to Evaluate the Long-Term Safety of Subcutaneous Injections of Palynziq® (pegvaliase) in Subjects with Phenylketonuria (PALace)

First published: 21/03/2022

Last updated: 07/05/2024

Study | Ongoing

Administrative details

EU PAS number

EUPAS34992

Study ID

49099

DARWIN EU® study

No

Study countries

Germany

Italy

United States

Study description

This is a 10-year multi-center, global, observational study to further characterize the safety profile of pegvaliase, including hypersensitivity reactions, long-term safety and tolerability, and the effectiveness of the additional risk minimization measures (aRMMs) in subjects receiving pegvaliase for the treatment of PKU. Subjects for whom a clinical decision has been made that they will receive pegvaliase to treat their PKU within 30 days following the date of enrollment (incident-users) or have previously started treatment with pegvaliase at the date of enrollment (prevalent-users) are eligible for participation in this study.

Study status

Ongoing

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Institution

Contact details

Study institution contact

Global Medical Information medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

Program 165-501 - Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/01/2019

Actual: 18/11/2020

Study start date

Planned: 28/02/2022

Actual: 23/06/2022

Data analysis start date

Planned: 09/08/2023

Actual: 24/08/2023

Date of interim report, if expected

Planned: 21/12/2025

Actual: 20/12/2021

Date of final study report

Planned: 31/05/2033

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin Pharmaceutical Inc.

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 type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

To further characterize the safety profile of pegvaliase, including hypersensitivity reactions, long-term safety and tolerability, and the effectiveness of the additional risk minimization measures (aRMM) (European Union EU only) in subjects receiving pegvaliase for the treatment of phenylketonuria (PKU) in a real-world setting.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

PALYNZIQ

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

PEGVALIASE

Anatomical Therapeutic Chemical (ATC) code

(A16AB19) pegvaliase

pegvaliase

Medical condition to be studied

Phenylketonuria

Population studied

Age groups

- 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

Hepatic impaired

Renal impaired

Estimated number of subjects

450

Study design details

Outcomes

To quantify and characterize the risk of the protocol-defined safety events in incident users receiving pegvaliase for the treatment of PKU in a real-world setting

• Acute systemic hypersensitivity reaction

• Anaphylaxis

• Angioedema

• Serum sickness

• Severe hypersensitivity reaction

• Severe or Persistent (≥ 6 months) arthralgia

• Severe injection site reaction

• Hypophenylalaninemia,

Quantify & characterize the risk of:
• Complications of immune-complex formation or PEG accumulation resulting in end-organ damage, SAEs, Severe ADRs, ADRs leading to treatment interruption/discontinuation and/or study discontinuation.
• Safety event in subject: receiving treatment with other PEG injectable with pre-existing hepatic and renal impairment with hypoPhe ≥ 65yr < 16yr (excl. Germany).

Data analysis plan

The primary analysis is the incidence rate for each primary safety event will be calculated as the number of new events divided by the total exposure person-time at risk. The 95% confidence interval of the incidence rate will also be calculated. Additional analyses involving time to first event, incidence proportion, and event rate of each primary safety event will be explored using parametric and semi-parametric modeling. Analysis of the secondary endpoint will include the incidence rate of pre-specified safety events. In addition, incidence rates of each primary safety event and the safety events from the secondary endpoint will be provided within pre-specified subsets. Tertiary analysis will include the number and proportion of subjects in Europe who received aRRMs prior to the initiation of pegvaliase treatment. Tabulations of patient characteristics at enrolment and during the study will also be summarized.

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