A Multicentre, Observational Study to Evaluate the Real-World Outcomes of Palynziq® (Pegvaliase) in Subjects with Phenylketonuria (OPAL)

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Study Ongoing

Administrative details

EU PAS number

EUPAS41426

Study ID

50667

DARWIN EU® study

No

Study countries

- ☐ Germany
- ☐ Italy
- ☐ United States

Study description

A multicentre observational study evaluating data from routine clinical practice in subjects receiving Pegvaliase for the treatment of PKU. Subjects with PKU who are receiving or have been recommended to receive Pegvaliase to treat their PKU are eligible for participation. The study will have an enrolment period of approximately 32 months and a planned subject data collection period of 24 months. Up to 150 subjects will be enrolled across all sites. During the study, relevant medical information will be obtained via review of the subject's medical records. Subjects who consent to participate in the study will be requested to provide relevant PKU disease history, including PKU treatment history, MNT (including medical foods, intact protein foods natural protein sources, and special low protein foods), concomitant medication use, and any patient-centred outcome data collected as part of routine clinical practice. Information on all blood Phe levels available will be collected as well as Pegvaliase dosing and titration information and prescribed dietary protein and MNT.

Study status

Ongoing

Research institutions and networks

Institutions

University of Naples Federico II

First published: 01/02/2024

Last updated: 01/02/2024

Institution

University of Ulm

Germany

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Educational 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

The Medical College of Wisconsin

First published: 01/02/2024

Last updated: 01/02/2024

Institution

BioMarin Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Universitätsklinikum Schleswig-Holstein

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Institution

Universitätsklinikum Münster (UKM) Munster, Germany

Kreiskliniken Reutlingen Reutlingen, Germany

Universität Leipzig Leipzig, Germany

Universität Gießen Giessen, Germany

Hospital Carl-Thiem-Klinikum Cottbus Cottbus, Germany

Universitätsklinikum Düsseldorf Dusseldorf, Germany

Klinikum Bremen Mitte Bremen,Germany

Universitäasklinikum Zentrum fur Kinder und

Jugenmedizin Mainz, Germany

Policlinico S.Orsola Malpighi, AOU di Bologna Bologna, Italy

Ospedale San Paolo Milano, Italy

University of Kentucky Lexington, KY, United States

St. Christopher's Hospital for Children Philadelphia, PA, United States

Oregon Health & Science University Portland, OR, United States

Indiana University School of Medicine Indianapolis, IN, United States

Children's Hospital of Pittsburgh Pittsburgh, PA, United States

University of Texas Southwestern Medical Center Dallas, TX, United States

University of Texas Houston Medical School Houston, TX, United States

Emory University Decatur, Georgia, United States

Children's Hospital Colorado Aurora, Colorado, United States

Tulane University Medical Center New Orleans, LA, United States

Ann and Robert H. Lurie Children's Hospital of Chicago Chicago, IL, United States

University of Florida (Gainesville) Gainesville, FL, United States

Contact details

Study institution contact

165-508 Program Director medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

165-508 Program Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 09/04/2021

Study start date
Actual: 20/05/2021

Date of final study report
Planned: 31/07/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

Provide real-world evidence data on use of Palynziq in PKU

Main study objective:

The efficacy and safety of Pegvaliase has been established in clinical trials as required for regulatory approval. There remains an important need to provide data to prescribers and the phenylketonuria (PKU) community on the real-world usage of Pegvaliase and associated outcomes to support clinical practice optimisation and provide greater understanding of important clinical issues in PKU.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational study on routine clinical practice

Study drug and medical condition

Name of medicine

PALYNZIQ

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

Pregnant women

Estimated number of subjects

150

Study design details

Outcomes

To evaluate outcomes achieved with Pegvaliase in a real-world setting as measured by blood phenylalanine (Phe) level over time. Describe: dosing intervals and dosing of Pegvaliase in each phase of treatment and length of

titration phase, reported MNT in each treatment phase, relationship between changes in reported MNT and blood Phe, co-medications of interest used in each treatment phase, impact of blood Phe on subjects' wellbeing and health-related Quality of Life and impact of blood Phe on socioeconomic parameters.

Data analysis plan

Data will be collected from patients who are receiving Pegvaliase as part of their routine clinical care undertaken by their PKU treating physician. No formal hypothesis testing or inference will be made. The study will use descriptive summary statistics and various statistical analysis methods for longitudinal, repeated measures data for estimation and summarisation of outcomes, including statistical modelling and graphical methods.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection, Site’s routine standard of care patient notes

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