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

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Last updated: 20/06/2024

Study | Ongoing

Administrative details

PURI
https://redirect.ema.europa.eu/resource/50667

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 institution 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
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Institution

BioMarin Pharmaceuticals

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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
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
200000005139
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