A Global, Multicenter Study to Assess Maternal, Fetal and Infant Outcomes of Exposure to Palynziq® (pegvaliase) During Pregnancy and Breastfeeding (PALomino)

First published: 05/12/2022

Last updated: 17/06/2024

Study

Ongoing

Administrative details

EU PAS number

EUPAS35156

Study ID

50385

DARWIN EU® study

No

Study countries

- [ ] Canada
- [ ] Germany
- [ ] Italy

United States

Study description

This is an observational, prospective surveillance study of subjects with Phenylketonuria (PKU) exposed to pegvaliase during pregnancy. No study medication is provided as part of participation and all direction for medication usage is at the discretion of the prescribing physician in accordance with standard practice and the local label. The assignment of a subject to pegvaliase is not decided in advance or influenced by the study protocol, and the decision to prescribe pegvaliase is independent of the decision to include the subject in the study. Retrospective data collection of pegvaliase exposure and disease data will be collected for at least 3 months prior to Last Menstrual Period (LMP). Pegvaliase exposure will also be recorded during pregnancy and breastfeeding including exposure during each trimester of pregnancy. For these measures, first trimester exposure will be defined as any dose between 2 weeks prior to the first day of LMP and 13 weeks gestation, second trimester as 14 weeks through 27 weeks gestation, and third trimester as 28 weeks gestation onwards. Additionally, ongoing pegvaliase exposure will be recorded among subjects with live-birth outcomes who initiate breastfeeding, as long as breastfeeding continues up to (and not past) infant age of 12 months.

Study status

Ongoing

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

165-504 Global Medical Information medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

Program 165-504 Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/01/2020

Actual: 08/06/2020

Study start date

Planned: 01/12/2022

Actual: 22/11/2022

Data analysis start date

Planned: 31/08/2025

Date of interim report, if expected
Planned: 15/12/2025

Actual: 21/12/2021

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:

The purpose of this observational study is to further increase knowledge about the outcomes of pregnant women with PKU and their offspring exposed to pegvaliase during pregnancy and breastfeeding.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

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)

Special population of interest

Pregnant women

Estimated number of subjects

70

Study design details

Outcomes

Estimate the frequency of pregnancy outcomes (eg,spontaneous abortion,stillbirth,live birth,and termination) among subjects with PKU treated with pegvaliase during pregnancy and fetal/infant outcomes (all major congenital malformations and specifically microcephaly and congenital heart defects, FGR, small for gestational age,low birth weight,preterm birth,failure to thrive,and developmental delays, Pregnancy/infant outcomes among pegvaliase treated/untreated. Differences in pregnancy/infant outcomes by maternal Phe level. SAEs other than congenital malformations (CMs) in infants through their first year of life. Outcomes of subjects treated with pegvaliase during breastfeeding (low milk supply) and their infants (failure to thrive and SAEs) through their first year of life.

Data analysis plan

The primary analysis is the prevalence of pregnancy outcomes, including major congenital malformations. A 95% exact confidence interval of the prevalence will also be calculated. Results from each pregnancy outcome will also be descriptively compared with published data on outcomes in non-pegvaliase exposed PKU pregnancies and, when appropriate, general population reference literature. Analysis of secondary endpoints will include prevalence of each pregnancy outcome by varying blood Phe concentration levels. In addition, the number and incidence of infant SAEs and infants who meet the failure to thrive criteria will be provided. The number and incidence of low milk supply among breastfeeding women will be examined as well. Additional analysis will include the number and proportion of major congenital malformations, infant hospitalization, and infant death. Tabulations of patient (and infant) characteristics at enrolment (and at birth) and during the study will 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