

AN OBSERVATIONAL, LONGITUDINAL, PROSPECTIVE, LONG-TERM REGISTRY OF PATIENTS WITH HYPOPHOSPHATASIA (ALX- HPP-501)

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Study

Ongoing

Administrative details

PURI

<https://redirect.ema.europa.eu/resource/47907>

EU PAS number

EUPAS13514

Study ID

47907

DARWIN EU® study

No

Study countries

- ☐ Australia
 - ☐ Austria
 - ☐ Canada
 - ☐ France
 - ☐ Germany
 - ☐ Italy
 - ☐ Japan
 - ☐ Poland
 - ☐ Saudi Arabia
 - ☐ Spain
 - ☐ United Kingdom
 - ☐ United States
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Study description

HPP is a rare disease that has historically been largely treated symptomatically. Only one therapy designed to treat the underlying cause of the disease (Strensiq® asfotase alfa) has been approved for commercial use. Due to the rare nature of this disease, and considering the lack of information regarding diagnosis patterns and health care management in a “real world” setting, this study will collect data on epidemiology, HPP history, genetics (ALPL variants) clinical course, symptoms (including systemic aspects of the disease), and burden of disease from patients of any age who have a diagnosis of HPP, including patients who are either untreated or receiving treatment for HPP. For patients treated with asfotase alfa, the Registry collects data on asfotase alfa dosing, effectiveness of treatment, serious adverse events (SAEs), immunogenicity, pregnancy and neonatal outcome data, and pre-defined targeted events. Accordingly, the Registry will permit better delineation between the natural disease course of HPP and the disease course in patients who are treated.

Study status

Ongoing

Contact details

Study institution contact

Anna Petryk

Study contact

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Primary lead investigator

Anna Petryk

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/07/2014

Actual: 31/07/2014

Study start date

Planned: 20/01/2015

Actual: 20/01/2015

Date of final study report

Planned: 28/08/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alexion Pharmaceuticals, Inc.

Study protocol

[Asfotase alfa_ALX-HPP-501 Protocol Amendment 4.2_06May2016_Final.pdf](#)

(586.83 KB)

[ALX-HPP-501 PA 6.8_EU exc Germany Final_24Nov2020_signed.pdf](#)(474.78 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Drug utilisation

Effectiveness study (incl. comparative)

Main study objective:

To collect information on the natural history of hypophosphatasia (HPP) from patients of all ages, including infants, children, and adults with HPP, regardless of age at onset. To characterize the epidemiology of the HPP population. To collect and evaluate long-term safety and effectiveness data in HPP patients who have/are receiving treatment with asfotase alfa.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Longitudinal observational registry

Study drug and medical condition

Name of medicine

STRENSIQ

Medical condition to be studied

Hypophosphatasia

Population studied

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)

Estimated number of subjects

1100

Study design details

Data analysis plan

Prior to the conduct of data analysis each year, details of planned analyses and patient cohorts will be prespecified in an a priori Epidemiological and Statistical Analysis Plan (ESAP). Categorical variables will be described using frequencies and percentages and modeled using logistic regression, while continuous variables will be described using means, standard deviations, medians, and inter-quartile ranges with modeling accomplished through generalized linear models, where appropriate. Study results will be summarized and reviewed at

appropriate intervals based on patient enrollment, scientific considerations, and regulatory requirements. At a minimum, study results will be summarized annually and reported, and may include patient and clinical characteristics, as well as safety and effectiveness outcomes. Following termination of the Registry, a final analysis and report will also be prepared.

Documents

Study publications

[Martos-Moreno GÁ, Linglart A, Petryk A, Kishnani PS, Rockman-Greenberg C, Dahir...](#)

[Seefried L, Dahir K, Petryk A, Högler W, Linglart A, Martos-Moreno GÁ, Ozono K,...](#)

[Högler W, Langman C, Gomes da Silva H, Fang S, Linglart A, Ozono K, Petryk A, R...](#)

[Linglart et al. Frequency of ectopic calcifications among patients with hypopho...](#)

[Dahir KM, Angel MM, Linglart A, Petryk A, Kishnani PS, Rockman-Greenberg C, Mar...](#)

Data management

Data sources

Data sources (types)

[Other](#)

Data sources (types), other

Disease registry, Subject medical records

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