

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

**First published:** 20/05/2016

**Last updated:** 24/06/2025

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS13514

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### Study ID

47907

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### DARWIN EU® study

No










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### 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.

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## **Study status**

Ongoing

## Contact details

### Study institution contact

Anna Petryk [adeline.merlet@alexion.com](mailto:adeline.merlet@alexion.com)

Study contact

[adeline.merlet@alexion.com](mailto:adeline.merlet@alexion.com)

### Primary lead investigator

Anna Petryk

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 31/07/2014

Actual: 31/07/2014

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### Study start date

Planned: 20/01/2015

Actual: 20/01/2015

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### 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

[AA ALX-HPP-501 redacted Protocol Amendment 4.2\\_06May2016\\_Final.pdf](#)  
(693.24 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

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### **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

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**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

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**Non-interventional study design, other**

Longitudinal observational registry

## Study drug and medical condition

**Medicinal product name**

STRENSIQ

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**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)
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## **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...

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## 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

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### **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

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No