X-Linked Hypophosphatemia (XLH) Life Course Analysis: A Retrospective Cross-Sectional Study

First published: 04/12/2019

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Study Finalised

Administrative details

EU PAS number

EUPAS32590

Study ID

44131

DARWIN EU® study

No

Study countries

United Arab Emirates

Study description

The objective of this study is to undertake a life course analysis of X-Linked Hyophosphatemia (XLH) in adulthood (from 18 years old onwards) in order to gain an understanding of adult disease manifestations, reported healthcare resource use and patient reported quality over the XLH life course.

Study status

Finalised

Research institutions and networks

Institutions

Kyowa Kirin

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Institution

Contact details

Study institution contact

Study Director Kyowa Kirin
RWEtransparency@kyowakirin.com

Study contact

RWEtransparency@kyowakirin.com

Primary lead investigator

Study Director Kyowa Kirin

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 03/06/2019

Actual: 02/09/2019

Study start date
Planned: 01/10/2019

Actual: 07/10/2019

Date of final study report
Planned: 01/12/2020

Actual: 01/11/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Kyowa Kirin

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 topic:

Disease /health condition

Other

Study topic, other:

Disease/Epidemiology study

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Data collection methods:

Primary data collection

Main study objective:

The objective of this study is to undertake a life course analysis of XLH in adulthood (from 18 years old onwards) in order to gain an understanding of adult disease manifestations, reported healthcare resource use and patient reported quality over the XLH life course

Study Design

Non-interventional study design

Cross-sectional

Study drug and medical condition

Medical condition to be studied

Hereditary hypophosphataemic rickets

Population studied

Short description of the study population

Adults aged 18 to 65 years with a diagnosis of XLH supported by clinical and biochemical features consistent with the disease, and/or a confirmed PHEX mutation in the adult or a family member, were included. To be eligible, adults also needed to have skeletal pain, defined as a Brief Pain Inventory (BPI) worst pain score ≥ 4 in the last 24 hours prior to screening, attributed to XLH/osteomalacia

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Age groups

- 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

Other

Special population of interest, other

Patients with X-Linked Hypophosphatemia

Estimated number of subjects

336

Study design details

Outcomes

1. Adult manifestations of XLH (fractures and associated specific co-morbidities) 2. Healthcare resource use (according to recordings of analgesia and orthopaedic surgery) 3. Quality of life, according to patient reported outcomes

Data analysis plan

A descriptive analysis will be conducted on the two patient populations. The number and percentage of different disease manifestations for patients with XLH in each age cohort (18-29, 30-39, 40-49, 50-59 and 60+years) will be calculated. Categorical data (e.g. yes/no responses) will be summarized for each study and age cohort using simple tables of counts and percentages and/or Forest plots which will include the associated 95% confidence interval, calculated using the Wilson method (Altman et al. 2000). Continuous data (e.g. age at diagnosis) will be summarized for each study and age cohort using

tables of summary statistics and / or Box plots, which will show the mean, median and quartiles, as well as extreme observations. All analysis will be conducted in Statistical Analysis Software (SAS®).

Documents

Study publications

Javaid MK, Ward L, Pinedo-Villanueva R, Rylands AJ, Williams A, Insogna K, Imel...

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, The second dataset is taken from an existing clinical trial

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