A national register based study examining the prevalence, comorbidities, healthcare resource utilisation and burden of illness of hereditary hypophosphatemia in Demark

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Study Planned

Administrative details

EU PAS number

EUPAS44133

Study ID

44134

DARWIN EU® study

No

Study countries

Denmark

Study description

The aims of the current study are twofold 1. first to examine the incidence and prevalence of a confirmed diagnosis of hereditary hypophosphatemia in Denmark across the life span and 2. secondly to understand the life course of the disease and the use of healthcare resources and social benefits compared to a reference population (called the control population). The control population will be used as a baseline and will serve as a comparator to the population with a confirmed diagnosis of hereditary hypophosphatemia (called the case population). We hypothesize that patients with hereditary hypophosphatemia will have progressing and accumulating comorbidities and an increased need for health care services and social benefits compared to the control population, causing a higher level of disease burden.

Study status

Planned

Research institutions and networks

Institutions

Kyowa Kirin

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Institution

Contact details

Study institution contact

Study Director Kyowa Kirin
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Study contact

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Primary lead investigator

Study Director Kyowa Kirin

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 01/06/2021

Actual: 01/11/2021

Study start date
Planned: 01/12/2021

Date of final study report
Planned: 01/11/2022

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 type:

Non-interventional study

Scope of the study:

Disease epidemiology

Main study objective:

The objective of this study identify the prevalence and incidence of a confirmed diagnosis of hereditary hypophosphatemia.

Study Design

Non-interventional study design

Case-control

Study drug and medical condition

Medical condition to be studied

Hereditary hypophosphataemic rickets

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

100

Study design details

Outcomes

To identify the prevalence and incidence of a confirmed diagnosis of hereditary hypophosphatemia. To examine the natural history of a patient population with a confirmed diagnosis of hereditary hypophosphatemia (called the case population), in detail, to understand the life course of the disease and the use of healthcare resource and social benefits, key co-morbidities and other clinical

outcomes and mortality across different age cohorts.

Data analysis plan

This is a case-controlled study with a control population (the control population is the case population multiplied by 50 and matched by gender and year of birth). The two populations will be compared according to age groups for comorbidity, burden of disease and costs to the public. Data will be described according to the number of unique persons in each stratum of the subpopulation. Descriptive statistics will be calculated using Paired t-test, Wilcoxon signed ranks test, McNemars test, optionally time rank analysis as well as Kaplan-Meier estimator. Data will be presented as mean and standard deviation (SD) or median (interquartile range IQR or range) as appropriate. Number of events and percent of the population, as appropriate.

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 source(s)

Danish registries (access/analysis)

Data source(s), other

Danish Registries (access/analysis)

Data sources (types)

Administrative healthcare records (e.g., claims)

Disease registry

Drug dispensing/prescription data

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