PedNet Haemophilia registry

First published: 01/02/2024

Last updated: 17/10/2024

Data source | Human | Disease registry | Other

Administrative details

Administrative details

Data source ID

46153

Data source acronym

PHR

Data holder

PedNet Haemophilia Research Foundation

Data source type

Disease registry

Other

Data source type, other

Exposure registry, Prospective studies database

Main financial support

Funding from industry or contract research

Care setting

Hospital inpatient care

Hospital outpatient care

Data source qualification

If the data source has successfully undergone a formal qualification process (e.g., from the EMA, ISO or other certifications), this should be described.

No

Data source website

https://www.pednet.eu

Contact details

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Main

info@pednet.eu

Gili Kenet info@pednet.eu

Alternate

info@pednet.eu

Data source regions and languages

Data source countries

Austria

Belgium

Canada

Czechia

Denmark

Finland

France

Germany

Greece

Israel

Italy

Netherlands

Norway

Portugal

Spain

Sweden

Switzerland

United Kingdom (Northern Ireland)

Data source languages

English

Data source establishment

Data source established

01/01/2000

Data source time span

First collection: 01/01/2000

The date when data started to be collected or extracted.

Publications

Data source publications

Labarque V, Mancuso ME, Kartal-Kaess M, Ljung R, S. Mikkelsen T, G. Andersson N. F8/F9 variants in the population-based PedNet Registry cohort compared with locus-specific genetic databases of the European Association for Haemophilia and Allied Disorders and the Centers for Disease Control and Prevention Hemophilia A or Hemophilia B Mutation

Fischer K, Ljung R, Platokouki H, Liesner R, Claeyssens -Donadel S, Smink E, van den Berg HM. Prospective observational cohort studies for studying rare diseases: the European PedNet Haemophilia Registry

Ljung R, de Kovel M, van den Berg HM, on behalf of the PedNet study group. Primary prophylaxis in children with severe haemophilia A and B – Implementation over the last 20 years as illustrated in real-world data in the PedNet cohorts

Gouw SC, van den Berg HM, Fischer K, Auerswald G, Carcao M, Chalmers E, Chambost H, Kurnik K, Liesner R, Petrini P, Platokouki H, Altisent C, Oldenburg J, Nolan B, Garrido RP, Mancuso ME, Rafowicz A, Williams M, Clausen N, Middelburg RA, Ljung R, van der Bom JG PedNet and Research of Determinants of Inhibitor development (RODIN) Study Group. Intensity of factor VIII treatment and inhibitor development in children with severe hemophilia A: the RODIN study.

Male C, Andersson NG, Rafowicz A, Liesner R, Kurnik K, Fischer K, Platokouki H, Santagostino E, Chambost H, Nolan, B, Königs C, Kenet G, Ljung R, van den Berg HM. Inhibitor Incidence In An Unselected Cohort Of Previously Untreated Patients With Severe Haemophilia B: A PedNet Study

Studies

List of studies that have been conducted using the data source

NN7999-4413: Adverse Event Data Collection from External Registries on Nonacog Beta Pegol

Emicizumab Use in Pediatric Patients in the Real World: an Analysis of the PedNet Registry

Establish an EU catalogue of sources of real-world data, characterised by a common set of metadata and data quality measurements

Eptacog Beta post marketing safety surveillance using the PedNet registry (F7TG2207 – PedNet Registry)

Data elements collected

The data source contains the following information

Disease information

Does the data source collect information with a focus on a specific disease? This might be a patient registry or other similar initiatives.

Yes

Disease details (other)

For Haemophilia A and B: details on treatment, joint health status, adverse events, quality of life, baseline measurements

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Rare diseases

Are rare diseases captured? In the European Union a rare disease is one that affects no more than 5 people in 10,000.

Yes

Pregnancy and/or neonates

Does the data source collect information on pregnant women and/or neonatal subpopulation (under 28 days of age)?

Yes

Hospital admission and/or discharge

Yes

ICU admission

Is information on intensive care unit admission available?

No

Cause of death

Captured

Cause of death vocabulary

Not coded (Free text)

Prescriptions of medicines

Captured

Dispensing of medicines

Not Captured

Advanced therapy medicinal products (ATMP)

Is information on advanced therapy medicinal products included? A medicinal product for human use that is either a gene therapy medicinal product, a somatic cell therapy product or a tissue

engineered products as defined in Regulation (EC) No 1394/2007 [Reg (EC) No 1394/2007 Art 1(1)].

No

Contraception

Is information on the use of any type of contraception (oral, injectable, devices etc.) available?

No

Indication for use

Does the data source capture information on the therapeutic indication for the use of medicinal products?

Captured

Indication vocabulary

Not coded (Free text)

Medical devices

Is information on medicinal devices (e.g., pens, syringes, inhalers) available?

No

Administration of vaccines

No

Procedures

Does the data source capture information on procedures (e.g., diagnostic tests, therapeutic, surgical interventions)?

Captured

Procedures vocabulary

Not coded (Free text)

Healthcare provider

Is information on the person providing healthcare (e.g., physician, pharmacist, specialist) available? The healthcare provider refers to individual health professionals or a health facility organisation licensed to provide health care diagnosis and treatment services including medication, surgery and medical devices.

Yes

Clinical measurements

Is information on clinical measurements (e.g., BMI, blood pressure, height) available?

Yes

Genetic data

Are data related to genotyping, genome sequencing available?

Captured

Genetic data vocabulary

Other

Genetic data vocabulary, other

non-specified/according to local laboratory

Biomarker data

Does the data source capture biomarker information? The term “biomarker” refers to a broad subcategory of medical signs (objective indications of medical state observed from outside the patient), which can be measured accurately and reproducibly. For example, haematological assays, infectious disease markers or metabolomic biomarkers.

Captured

Biomarker data vocabulary

Other

Biomarker vocabulary, other

according to local laboratory

Patient-reported outcomes

Is information on patient-reported outcomes (e.g., quality of life) available?

Yes

Patient-generated data

Is patient-generated information (e.g., from wearable devices) available?

No

Units of healthcare utilisation

Are units of healthcare utilisation (e.g., number of visits to GP per year, number of hospital days) available or can they be derived? Units of healthcare utilisation refer to the quantification of the use of services for the purpose of preventing or curing health problems.

Yes

Unique identifier for persons

Are patients uniquely identified in the data source?

Yes

Diagnostic codes

Captured

Diagnosis / medical event vocabulary

Not coded (Free text)

Medicinal product information

Captured

Medicinal product information collected

Brand name

Formulation

Strength

Dose

Dosage regime

Medicinal product vocabulary

ART 57

SPN

Quality of life measurements

Captured

Quality of life measurements vocabulary

EQ5D

Lifestyle factors

Not Captured

Sociodemographic information

Captured

Sociodemographic information collected

Age

Gender

Country of origin

Quantitative descriptors

Population Qualitative Data

Population age groups

Paediatric Population (< 18 years)

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)

Estimated percentage of the population covered by the data source in the catchment area

95% in participating centres.

Description of the population covered by the data source in the catchment area whose data are not collected (e.g., people who are registered only for private care)

refused-consented patients. Patients outside study area.

Family linkage

Family linkage available in the data source permanently or can be created on an ad hoc basis

Ad hoc

Population

Population size

2759

Active population size

1902

Population by age group

Age group	Population size	Active population size
Paediatric Population (< 18 years)	2759	1902

Median observation time

Median time (years) between first and last available records for unique individuals captured in the data source

18.00

Median time (years) between first and last available records for unique active individuals (alive and currently registered) capt

18.00

Data flows and management

Access and validation

Governance details

Documents or webpages that describe the overall governance of the data source and processes and procedures for data capture and management, data quality check and validation results (governing data access or utilisation for research purposes).

https://www.pednet.eu

Biospecimen access

Are biospecimens available in the data source (e.g., tissue samples)?

No

Access to subject details

Can individual patients/practitioners/practices included in the data source be contacted?

Yes

Description of data collection

https://pednet.eu/wp-content/uploads/2023/01/Protocol-of-the-PedNet-Haemophilia-Registry-version-6.4.pdf

Event triggering registration

Event triggering registration of a person in the data source

Disease diagnosis

Event triggering de-registration of a person in the data source

Practice deregistration

Loss to follow up

Death

Other

Event triggering de-registration of a person in the data source, other

becoming of age >18 years

Event triggering creation of a record in the data source

Specialist encounter on each regular visit to the centre.

Data source linkage

Linkage

Is the data source described created by the linkage of other data sources (prelinked data source) and/or can the data source be linked to other data source on an ad-hoc basis?

No

Data management specifications that apply for the data source

Data source refresh

Yearly

Informed consent for use of data for research

Other

Possibility of data validation

Can validity of the data in the data source be verified (e.g., access to original medical charts)?

Yes

Data source preservation

Are records preserved in the data source indefinitely?

Yes

Approval for publication

Is an approval needed for publishing the results of a study using the data source?

Yes

Informed consent, other

There is a committee to evaluate requests for data access

Data source last refresh

01/01/2023

Common Data Model (CDM) mapping

CDM mapping

Has the data source been converted (ETL-ed) to a common data model?

No