# PedNet Haemophilia registry

**First published:** 01/02/2024

**Last updated:** 17/10/2024

Data source | Outdated | Human | Disease registry | Other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)

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**Data source languages**

English

## Data source establishment

**Data source established**

01/01/2000

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**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

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**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

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### Clinical measurements

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

Yes

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### Genetic data

Are data related to genotyping, genome sequencing available?

Captured

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### Genetic data vocabulary

Other

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### Genetic data vocabulary, other

non-specified/according to local laboratory

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### 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

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### Biomarker data vocabulary

Other

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### Biomarker vocabulary, other

according to local laboratory

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### Patient-reported outcomes

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

Yes

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### Patient-generated data

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

No

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### 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

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### Unique identifier for persons

Are patients uniquely identified in the data source?

Yes

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### Diagnostic codes

Captured

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### Diagnosis / medical event vocabulary

Not coded (Free text)

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### Medicinal product information

Captured

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### 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)

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

95% in participating centres.

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**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**

2.759

**Active population size**

1.902

## Population by age group

| Age group | Population size | Active population size |
|---|---|---|
| Paediatric Population (< 18 years) | 2.759 | 1.902 |

## 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

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### Informed consent, other

There is a committee to evaluate requests for data access

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### 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