The World Federation of Hemophilia Gene Therapy Registry

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Data source | Outdated | Human | Other

Administrative details

Administrative details

Data source ID

50044

Data source acronym

The WFH Gene Therapy Registry

Data holder

World Federation of Hemophilia (WFH)

Data source type

Other

Data source type, other

Prospective studies database

Main financial support

Funding from industry or contract research

Care setting

Primary care – specialist level (e.g. paediatricians)

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.

Yes

Description of the qualification

EMA qualification process ongoing

Data source website

https://www.wfh.org

Contact details

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Data source regions and languages

Data source countries

Belgium

France

Germany

Ireland

Italy

Netherlands

Spain

Sweden

Switzerland

United Kingdom

Data source languages

English

Data source establishment

Data source established

15/06/2022

Data source time span

First collection: 15/10/2023

The date when data started to be collected or extracted.

Publications

Data source publications

World Federation of Hemophilia Gene Therapy Registry

Core data set on safety, efficacy, and durability of hemophilia gene therapy for a global registry: Communication from the SSC of the ISTH

Studies

List of studies that have been conducted using the data source

GENEr8-GTR: A Retrospective Cohort Study of Patients Treated with ROCTAVIAN™ (valoctocogene roxaparvovec): An Analysis of Patient Registries (BMN 270-801)

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)

Haemophilia A and B

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)?

No

Hospital admission and/or discharge

No

ICU admission

Is information on intensive care unit admission available?

No

Cause of death

Captured

Cause of death vocabulary

MedDRA

Prescriptions of medicines

Captured

Prescriptions vocabulary

other

Prescriptions vocabulary, other

WHODrug

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?

Not Captured

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)

Other

Procedures vocabulary, other

The data source has a dropdown list of surgeries: Abdominal surgery Orthopedic surgery Dental procedure Central device Neurosurgery Other Unknown

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.

No

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?

Not Captured

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.

Not Captured

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?

Yes

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.

No

Unique identifier for persons

Are patients uniquely identified in the data source?

No

Diagnostic codes

Captured

Diagnosis / medical event vocabulary

MedDRA

Medicinal product information

Captured

Medicinal product information collected

Batch number

Brand name

Dose

Medicinal product vocabulary

WHO Drug

Quality of life measurements

Captured

Quality of life measurements vocabulary

EQ5D

other

Quality of life measurements, other

PROBE, coreHEM MH

Lifestyle factors

Captured

Lifestyle factors

Alcohol use

Sociodemographic information

Captured

Sociodemographic information collected

Age

Ethnicity

Gender

Quantitative descriptors

Population Qualitative Data

Population age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (≥ 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

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

Data collection has not started. The data source is aiming to collect data on 100% of patients who receive gene therapy for hemophilia. Gene therapy is currently only available to adults (18+).

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)

Nation-wide

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.dropbox.com/s/sy6j4xs60vxpfr2/5.%20WFH%20GTR%20Governance_22June2

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

Data will be collected by physicians and entered in the electronic Data Capture Tool. Patients will provide patient-reported outcome data via the patient engagement tool, myGTR.

Event triggering registration

Event triggering registration of a person in the data source

Start of treatment

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

Death

Loss to follow up

Other

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

Request to de-register

Event triggering creation of a record in the data source

If a patient receives gene therapy for hemophilia they are elligibile to participate in the registry and will be invited. Data will be collected at months 3, 6, 9, 12, 18, 24, and annually thereafter. Adverse events can be entered in the database at any time.

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?

Yes

Linkage description, possible linkage

Through a data linkage protocol. The data source does not link different records for patients already in the system (myGTR). New patients, not otherwise included in the system, can be included through data transfer from their national registry.

Linked data sources

Pre linked

Is the data source described created by the linkage of other data sources?

No

Data source, other

Any national hemophilia registry collecting data on patients who receive gene therapy can link with the GTR.

Linkage strategy

Other

Linkage variable

Unique patient identifiers will be used to ensure no duplication of patients.

Linkage completeness

Completeness of linkages cannot be provided at this moment. The data source expects a high percentage for completeness as the data set was shared with

several national registries who will transfer data.

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?

No

Informed consent, other

There is a committee to evaluate requests for data access

Common Data Model (CDM) mapping

CDM mapping

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

No