A post-authorization study to assess the safety and efficacy of Fanhdi (Double-inactivated human anti-hemophilic factor) in subjects with Von Willebrand disease (PostAuthorization Study with Fanhdi in VWD patient)

First published: 02/11/2018

Last updated: 22/02/2024

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/49679

EU PAS number

EUPAS25809

Study ID

49679

DARWIN EU® study

No

Study countries

Spain

Study description

Multicenter, observational, prospective, post-authorization cohort study done in subjects with von Willebrand disease.

Study status

Finalised

Research institutions and networks

Institutions

University Hospital Vall d’Hebron (HUVH)

Spain

First published: 01/02/2024

Last updated: 01/02/2024

Institution | **Educational Institution** | **Hospital/Clinic/Other health care facility**

Clinical Pharmacology, Vall d'Hebron Institut de Recerca (VHIR)

Spain

First published: 18/05/2021

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Institution | Hospital/Clinic/Other health care facility | ENCePP partner

Hospital la Paz Madrid, Hospital Arnau de Vilanova Lleida, Hospital Carlos Haya Málaga, Hospital Virgen del Rocío Sevilla, Complejo Hospitalario de Jaén Jaén, Hospital de Guadalajara Guadalajara, Hospital Santa Creu i Sant Pau Barcelona

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Olga Benítez

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 31/10/2018

Actual: 31/10/2018

Study start date
Planned: 15/02/2019

Actual: 20/03/2019

Data analysis start date
Planned: 26/05/2022

Date of final study report
Planned: 28/02/2023

Actual: 09/03/2023

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Instituto Grifols, S.A.

Study protocol

IG1403 Protocol V3.0 2018.05.18.pdf(872.73 KB)

Protocolo v 4.0 (2021_01_20)_redacted.pdf(7.88 MB)

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Main study objective:

To evaluate the safety (immunogenicity and thrombogenicity) associated with long term use of Fanhdi.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Phase 4, observational, multi-center, prospective, post-authorization study

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(B02BD06) von Willebrand factor and coagulation factor VIII in combination

von Willebrand factor and coagulation factor VIII in combination

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Medical condition to be studied

Von Willebrand's disease

Population studied

Short description of the study population

Subjects aged 18 years or older diagnosed with Von Willebrand's disease (VWD) treated with Fanhdi under routine clinical practice.

Inclusion criteria:

1. Male and female subjects ≥18 years of age diagnosed with hereditary VWD of any type and severity who require replacement therapy with VWD/FVIII concentrates when desmopressin (DDAVP) treatment alone is ineffective or contra-indicated.
2. Subjects with a history of receiving prior treatment with VWF concentrates due to bleeding episodes and/or surgery or invasive procedures (on demand prophylaxis).
3. Subjects who are expected to experience bleeding episodes and/or surgeries or invasive active bleeding at the time of inclusion.
4. Subjects who are willing and able to provide written informed consent or have an authorized representative able to provide written informed consent on behalf of the subject in accordance with local law and institutional policy.

Exclusion criteria:
1. Subjects diagnosed with acquired VWD.
2. Subjects with a congenital or acquired platelet function disorder or other concomitant processes that may interfere with coagulation.
3. Subjects who are positive for anti-VWF or anti-FVIII antibodies (≥0.5 Bethesda units) or has been positive in the history of their disease.
4. Subjects with a known intolerance to any substance contained in Fanhdi.
5. Subjects with a history of anaphylactic reactions to blood or blood components.
6. Subjects who are participating in another clinical study involving an investigational treatment or have participated in one in the past 4 weeks.
7. Subjects who, in the opinion of investigator, may have compliance problems with the protocol.

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 Von Willebrand's disease

Estimated number of subjects

15

Study design details

Outcomes

* Adverse events including SAEs and suspected adverse drug reactions (ADRs) * Clinical laboratory values including inhibitor (immunogenicity) and functional activity testing * Thrombogenicity assessment * Vital signs * Physical examination, * Bleeding duration and severity * Investigator’s qualitative assessment of hemostasis * Amount of Fanhdi (IU/kg) used per subject, per year and per infusión * Use of other hemoderivatives per bleeding episode * Overall clinical efficacy

Data analysis plan

The safety analyses will be addressed by listing and tabulation of AEs (includes suspected ADRs), clinical laboratory tests including inhibitor (immunogenicity) and functional activity testing, thrombogenicity, vital signs, and physical examinations. Data will be described using descriptive analyses.

Documents

Study results

2-synopsis_redacted.pdf(1.2 MB)

Data management

Data sources

Data sources (types)

Administrative healthcare records (e.g., claims)

Disease registry

Other

Data sources (types), other

Prospective patient-based data collection, Prescription event monitoring

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