A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment for People with Severe Von Willebrand Disease (VWD) (ATHN 9: Severe VWD Natural History Study)

First published: 09/04/2020

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Study Ongoing

Administrative details

EU PAS number

EUPAS34614

Study ID

34615

DARWIN EU® study

No

Study countries

☐ United States

Study description

The overarching objective of this longitudinal, observational and prospective study is to characterize the safety and effectiveness of factor replacement in participants with clinically severe congenital VWD (VWF:Ag, VWF:GPIbM or VWF:RCo of ≤30% or ≤40% of normal with severe bleeding phenotype defined as requiring recurrent use of factor concentrates) enrolled in the American Thrombosis and Hemostasis Network (ATHN) ATHNdataset. This is a longitudinal, observational cohort study being conducted at up to at least 30 ATHN-affiliated sites. Participants will be followed for 2 years from time of study enrollment. The total study duration is 3 years. The primary objective is to assess the safety of various VWF treatment regimens for different indications (on-demand, surgery and prophylaxis) in adult and pediatric participants with clinically severe congenital VWD. Safety will be measured by the number of reported events defined by the European Haemophilia Safety Surveillance (EUHASS) program. All treatment regimens will be at the discretion of the participant's hemophilia healthcare providers. Investigators will emphasize that clinical care and participation in the study is not determined based on their selection of clotting factor replacement or non-factor products. No treatment will be provided by the study. All study visits, procedures and follow-up will be timed to coincide with routine, scheduled bleeding disorder care whenever possible.

Study status

Ongoing

Research institutions and networks

Institutions

American Thrombosis and Hemostasis Network (ATHN)

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Robert Sidonio

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 14/12/2017

Study start date

Actual: 04/09/2019

Data analysis start date
Planned: 29/08/2020

Date of interim report, if expected
Planned: 29/12/2020

Date of final study report
Planned: 20/06/2023

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Takeda

Regulatory

Was the study required by a regulatory body?

No

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

Not applicable

Other study registration identification numbers and links

ClinicalTrials.gov Identifier: NCT03853486

Methodological aspects

Study type

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

To assess the safety of various Von Willebrand Factor (VWF) regimens for different indications (on-demand, surgery and prophylaxis) in adult and pediatric participants with clinically severe congenital VWD.

Main study objective:

To assess the safety of various Von Willebrand Factor (VWF) regimens for different indications (on-demand, surgery and prophylaxis) in adult and pediatric participants with clinically severe congenital VWD.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medical condition to be studied

Von Willebrand's disease

Population studied

Age groups

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

130

Study design details

Outcomes

Safety will be measured by the number of reported events as defined by the European Haemophilia Safety Surveillance (EUHASS) program. -standardized diagnostic battery using an VWF assay, and genetic sequence analysis of VWF coding regions and adjacent non-coding regions.-establish a platform for sub-studies -evaluate the use of factor replacement as prophylaxis -describe bleeding events and annualized bleeding rate -describe effectiveness of VWD treatment as measured by:Health care utilization and Quality of Life

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Data analysis plan

Descriptive statistics will be calculated to analyze the primary and secondary outcomes. Most of the study outcome variables are discrete in nature, such as mortality, newly developed inhibitor, bleeding rate, etc. Some outcome

measurements will be treated as continuous, like health-related quality of life. For each categorical variable, its frequency and percentage will be reported. In terms of a continuous measurement, its mean, median, standard deviation, interquartile range, minimum, and maximum values will be disclosed. During the course of the study, the Steering Committee will evaluate the appropriateness of various statistical approaches based on the amount and quality of data collected.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection

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