

# 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

**Last updated:** 14/03/2024

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS34614

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### Study ID

34615

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### DARWIN EU® study

No

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### Study countries

 United States

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## **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  $\leq 30\%$  or  $\leq 40\%$  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.

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## **Study status**

Ongoing

## **Research institutions and networks**

### **Institutions**

# American Thrombosis and Hemostasis Network (ATHN)

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

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**Institution**

## Contact details

### Study institution contact

Robert Sidonio [nhirsh@athn.org](mailto:nhirsh@athn.org)

**Study contact**

[nhirsh@athn.org](mailto:nhirsh@athn.org)

### Primary lead investigator

Robert Sidonio

**Primary lead investigator**

## Study timelines

### Date when funding contract was signed

Actual: 14/12/2017

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### Study start date

Actual: 04/09/2019

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**Data analysis start date**

Planned: 29/08/2020

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**Date of interim report, if expected**

Planned: 29/12/2020

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**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

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**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

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**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)
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## **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

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

### Data sources (types)

[Other](#)

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### 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

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No