Surveillance of Safety and Efficacy of wilate® in patients with von Willebrand disease (Wil-20)

First published: 24/02/2016

Last updated: 02/07/2024

Study | Finalised

Administrative details

EU PAS number

EUPAS12560

Study ID

26513

DARWIN EU® study

No

Study countries

- Argentina
- Canada
- Colombia
- Czechia

Germany
Portugal
Spain
Sweden
United Kingdom
United States
Uruguay

Study description

Primary objective is to document the safety and tolerability of wilate® for prophylaxis and treatment of bleeding in VWD, incl. surgeriesSecondary objective is to document the efficacy of wilate® in the treatment of acute bleeding, in the prophylaxis of VWD and in interventional procedures (e.g. minor/major surgery, dental care, invasive diagn. proced. etc.).Population:VWD patients of any gender, age, or VWD type, previously treated (PTPs) or previously untreated patients (PUPs).Investigational and reference therapy:wilate® - human coagulation factor VIII and human von Willebrand factor (VWF)Design:Open-label, prospective, multicentre, multinational, post-marketing, observational, non-interventional surveillanceEfficacy assessments:Assessment of efficacy of wilate® in prevention and/or treatment of bleeding episodes and in surgical procedures will be based on a 4-point hemostatic efficacy scale as “excellent”, “good” “moderate” or “none”. The frequency of bleeding episodes in total and per bleeding site, days of treatment of bleeding episodes in total and per bleeding site, exposure days and consumption of wilate® per event, per patient and in total will be calculated.Safety/Tolerability assessments:Assessment of safety will be based on recorded Adverse Drug Reactions during the full course of the observation. Assessment of tolerability will be based on a 3 point Verbal Rating Scale.As recomm. assessment, this study will observe development of inhibitors against VWF in response to wilate® treatment (ELISA). Inhibitor assessment should be

performed before and after first wilate® application, and then the every 3 months.As recomm. assessment, study will observe the coagulation parameters based on assessment of prothrombin fragment 1 and 2 (F1+2) and D-dimer (DD) by latex enhanced immunoturbimetric test. Thrombogenicity assessment should be performed before first wilate® application, 1 hour, 3 and 24 hours after application and every 3 months

Study status

Finalised

Research institutions and networks

Institutions

Octapharma

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Institution

Multiple centres: 30 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Irina Kruzhkova

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 08/12/2010

Study start date

Actual: 27/02/2011

Date of final study report

Planned: 31/05/2018

Actual: 29/06/2018

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Octapharma

Regulatory

Was the study required by a regulatory body?

Unknown

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Primary data collection

Main study objective:

Primary objective is to document the safety and tolerability of wilate® for prophylaxis and treatment of bleeding in VWD, incl. surgeriesSecondary

objective:Secondary objective is to document the efficacy of wilate® in the treatment of acute bleeding, in the prophylaxis of VWD and in interventional procedures (e.g. minor/major surgery, dental care, invasive diagnostic procedures etc.).

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational post-marketing surveillance

Study drug and medical condition

Medicinal product name, other

Wilate - B02BD06

Medical condition to be studied

Von Willebrand's disease

Population studied

Short description of the study population

von Willebrand disease (VWD) patients of any gender, age, or VWD type, previously treated (PTPs) or previously untreated patients (PUPs).

Age groups

- 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)
- 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

von Willebrand disease (VWD) patients

Estimated number of subjects

50

Study design details

Outcomes

Assessment of safety will be based on recorded Adverse Drug Reactions during the full course of the observation. Assessment of tolerability will be based on a 3 point Verbal Rating Scale. Assessment of efficacy of wilate® in prevention and/or treatment of bleeding episodes and in surgical procedures will be based on a 4-point hemostatic efficacy scale as “excellent”, “good” “moderate” or

“none”.

Data analysis plan

The responsibility for the statistical analyses presented in the final report belongs to: contract research organisation: GASD, Gesellschaft für Angewandte Statistik + Datenanalyse mbH, Am Konvent 8 - 10, 41460 Neuss, Germany.This is a prospective post-licensure surveillance that will be conducted as an international multi-centre non-interventional surveillance. All items of the CRF will be analyzed by means of descriptive statistical methods.

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

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

Unknown