ISRCTN11568655 https://doi.org/10.1186/ISRCTN11568655

Collection of data on the use of a VWF/FVIII concentrate (wilate®) in all clinical applications in von Willebrand disease

Submission date	Recruitment status	[_] Prospectively registered
19/01/2022	No longer recruiting	[_] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
09/02/2022	Ongoing	[_] Results
Last Edited	**Condition category**	[_] Individual participant data
27/05/2025	Haematological Disorders	[X] Record updated in last year

Plain English summary of protocol

Background and study aims
Von Willebrand disease (VWD) is a blood disorder in which the blood does not clot properly due to low levels of the clotting protein von Willebrand factor (VWF). Medicines such as wilate can be used to replace the missing factor. The aim of this study is to determinate the effectiveness and tolerability of wilate in the prophylaxis (prevention) and treatment of bleeding as well as in surgical interventions under everyday clinical conditions.

Who can participate?
Patients with VWD requiring treatment with VWF-containing product (wilate)

What does the study involve?
The routine treatment of VWD patients will be documented, including all treatments with Wilate, any bleeding episodes, surgical interventions and optional data on quality of life and joint health.

What are the possible benefits and risks of participating?
All information collected in the course of the treatment with wilate will add knowledge on the use of wilate and treatment of the VWD under routine conditions. There are no risks arising from participation.

Where is the study run from?
Gerinnungszentrum Rhein-Ruhr (GZRR) (Germany)

When is the study starting and how long is it expected to run for?
January 2020 to May 2025

Who is funding the study?
Octapharma GmbH (Germany)

Who is the main contact?
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Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number
Nil known

IRAS number

ClinicalTrials.gov number
Nil known

Secondary identifying numbers
WIL-34

Study information

Scientific Title
Non-interventional study to capture data on the use of a VWF/FVIII concentrate (wilate®) in all clinical settings of von Willebrand disease including health-related quality of life

Acronym
wilate-NEWS

Study objectives
Wilate is efficacious and safe for the treatment of von Willebrand's disease in clinical practice.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Approved 28/09/2020, Ethics Committee of Medical Association of North Rhine (Ärztekammer Nordrhein, Tersteegenstraße 9, 40474 Düsseldorf, Germany; +49 (0)211 4302 2272; ethik@aekno.de), ref: 2020260

Study design
Open prospective multi-centre national non-interventional study

Primary study design
Observational

Secondary study design
Cohort study

Study setting(s)
Other

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details to request a patient information sheet

Health condition(s) or problem(s) studied
Von Willebrand disease (VWD)

Interventions
wilate-NEWS is an non-interventional study for which the routine treatment of VWD patients on prophylaxis or on-demand treatment is documented. All treatments occurring with wilate, any bleeding episodes occurring or surgical procedures are carefully documented. Additionally, a joint score (HJHS), quality of life questionnaire (SF-36) and/or a pictorial blood assessment chart (PBAC) can be documented on an optional basis. The observation period of each patient is not fixed. Follow-up is planned at regular intervals (every 6 months) after the inclusion of the patients.

Intervention Type
Drug

Phase
Not Applicable

Drug/device/biological/vaccine name(s)
wilate®

Primary outcome measure
Percentage of haemostatic efficacy ratings of "excellent" and "good" for bleeding treatments and surgical interventions, as well as the annual bleeding rate in prophylactically treated

patients, measured at the end of the study. According to the study protocol, efficacy is rated by the treating physicians for every bleeding event treated with Wilate and surgery respectively treated with Wilate on a 4-point Likert scale (excellent, good, moderate, none).

Secondary outcome measures
1. Safety assessed using the number of patients experiencing at least one adverse drug reaction (ADR) measured at the end of the study
2. Calculation of the VWF dose/kg body weight per injection and calculation of the duration of use in the different treatment options (prophylaxis, bleeding treatment, surgical intervention), depending on the VWD type or severity of bleeding or intervention, at the end of the study
3. Health-related quality of life measured optionally half-yearly using SF-36 during treatment with Wilate, including a comparison between types of VWD and between female and male patients - as far as case numbers allow
4. Comparison of joint status determined optionally half-yearly using the Haemophilia Joint Health Score (HJHS) and annualised bleeding rate, taking into account the treatment mode as well as the VWD type (as far as the case numbers allow), at the end of the study
5. PBAC (Pictorial Blood Assessment Chart) scores in relation to treatment regimen and dosages at the end of the study

Overall study start date
02/01/2020

Completion date
31/05/2030

Eligibility

Key inclusion criteria
1. The patient has congenital or acquired Von Willebrand disease (VWD) and requires injections with VWF-containing concentrate (wilate)
2. With a positive bleeding history, other possible causes have been excluded

Participant type(s)
Patient

Age group
All

Sex
Both

Target number of participants
120

Key exclusion criteria
1. Patients with known contraindications as specified in the Summary of Product Characteristics (SPC)
2. The patient could actually be treated with DDAVP (Desmopressin Acetate)
3. The patient has a von Willebrand factor activity over 70% and there are no findings manifesting the VWD diagnosis

Date of first enrolment
01/06/2020

Date of final enrolment
31/05/2025

Locations

Countries of recruitment
Germany

Study participating centre
Gerinnungszentrum Rhein-Ruhr (GZRR)
Duisburg
Germany
47051

Sponsor information

Organisation
Octapharma GmbH

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Sponsor type
Industry

Website
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Funder(s)

Funder type
Other

Funder Name

Investigator initiated and funded

Results and Publications

Publication and dissemination plan
Planned publication as posters on congresses and in a peer-reviewed journal. Within 1 year after completion of the data collection, Octapharma GmbH submits a final report to the federal authority (Paul Ehrlich Institute) which is also made available to the public via an internet portal.

Intention to publish date
31/05/2026

Individual participant data (IPD) sharing plan
The data-sharing plans for the current study are unknown and will be made available at a later date.

IPD sharing plan summary
Data sharing statement to be made available at a later date