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Surveillance of results of long-term prophylactic treatment of von Willebrand disease with Wilate®

Submission date	Recruitment status	[X] Prospectively registered
22/08/2008	No longer recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
01/09/2008	Completed	[ ] Results
Last Edited	**Condition category**	[ ] Individual participant data
04/09/2008	Haematological Disorders	[ ] Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

Protocol serial number
WIL-18

Study information

Scientific Title

Acronym
WILCOME

Study objectives
Long-term prophylactic treatment of von Willebrand disease (VWD) patients in clinical practice.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Ethics approval received from the Ethics Committee of University Hospital Malmö on the 7th August 2008.

Study design
Prospective, open-labelled, international multi-centre post-marketing surveillance

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
von Willebrand's disease

Interventions
Treatment details:
The following dosing regimes are only recommendations, based on the Swedish experience with the long-term prophylaxis in VWD:
1. For mucosal, joint bleeds and menorrhagia, the suggested prophylactic dosing is 30 IU Wilate® (corresponding to 30 IU FVIII:C)/kg body weight 2 - 3 times/week
2. For gastrointestinal bleeds, the suggested prophylactic dosing is 40 IU Wilate® (40 IU FVIII:C) /kg body weight 2 - 3 times/week
Wilate® is administered as an intravenuos bolus injection. Dose and dosing schedule are at the full discretion of the treating physician.

Quality of life:
The Quality of Life assessments performed during the surveillance will include:
1. The patient's self-reported health-related quality of life prior to and during prophylaxis with Wilate using the validated generic instruments WHOQOL-BREF (for adults and/or parents/legal guardians) and KINDL (for children), and
2. A disease-specific VWD-QoL questionnaire (for adults, children and their parents/legal guardians)

Quality of life assessments will be measured prior to the start of the prophylaxis, as well as after 6 and 12 months of treatment.

Intervention Type
Drug

Phase

Phase IV

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

Primary outcome(s)
To assess the bleeding frequency in VWD patients prior to and after introduction of regular prophylactic therapy with the VWF-containing concentrate Wilate®. Outcomes will be measured at baseline, 6 and 12 months after treatment. Please note that the number of days the patients missed school or work, as well as occurred adverse drug reactions are documented when the patient visits his doctor, so outcomes may be measured more frequently than every 6 months.

Key secondary outcome(s))
1. To describe the joint morbidity prior to and during prophylaxis with Wilate®, using the haemophilia joint health score
2. To monitor absence from school/work prior to and during prophylaxis with Wilate®
3. To evaluate the patient's health-related quality of life prior to and during prophylaxis with Wilate® and the patient's self-reported health status prior to and during prophylaxis with Wilate®, using the validated generic instruments of World Health Organization Quality of Life-BREF (WHOQOL-BREF) (for adults) and the generic children's health-related quality of life (KINDL) (for children), and the disease specific VWD-QoL questionnaire
4. To assess treatment satisfaction and treatment efficacy prior to and during prophylaxis with Wilate®, using a 4-point Verbal Rating Scale (VRS), and the Hemo-SatA treatment questionnaire adapted for VWD (VWD-Sat)
5. To evaluate the tolerability of prophylactic treatment with Wilate®:
5.1. Using a 3-point VRS
5.2. By documenting all possibly related adverse drug reactions by the patients, and
5.3. By assessing and - if applicable - reporting all adverse drug reactions by the treating physician occurring during the treatment period with Wilate®

Outcomes will be measured at baseline, 6 and 12 months after treatment. Please note that the number of days the patients missed school or work, as well as occurred adverse drug reactions are documented when the patient visits his doctor, so outcomes may be measured more frequently than every 6 months.

Completion date
01/01/2013

Eligibility

Key inclusion criteria
1. Male and female patients of any age
2. Suffering from congenital VWD
3. In need of replacement therapy with factor concentrate
4. Patients starting with a prophylactic treatment must have documentation of at least three apparently spontaneous bleeding episodes (any bleeding site and treated with factor concentrate) in the 6 months prior to enrolment
5. Patients switching from a prophylactic treatment with another factor concentrate to prophylaxis with Wilate® should have anamnesis of bleeds with respective documentation in the period of 12 months prior to enrolment

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Not Specified

Sex
All

Key exclusion criteria
1. Presence of a bleeding disorder other than VWD
2. History of non-compliance
3. Difficulties in achieving venous access that would prohibit prophylaxis
4. Incapability to follow the requirements of the surveillance, e.g. unable to keep a patient diary

Date of first enrolment
01/09/2008

Date of final enrolment
01/01/2013

Locations

Countries of recruitment
Austria

Germany

Russian Federation

Sweden

Study participating centre
Oberlaaerstrasse 235
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Sponsor information

Organisation
Octapharma AG (Switzerland)

ROR
https://ror.org/002k5fe57

Funder(s)

Funder type
Industry

Funder Name
Octapharma AG (Switzerland)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration