CLINICAL STUDY TO INVESTIGATE THE EFFICACY AND SAFETY OF WILATE DURING PROPHYLAXIS IN PREVIOUSLY TREATED PATIENTS WITH VON WILLEBRAND DISEASE (VWD)

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Main Information

Primary registry identifying number
LBCTR2020063508

Protocol number
WIL-31

MOH registration number

Study registered at the country of origin
No

Study registered at the country of origin: Specify
Study is not registered in the country of origin (Switzerland) as the number of Von Willebrand Disease patients is low. Letter from the Sponsor is attached.

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency
01/06/2020

Primary sponsor
Octapharma AG

Primary sponsor: Country of origin
Switzerland

Date of registration in primary registry
06/07/2020

Date of registration in national regulatory agency
01/06/2020

Public title
CLINICAL STUDY TO INVESTIGATE THE EFFICACY AND SAFETY OF WILATE DURING PROPHYLAXIS IN PREVIOUSLY TREATED PATIENTS WITH VON WILLEBRAND DISEASE (VWD)

Acronym
WIL-31

Scientific title
CLINICAL STUDY TO INVESTIGATE THE EFFICACY AND SAFETY OF WILATE DURING PROPHYLAXIS IN PREVIOUSLY TREATED PATIENTS WITH VON WILLEBRAND DISEASE (VWD)

Acronym
WIL-31

Brief summary of the study: English
This is a prospective, non-controlled, international, multi-center phase 3 study investigating the efficacy and safety of Wilate in previously treated patients with Von Willebrand Disease. Participation in the study may help by reduce the number of bleeding episodes requiring on demand treatment. The treatment of VWD is already approved in Lebanon by the Ministry of Health under the name Wilate. The main goal of this study is to find out to what extent Wilate, when administered regularly and prophylactically, prevents the occurrence of bleeding episodes in patients with type 3, type 2 (except 2N), or severe type 1 VWD. Participating patients will be followed up for 12 months during which they will visit their treating physician 7 times.

Brief summary of the study: Arabic

هذه دراسة مستقبلية ، تحقق في فعالية وسلامة3 ، تحقق في فعالية وسلامة Wilate سبق في المرضى الذين سبق Wilate ، تحقق في فعالية وسلامة3هذه دراسة مستقبلية ، غير خاضعة للرقابة ، دولية ، متعددة المراكز ، مرحلة علاجهم بمرض فون ويليبراند. قد تساعد المشاركة في الدراسة عن طريق تقليل عدد نوبات النزف التي تتطلب معالجة عند الطلب. تمت الموافقة الهدف الرئيسي من هذه الدراسة هو معرفة إلى أي مدى يمنع .Wilate بالفعل في لبنان من قبل وزارة الصحة تحت اسم VWD على علاج أو النوع ، (2Nباستثناء) 2 ، النوع 3عند إعطائه بانتظام وبشكل وقائي ، حدوث نوبات النزف في المرضى الذين يعانون من النوع ، Wilate مرات7 شهرًا يزورون خلالها الطبيب المعالج 12. ستتم متابعة المرضى المشاركين لمدة 1الشديد VWD

Health conditions/problem studied: Specify

Von Willebrand Disease

Interventions: Specify

This is an open label study to provide VWD patients with Wilate prophylactically.

Key inclusion and exclusion criteria: Inclusion criteria

Inclusion Criteria:

Patients who meet all of the following criteria are eligible for the study:

- Aged ≥6 years at the time of screening
- VWD type 1 (baseline von Willebrand factor activity [VWF:Ristocetin Co-factor (RCo)] <30 IU/dL, 2A, 2B, 2M, or 3 according to medical history requiring substitution therapy with a VWF-containing product to control bleeding
- Currently receiving on-demand treatment with a VWF-containing product with at least 1, and an average of ≥2, documented spontaneous BEs per month in the last 6 months, with at least 2 of these BEs requiring treatment with a VWF-containing product
- Availability of records to reliably evaluate type, frequency, and treatment of BEs for at least 6 months of on-demand treatment before screening
- Female patients of child-bearing potential must have a negative urine pregnancy test at screening and agree to use adequate birth control measures; in case hormonal contra-ception is used, the medication class should remain unchanged for the duration of the study
- All patients to provide voluntarily given, fully informed written and signed consent obtained before any study-related procedures are conducted

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

6

Key inclusion and exclusion criteria: Age maximum

80

Key inclusion and exclusion criteria: Exclusion criteria

Exclusion Criteria:

Patients who meet any of the following criteria are not eligible for the study:

- Having received on-demand or prophylactic treatment with a VWF-containing product but having no records available to reliably evaluate the type, frequency, and treatment of BEs over a period of at least 6 months of on-demand treatment
- History, or current suspicion, of VWF or FVIII inhibitors
- Medical history of a thromboembolic event within 1 year before enrolment
- Severe liver or kidney diseases (alanine aminotransferase [ALAT] and aspartate trans-aminase [ASAT] levels >5 times of upper limit of normal, creatinine >120 µmol/L)
- Platelet count <100,000/mL at screening (except for VWD type 2B)
- Body weight <20 kg at screening
- Patients receiving, or scheduled to receive, immunosuppressant drugs (other than an-tiretroviral chemotherapy), such as prednisone (equivalent to >10 mg/day), or similar drugs
- Pregnant or breast-feeding at the time of enrolment
- Cervical or uterine conditions causing abnormal uterine bleeding (including infection, dysplasia)
- Treatment with any IMP in another interventional clinical study currently or within 4 weeks before enrolment
- Other coagulation disorders or bleeding disorders due to anatomical reasons
- Known hypersensitivity to any of the components of the study drug

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Prophylaxis

Trial scope: Specify scope

N/A

Study design: Allocation

N/A

Study design: Masking

Open (masking not used)

Study design: Control
Uncontrolled

Study phase
3

Study design: Purpose
Treatment

Study design: Specify purpose
N/A

Study design: Assignment
Single

Study design: Specify assignment
N/A

IMP has market authorization
Yes, Lebanon and Worldwide

IMP has market authorization: Specify
Worldwide

Name of IMP
Wilate

Year of authorization
2005

Month of authorization
2

Type of IMP
Plasma derived

Pharmaceutical class
plasma-derived coagulation factor complex consisting of von Willebrand factor/coagulation factor VIII complex

Therapeutic indication
Von Willebrand Disease type 3, type 2 (except 2N), and severe type 1.

Therapeutic benefit
To decrease bleeding events in patients with VWD.

Study model
N/A

Study model: Explain model
N/A

Study model: Specify model
N/A

Time perspective
N/A

Time perspective: Explain time perspective
N/A

Time perspective: Specify perspective
N/A

Target follow-up duration

Target follow-up duration: Unit

Number of groups/cohorts

Biospecimen retention
Samples with DNA**

Biospecimen description
The genetic testing will be done only to patients with VWD 2B using a blood sample. In patients aged ≥17 years, this blood sample will be taken during the Baseline IVR Visit, and in patients aged 6–16 years, it will be taken during the Screening Visit. If no mutations related to type 2B VWD are identified, the same sample will be used to perform genetic testing to exclude the presence of pseudo-VWD.

Target sample size
28

Actual enrollment target size

Date of first enrollment: Type
Anticipated

Date of first enrollment: Date
01/06/2020

Date of study closure: Type
Anticipated

Date of study closure: Date
30/11/2021

Recruitment status
Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan
No

IPD sharing statement description
Undecided

Additional data URL
https://clinicaltrials.gov/ct2/show/NCT04052698?term=WIL-31&draw=2&rank=1

Admin comments

Trial status
Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Clinicaltrials.gov	NCT04052698
EudraCT	2018-004675-13

Sources of Monetary or Material Support

Name
Octapharma AG

Secondary Sponsors

Name
N/A

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Hanen Hamid	Beirut	Lebanon	+9613761145	hanen.hamid@ergomedplc.com	Ergomed PLC
Scientific	Irina Kruzhkova	Lachen	Switzerland	+41554512173	Irina.kruzhkova@octapharma.com	Octapharma AG

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu de France	Dr. Claudia Khayat	Pediatric Hematolgy	Approved
American University of Beirut-Medical Center	Dr. Ali Taher	Hematology/Oncology	Not approved
Nini Hospital	Dr. Adlette Inati	Pediatric Hematology	Not approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	04/02/2020	Mrs. Virginia El Khoury	cue@usj.edu.lb	+961 1 421 229

Countries of Recruitment

Name
Lebanon
United States of America
Belarus
Bulgaria
Croatia
Hungary
Russian Federation
Ukraine

Health Conditions or Problems Studied

Condition	Code	Keyword
Hematology	Von Willebrand s disease (D68.0)	Von Willebrand Disease

Interventions

Intervention	Description	Keyword
Intravenous injection	prophylactic IV Infusion of Wilate	Wilate
PK sampling	only applicable for patients younger than 17 years	Pharmacokinetic testing

Primary Outcomes

Name	Time Points	Measure
total annualised bleeding rate decrease by more than 50%	12 months after recruitment	Number of on demand treatment with Von Willebrand Factor Containing product

Key Secondary Outcomes

Name	Time Points	Measure
Spontaneous annualised bleeding rate	1 month, 2 months, 3 months, 6 months, 9 months and 12 months after recruitment	Number of on demand treatment with Von Willebrand Factor Containing product
Von Willebrand Factor Activity	1 month, 2 months, 3 months, 6 months, 9 months and 12 months after recruitment	Blood testing and number of on demand treatment with Von Willebrand containing product
Safety and tolerability of Wilate	From the inclusion of patients till 12 months after recruitment	Adverse Events experienced by participating patients

Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files