



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





في المرضى الذين سبق Wilate ، تحقق في فعالية وسلامة3 هذه دراسة مستقبلية ، غير خاضعة للرقابة ، دولية ، متعددة المراكز ، مرحلة علاجهم بمرض فون ويلبراند. 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 ، (2N باستثناء) 2 ، النوع 3 عند إعطائه بانتظام وبشكل وقائي ، حدوث نوبات النزف في المرضى الذين يعانون من النوع ، Wilate ، مرات7 شهراً يزورون خلالها الطبيب المعالج 12. سيتم متابعة المرضى المشاركين لمدة 1 الشهر 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 $\mu\text{mol/L}$)
- Platelet count $< 100,000/\text{mL}$ at screening (except for VWD type 2B)
- Body weight < 20 kg at screening
- Patients receiving, or scheduled to receive, immunosuppressant drugs (other than anti-retroviral 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 design: Purpose

Treatment

Study design: Assignment

Single

IMP has market authorization

Yes, Lebanon and Worldwide

Name of IMP

Wilate

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: Specify model

N/A

Time perspective

N/A

Time perspective: Specify perspective

N/A

Target follow-up duration**Number of groups/cohorts****Biospecimen retention**

Samples with DNA**

Study phase

3

Study design: Specify purpose

N/A

Study design: Specify assignment

N/A

IMP has market authorization: Specify

Worldwide

Year of authorization

2005

Month of authorization

2

Study model: Explain model

N/A

Time perspective: Explain time perspective

N/A

Target follow-up duration: Unit**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	Hanan Hamid	Beirut	Lebanon	+9613761145	hanan.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 Hematology	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