

## 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 الشدديد 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  $\geq 6$  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  $\geq 2$ , 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  $\geq 17$  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.



<b>Target sample size</b> 28	<b>Actual enrollment target size</b>
<b>Date of first enrollment: Type</b> Anticipated	<b>Date of first enrollment: Date</b> 01/06/2020
<b>Date of study closure: Type</b> Anticipated	<b>Date of study closure: Date</b> 30/11/2021
<b>Recruitment status</b> Pending	<b>Recruitment status: Specify</b>
<b>Date of completion</b>	
<b>IPD sharing statement plan</b> No	<b>IPD sharing statement description</b> Undecided
<b>Additional data URL</b> <a href="https://clinicaltrials.gov/ct2/show/NCT04052698?term=WIL-31&amp;draw=2&amp;rank=1">https://clinicaltrials.gov/ct2/show/NCT04052698?term=WIL-31&amp;draw=2&amp;rank=1</a>	
<b>Admin comments</b>	
<b>Trial status</b> 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 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