

# Etude de la qualité de vie et de l'impact médico-économique de la maladie de Willebrand en France (WiSH-QoL)

**First published:** 14/12/2023

**Last updated:** 10/12/2025

Study

Finalised

## Administrative details

### EU PAS number

EUPAS107760

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### Study ID

107761

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### DARWIN EU® study

No

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### Study countries

France

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### Study description

This was an observational, non-interventional, descriptive study, without modification of the doctor-patient relationship, conducted across 28 French specialized VWD centers. Patient's recruitment concerns male and female of any age with congenital Von Willebrand Disease (VWD). Quality of life analysis is based on generic and Von Willebrand Disease-specific quality of life questionnaires.

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### **Study status**

Finalised

## Research institutions and networks

### Networks

[Centre de Référence de la Maladie de Willebrand \(CRMW\)](#)

## Contact details

### **Study institution contact**

Evelyne SAUTY [Evelyne.sauty@lfb.fr](mailto:Evelyne.sauty@lfb.fr)

**Study contact**

[Evelyne.sauty@lfb.fr](mailto:Evelyne.sauty@lfb.fr)

### **Primary lead investigator**

Annie Borel-Derlon

## Study timelines

### **Date when funding contract was signed**

Actual: 09/08/2014

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### **Study start date**

Actual: 01/10/2014

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### **Data analysis start date**

Actual: 31/03/2022

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### **Date of interim report, if expected**

Actual: 25/05/2018

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### **Date of final study report**

Actual: 30/11/2023

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

LFB BIOMEDICAMENTS

## Study protocol

[Synopsis WiSH-QoL v3.0 - 23 10 2020\\_VEnglish final.pdf](#) (234.6 KB)

## Regulatory

## Was the study required by a regulatory body?

No

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## Is the study required by a Risk Management Plan (RMP)?

Not applicable

## Methodological aspects

### Study type

### Study type list

#### **Study type:**

Non-interventional study

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#### **Main study objective:**

The main objective is the analysis of the quality of life of patients with Von Willebrand Disease Whatever their age, sex and phenotype.

### Study Design

#### **Non-interventional study design**

Other

### Study drug and medical condition

#### **Anatomical Therapeutic Chemical (ATC) code**

(B02BD10) von Willebrand factor

von Willebrand factor

## Population studied

### Age groups

- Preterm newborn infants (0 - 27 days)
  - Term newborn infants (0 - 27 days)
  - Infants and toddlers (28 days - 23 months)
  - Children (2 to < 12 years)
  - Adolescents (12 to < 18 years)
  - Adults (18 to < 46 years)
  - Adults (46 to < 65 years)
  - Adults (65 to < 75 years)
  - Adults (75 to < 85 years)
  - Adults (85 years and over)
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### Estimated number of subjects

350

## Study design details

### Outcomes

Analyse the quality of life of patients with Von Willebrand Disease using generic and Von Willebrand Disease-specific quality of life questionnaires. 1. Survey and identify therapeutic practices in Von Willebrand Disease 2. Evaluate the impact of treatment strategies on Von Willebrand 3. Investigate/analyse the impact of Von Willebrand Disease on the clinical status, treatments and complications in

subgroups of the population

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### **Data analysis plan**

Statistical analyses were mainly descriptive on each outcome. The parameters collected were described for all analyzable patients. The quality-of-life information was analyzed by "scoring" several dimensions of each questionnaire used for adults and for children and/or their parents: generic quality of life questionnaire, Von Willebrand Disease-specific questionnaire including treatment satisfaction and FABEL questionnaire about family impact due to chronic pathology. The quantitative variables were expressed as a mean  $\pm$  standard deviation, median, quartiles and range, and the qualitative variables as a frequency table. The descriptive analysis was carried out in following subgroups: age classes, phenotypes (type 1, type 2, type 3, type undetermined) and treatment strategy (on-demand, prophylaxis).

## Data management

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

### **Conflicts of interest of investigators**

[WiSH-QoL\\_Sites list.pdf](#) (488.46 KB)

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## Data sources

## **Data sources (types)**

Other

## Use of a Common Data Model (CDM)

### **CDM mapping**

No

## Data quality specifications

### **Check conformance**

Unknown

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No