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

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Study Finalised

Administrative details

EU PAS number

EUPAS107760

Study ID

107761

DARWIN EU® study

No

Study countries

France

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.

Study status

Finalised

Research institutions and networks

Networks

Centre de Référence de la Maladie de Willebrand (CRMW)

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Annie Borel-Derlon

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 09/08/2014

Study start date
Actual: 01/10/2014

Data analysis start date
Actual: 31/03/2022

Date of interim report, if expected
Actual: 25/05/2018

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

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

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

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)

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

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 ± standard deviation, median, quartiles and range, and the qualitative variables as a frequency table. The descriptive analysis was carried out in following sub-groups: 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)

Data sources

Data sources (types)

Other

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No