Real-World Evidence in Rare Diseases in France: Treatment Patterns, Healthcare Resource Use and Related Costs in Von Willebrand Disease (FORvWARD STUDY)

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INTRODUCTION

- Von Willebrand disease (VWD) is a rare disease caused by missing or defective von Willebrand factor (VWF).
- VWD-patients have an increased bleeding risk and can receive replacement therapies (RT) as long-term treatment (LTT-RT) or on-demand (OD-RT) as part of their disease management.
- To date, limited real-world evidence is available in France within patients treated with RT.
- The FORvWARD study is a national real-world study aiming to describe RT-treatment patterns, healthcare resource use (HRU) and related-costs in VWD-treated patients in France.

METHODS

STUDY DESIGN

- Observational retrospective cohort study using the French national healthcare claims database (SNDS), i.e., data on patient characteristics, hospitalizations, outpatient visits, drug dispensing, biological and medical acts, sick leaves, etc.[1]

PERIODS OF INTEREST

- Study period: from 2017/01/01 to 2021/12/31
- Pre-study period: 3 years before index date to identify comorbidities.
- Inclusion period: from 2017/01/01 to 2021/09/30
- Index date: 1st evidence of a RT dispensing (out or in-hospital during the inclusion period)
- End of follow-up: first event occurring among end of the study period, death or loss to follow-up

STUDY POPULATION

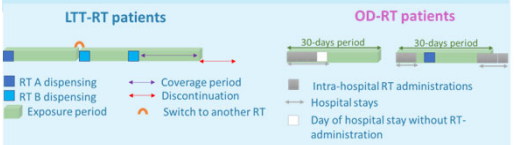
- Inclusion criteria: patients with ≥1 reimbursement of a RT containing VWF during the inclusion period. RT included all available in the French market (i.e., EQWILATE[a], VEYVONDI[b], VONCENTO[c], WILFACTIN[d] and WILSTART[e]).
- Exclusion criteria: patients with ≥1 diagnosis of haemophilia and no diagnosis of VWD identified through long-term disease status or hospitalization diagnoses between 2014/01/01 and 2021/12/31

DEFINITION OF EXPOSURE PERIODS (Figure 1)

Patients were classified as LTT-RT or OD-RT users, based on a previously presented algorithm based on frequencies, delivery types, and percentage of days covered by RT[2].

The exposure periods (EPs) were defined in each group of users (Figure 1):

FIGURE 1. EXPOSURE PERIODS IN LTT-RT AND OD-RT PATIENTS



- For LTT-RT patients, **the EP corresponded to the period from the 1st out-hospital RT dispensed** to the 1st event occurring (i.e., end of follow-up, switch to another LTT-RT or last day of coverage in case of discontinuation).
- For OD-RT patients, **the EPs were all 30-days periods** starting on the 1st day of a hospital stay with RT administration.

DATA ANALYSIS

HCRU/ related costs were described using descriptive statistics (mean [SD], median, Q1-Q3), over RT-EPs (No), separately within LTT-RT (annually) and OD-RT patients (per 30-days EP).

Doses per Kg were estimated in RT-IU/Kg based on published[3] weights by gender and age group, only in patients aged 18 year or older. **Co-treatments** were defined as FVIII, desmopressin or tranexamic acid. **VWD-related hospitalizations** were defined as hospitalizations with main or associated diagnosis of VWD and/or a RT administration. **VWD-related costs** (year 2021) were defined as costs to VWD-related hospitalizations, outpatient visits and nurse visits, RT and co-treatments dispensings, some medical procedures and outpatient biological acts.

REFERENCES

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MA Holders of VWF products

a: OCTAPHARMA France; b: TAKEDA France; c: CSL Behring GmbH; d,e: LFB BIOMEDICAMENTS

OBJECTIVES:

To describe RT-treatment patterns, healthcare resource use (HRU) and associated costs of VWD-treated patients in France, separately in LTT-RT and OD-RT patients

STUDY RESULTS

TABLE 1. PATIENT CHARACTERISTICS

LTT-RT, N=63	Variables of interest	OD-RT, N=3,291
39.8 (25.7)	Mean (SD) age at index date	44.1 (23.5)
58.7	% of females	55.0
4.4 (1.1)	Mean follow-up duration, in years (SD)	**2.6 (1.5)**
116 ; 1.8 (1.4)	**Total # of EPs**; Mean # of EPs per patient (SD)	**4,908***; 1.6 (1.3)
3.2 (1.6)	Mean duration of the EPs per patient, in years (SD)	**

*The 4,908 exposure periods concerned 3,006 OD-RT patients with at least one EP
**EPs are 30-days periods in OD-RT patients. No: number of exposure periods

Within LTT-RT PATIENTS

Within OD-RT PATIENTS

TREATMENT PATTERNS

TABLE 2. ANNUAL TREATMENT PATTERNS PER EP (LTT-RT PATIENTS)

Annual rate of out-hospital RT-dispensing	Mean (SD)	17.8 (23.5)
	Median (Q1-Q3)	**13.0** (10.7-15.7)
Annual RT-IU dispensed out-hospital	Mean (SD)	337,957 (226,024)
	Median (Q1-Q3)	**305,333** (183,051-440,850)
Annual total RT dose dispensed (IU/Kg)	No=80 (69.0%)	
	Mean (SD)	4,880 (2,939)
	Median (Q1-Q3)	**4,638** (3,034 - 6,400)
Annual total RT-IU, incl. both in- and out-hospital injections	Mean (SD)	349,193 (236,747)
	Median (Q1-Q3)	**324,990** (185,113 - 452,173)
#EP with ≥1 in-hospital RT-administration	No=71 (61.2%)	

TABLE 4. TREATMENT PATTERNS PER 30-DAYS EP (OD-RT PATIENTS)

# of days with RT-administration during the stay*	Mean (SD)	2.0 (2.1)
	Median (Q1-Q3)	**1.0** (1.0 - 2.0)
# of RT-IU received/ day of administration*	Mean (SD)	4,460 (4,801)
	Median (Q1-Q3)	**3,000** (2,000- 5,000)
Dose of RT-IU/kg received per day of administration	No=4,210 (85.8%)	
	Mean (SD)	66.0 (66.5)
	Median (Q1-Q3)	**49.1** (35.1 - 73.6)
≥1 out-hospital RT-dispensing during the EP	No=780 (15.9%)	
Total amount of RT-IU, incl. both in- and out-hospital injections	Mean	11,810 (18,425)
	Median (Q1-Q3)	**6,000** (3,000 - 13,500)

* During the stay leading the 30-days period

HCRU & ASSOCIATED COSTS

TABLE 3. ANNUAL VWD-RELATED HRU PER EP (LTT-RT PATIENTS)

Outpatient visits to GP	Mean (SD)	**5.8** (7.0)
	Median (Q1-Q3)	3.7 (0.9 - 8.6)
Nurse visits	Mean (SD)	**87.5** (76.7)
	Median (Q1-Q3)	96.8 (14.8 - 125.2)
Dosage of VWF	Mean (SD)	**4.6** (24.2)
	Median (Q1-Q3)	0.4 (0.0 - 1.8)
Dosage of factor VIII	Mean (SD)	4.6 (24.2)
	Median (Q1-Q3)	0.4 (0.0 - 1.6)
Dosage of fibrinogen	Mean (SD)	2.2 (16.9)
	Median (Q1-Q3)	0.2 (0.0 - 0.8)
Administration of co-treatments	Mean (SD)	**4.9** (9.4)
	Median (Q1-Q3)	1.4 (0.4 - 4.5)
VWD-related hospitalizations	Mean (SD)	**3.1** (17.0)
	Median (Q1-Q3)	0.8 (0.0 - 2.0)

TABLE 5. VWD-RELATED HRU PER 30-DAYS EP (OD-RT PATIENTS)

Outpatient visits to GP	Mean (SD)	0.6 (0.9)
	Median (Q1-Q3)	0.0 (0.0 - 1.0)
Nurse visits	Mean (SD)	**3.0** (5.8)
	Median (Q1-Q3)	0.0 (0.0 - 3.5)
Dosage of VWF	Mean (SD)	**0.1** (0.3)
	Median (Q1 – Q3)	0.0 (0.0 - 0.0)
Dosage of factor VIII	Mean (SD)	0.1 (0.4)
	Median (Q1-Q3)	0.0 (0.0 - 0.0)
Dosage of fibrinogen	Mean (SD)	0.1 (0.4)
	Median (Q1-Q3)	0.0 (0.0 - 0.0)
Administration of co-treatments	Mean (SD)	**0.5** (0.8)
	Median (Q1-Q3)	0.0 (0.0 - 1.0)
VWD-related hospitalizations	Mean (SD)	**1.2** (0.6)
	Median (Q1-Q3)	1.0 (1.0 - 1.0)

FIGURE 2. ANNUAL COSTS RELATED TO VWD (LTT-RT PATIENTS)

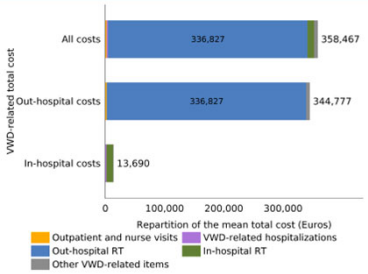
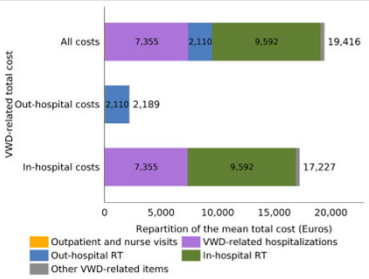


FIGURE 3. EP COSTS RELATED TO VWD (OD-RT PATIENTS)



CONCLUSIONS

- The FORvWARD study is the **first large-scale real-world study describing RT patterns of use, HRU and associated costs** by type of RT-users in a VWD-treated population in France.
- These findings allow the **quantification of management costs related to each RT type of user**.
- Future comparative analyses might identify potential differences across treatments.

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DECLARATION OF INTEREST

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This poster is intended for healthcare professionals.

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