LEVERAGING NATIONAL REAL-WORLD EVIDENCE TO UNDERSTAND DIFFERENCES OF ECONOMIC BURDEN IN RARE DISEASES: THE CASE OF VON WILLEBRAND DISEASE IN FRANCE

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INTRODUCTION

Von Willebrand disease (VWD) is a genetic disorder caused by missing or defective von Willebrand factor (VWF), inducing increased risk of bleeding. Replacement treatments (RT), i.e. treatments containing VWF ± factor VIII, are key in VWD management.

OBJECTIVES

To describe the key characteristics of patients and compare costs associated to healthcare resource use of occasional RT users (on-demand [OD]-users).

METHODS

STUDY DESIGN: Retrospective observational study.

Data source: The French national healthcare claims database (SNDS)[1], that contains information about patients (sociodemographic information, long-term disease status, vital status), information related to hospitalizations (associated diagnoses, dispensing of costly medications, entry and discharge dates), outpatient visits and drug dispensing, biological and medical acts, sick leaves, etc.

STUDY POPULATION

- **Inclusion criteria**: patients with ≥1 reimbursement of RT between January 1st, 2017 and September 30th, 2021 (study period). RT included: Veyvondi[b], Wilfactin[d], Voncento[c], Eqwilate[a], Wilstart[e].
- **Exclusion criteria**: patients with long-term disease status or hospitalization diagnoses of hemophilia without diagnoses of VWD over the period 2014-2021.

Among the study population, OD-users aged 18+ were identified using the previously presented algorithm based on frequencies, delivery types, and percentage of days covered by RT[2].

Only OD-users having at least 1 hospitalization were considered.

STUDY PERIOD : From January 1st, 2017 to December 31st, 2021

- **Index date:** date of the 1st evidence of a RT dispensing or in-hospital administration during the inclusion period
- **Follow-up period**: until end of the study period, death or loss to follow-up
- **Pre-study period**: 3 years before index date to identify comorbidities

DEFINITION OF EXPOSURE PERIOD AND COSTS EVALUATED

- For all patients identified as OD users, costs were assessed for HRCU occurring over exposure periods (EP) defined as all 30-days periods starting on the 1st day of a hospital stay with RT administration. One given patient may have several EP with different RT.
- Costs evaluated by EP by RT were: in-hospital costs, i.e. costs of hospitalizations and in-hospital doses of RT and additional FVIII, and out-hospital costs, i.e. costs of visits to general practitioner (GP) and nurse and out-hospital RT and FVIII.

DATA ANALYSIS

- Patient characteristics', exposure periods (EP) and related costs were described using descriptive statistics (mean over a period of 30-days for each exposure period [EP]).
- Related costs were compared across RTs (Veyvondi, Wilfactin, Voncento, Eqwilate) using Generalized Estimating Equation models (GEE) accounting for intra-patient correlation and for potential confounding factors (age, gender, geographical area, duration of the first stay and history of RT exposure).

RESULTS

Table 1. Patients' and exposure periods' characteristics by RT

	Overall	Veyvondi[b]	Wilfactin[d]	Voncento[c]	Eqwilate[a]	Wilstart[e]
Number of patients (%)	2,750 (100)	252 (9.2)	2,072 (75.3)	327 (11.9)	46 (1.7)	57 (2.1)
Number of EP (%)	4,210 (100)	356 (8.5)	3,250 (77.2)	472 (11.2)	55 (1.3)	77 (1.8)
Mean (SD) number of EP/patient*	1.7 (1.3)	1.4 (0.8)	1.6 (1.3)	1.4 (0.9)	1.2 (0.7)	1.4 (0.9)
Mean follow-up duration in years (SD)	2.6 (1.5)	1.2 (0.6)	2.7 (1.5)	2.8 (1.4)	1.0 (0.6)	3.3 (1.4)
Mean age in years (SD)	51.2 (18.7)	49.0 (17.5)	51.7 (18.7)	50.6 (19.0)	50.8 (17.3)	58.2 (19.6)
% females	57.5%	60.7%	58.6%	59.0%	54.3%	38.6%
Diagnosis associated to the stay‡, No (%)*						
Bleeding	449 (10.7)	29 (8.1)	332 (10.2)	63 (13.3)	9 (16.4)	16 (20.8)
Surgery	3,038 (72.2)	243 (68.3)	2,402 (73.9)	320 (67.8)	35 (63.6)	38 (49.4)
Other	723 (17.2)	84 (23.6)	516 (15.9)	89 (18.9)	11 (20.0)	23 (29.9)
Mean Charlson Comorbidities index (SD)	1.8 (2.0)	1.4 (1.6)	1.8 (2.1)	1.6 (2.1)	1.5 (1.5)	2.4 (1.8)

*In patients with at least one exposure period

EP: exposure period; No: number of EP; ‡ indicates the diagnosis associated to the stay starting the EP.

Figure 1. Mean costs by type of healthcare resource use by RT

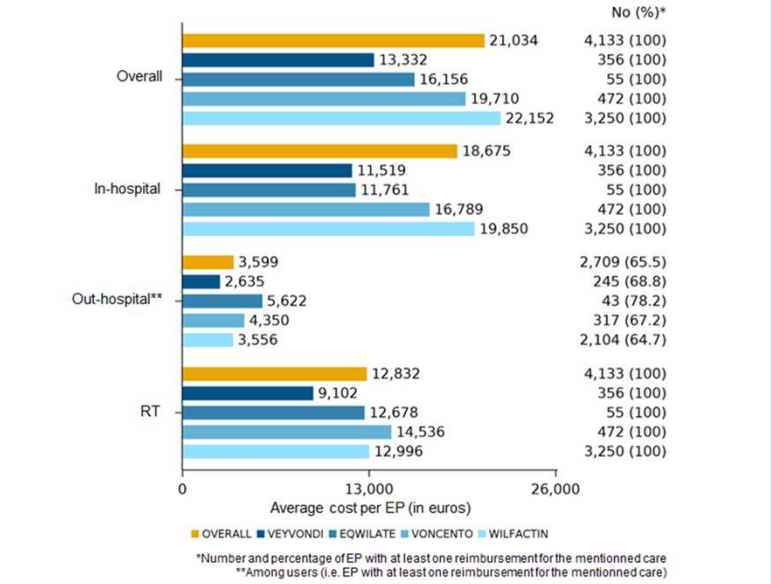
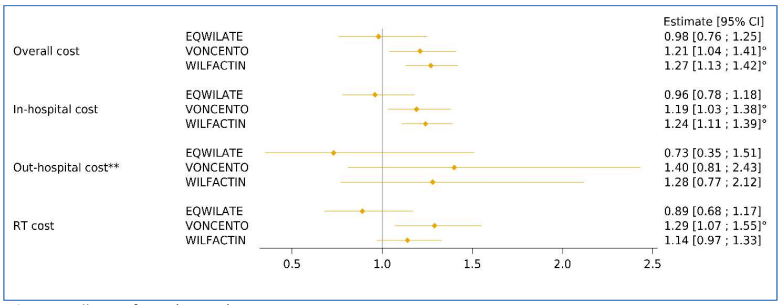


Figure 2. Costs adjusted comparisons (Veyvondi as reference group)



°statistically significant (p<0.05)

**Among users in each RT (i.e. EP with at least one reimbursement for the mentioned care)

Wilstart[e] EPs were excluded from the comparison analysis, as no relevant clinical profile could be linked to patients treated only with Wilstart[e].

Key findings

- The **overall mean costs** (21,034 €/EP) were significantly higher in Wilfactin-treated EP (22,152 €/EP) and in Voncento-treated EP (19,170 €/EP) than in Veyvondi-treated EP (13,332 €/EP).
- Costs were mainly driven by **in-hospital costs** (18,675 €/EP), which were significantly higher in Wilfactin-treated EP(19,850 €/EP) and in Voncento-treated EP (16,789 €/EP) than in Veyvondi-treated EP (11,519 €/EP).
- **Costs associated to RT** were significantly higher in Voncento-treated EP (14,536 €/EP) than in Veyvondi-treated EP (9,102 €/EP).
- No statistically significant difference across RT was observed within out-hospital costs.

CONCLUSIONS

- This study is the first large scale real-world study describing and comparing costs of occasional RT users in VWD, based on the SNDS data.
- Overall costs were the lowest within Veyvondi-treated exposure periods (EP), while costs were significantly higher in Wilfactin- and Voncento-treated EP.
- Future studies accounting for clinical data should provide evidence on predictive factors which could explain the observed differences across RTs.

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MA HOLDERS OF VWF PRODUCTS

A: OCTAPHARMA FRANCE; B: TAKEDA FRANCE; C: CSL BEHRING GMBH; D,E: LFB BIOMEDICAMENTS. EACH PRODUCT'S TRADEMARK IS THE PROPERTY OF THE RESPECTIVE MA HOLDER OR ITS AFFILIATE(S).

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