Cost Model of Long-Term Prophylaxis Treatment in von Willebrand Disease

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Introduction

- Plasma-derived von Willebrand Factor (VWF)/factor VIII (FVIII) concentrates can be used as prophylaxis for individuals with von Willebrand Disease (VWD) when desmopressin is ineffective (1).
- The clinical effectiveness of VWF/FVIII concentrate is well-documented in patients with VWD by the literature (2).
- However, there is limited evidence regarding the cost implications of this treatment.

Objective

- To perform a cost analysis surey based on the dosage of available VWF concentrates in Spain (Hamate-P®, Wilate®, and Fandhi®) for long-term prophylaxis treatment in patients with VWD.

About the von Willebrand Disease

- VWD is a hereditary blood clotting disorder caused by the deficiency of VWF, an essential protein in the coagulation process (3).
- vWD affects men and women equally with a prevalence of up to 1% of the general population (4), and is classified into three types based on severity:
  - **Type 1** involves a quantitative deficiency of VWF and accounts for 70-80% of cases (5).
  - **Type 2** is characterized by dysfunctional VWF leading to reduced VWF antigen levels, affecting about 20% of patients (5).
  - **Type 3**, the most severe form, is marked by the absence of circulating VWF and occurs in less than 5% of cases (5).

Methods

- A cost analysis was carried out based on:
  - The dosage of VWF:Rco per kg of each plasma-derived VWF concentrate (5, 6), and the dosing regimen recommended in the SmPC (7, 8, 9).
  - Patients' weights were modelled continuously from 20 kg to 100 kg.
- Calculations were performed using a low and a high dose for each treatment. The specific dose used in the calculations are presented in Table 1.
- The unitary cost of each treatment was obtained from the official public database of the Spanish National Health System (see Table 2).

Assuming no vial sharing, the annual cost per patient was estimated using the following formula:

$$CP_y = C_u \cdot U_v \cdot M_v \cdot 104$$

- CP_y represents the annual cost per patient.
- C_u is the cost per IU.
- U_v represents the number of IU per vial.
- M_v is the minimum number of vials required to meet the dose for each administration.
- 104 corresponds to the number of administrations per year (52 weeks with two administrations per week).

Table 1. Range of doses for each treatment

Treatment	Low dose*	High dose*
Haemate-P® (a)	10 IU/kg	70 IU/kg
Fanhdi® (b)	10 IU/kg	70 IU/kg
Wilate® (b)	20 IU/kg	40 IU/kg

(a) Dosing according to SmPC, (b) Dosing according to published evidence (5, 6); IU: international units. * Range of doses tested from sources (a) and (b).

Table 2. Unit costs

Vial	€ vial	€ / IU VWF
Haemate-P® 1200 IU*	197.48	0.16
Fanhdi® 1200 IU*	396.96	0.33
Wilate® 1000 IU*	396.96	0.39

Source: BotPlus (9) and OSG (10); IU: international units; * IU of von Willebrand Factor

Results

Weight range 69–75 kg (low doses):
- Wilate®: €40,872 higher annual cost than Haemate-P®.
- Fanhdi®: €21,216 higher annual cost than Haemate-P®.

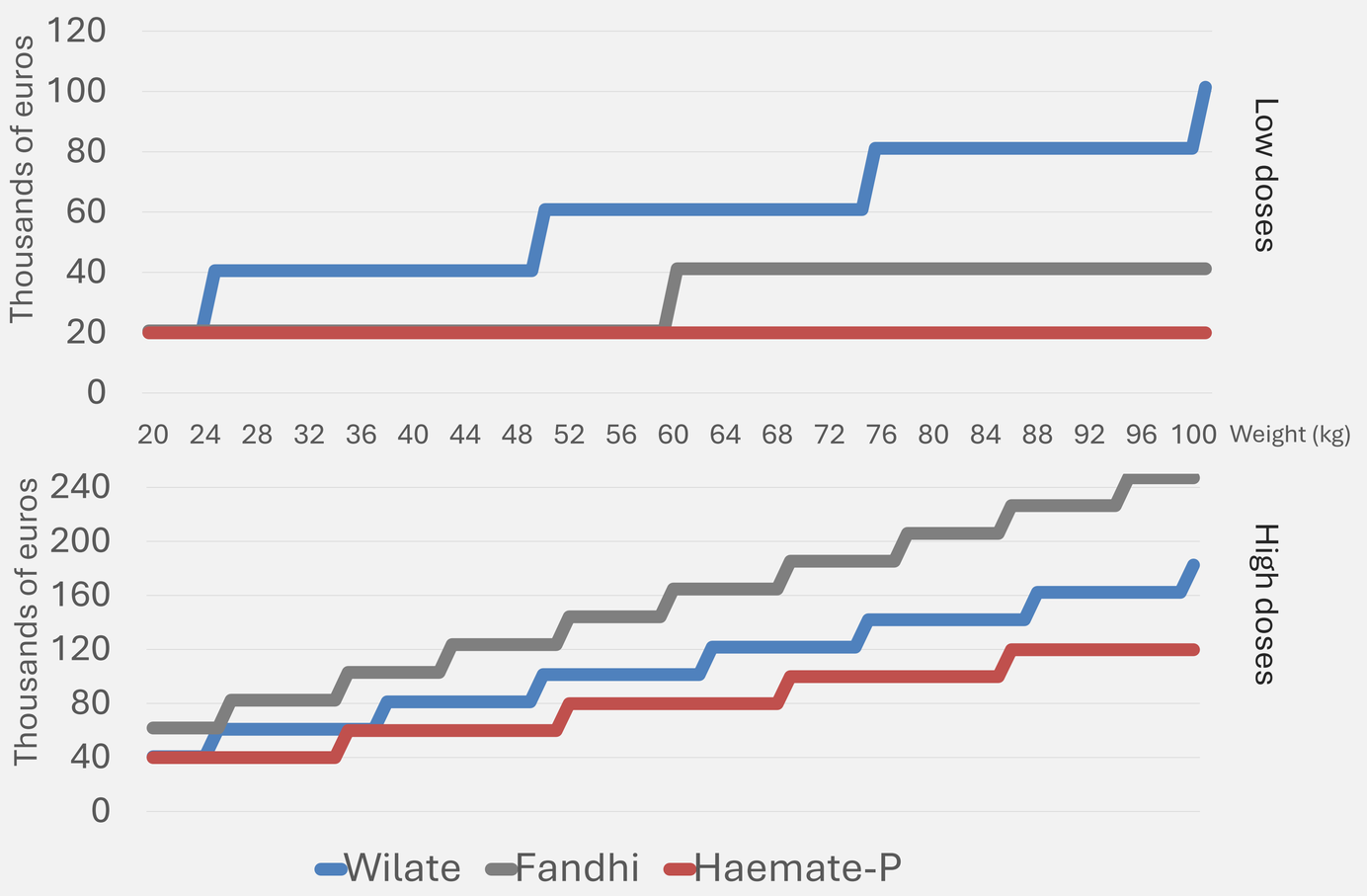
Weight range 69–75 kg (high doses):
- Wilate®: €21,840 higher annual cost than Haemate-P®.
- Fanhdi®: €85,488 higher annual cost than Haemate-P®.

Weight range 25–34 kg (low doses):
- Wilate®: €20,592 higher annual cost than Haemate-P®.
- Fanhdi®: €624 higher annual cost than Haemate-P®.

Weight range 25–34 kg (high doses):
- Wilate®: €20,904 higher annual cost than Haemate-P®.
- Fanhdi®: €42,432 higher annual cost than Haemate-P®.

In all scenarios, Haemate-P® was the most economic option for long-term prophylaxis compared to Wilate® and Fanhdi® (Figure 1). Both the total VWF IU consumption and the number of vials used per year were higher for Fanhdi® and Wilate® compared to Haemate-P®.

Figure 1. Cost per year



Conclusion

Long-term prophylaxis with Haemate-P® was more efficient than Fanhdi® and Wilate® in VWD treatment based on pharmacological cost acquisition. More pharmacoeconomic analysis is necessary to assess cost-effectiveness from society perspective in long-term prophylaxis in persons with VWD.

References

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Abbreviations

VWF: von Willebrand Factor, **FVIII:** Factor VIII, **VWD:** von Willebrand Disease, **VWD:Rco:** von Willebrand Factor Ristocetin Cofactor, **SmPC:** summary of producto characteristics,

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