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Budget impact analysis of Haemate-*P* as long-term prophylaxis and on-demand therapy for von Willebrand disease in Spain

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ABSTRACT

Objectives: A decision-analytic model was developed to assess the Spanish healthcare system budget impact associated with increased use of Haemate-*P* in individuals with von Willebrand disease.

Methods: The model considered long-term prophylaxis and on-demand treatment with each of three von Willebrand factor (vWF) replacement products (Haemate-*P*, Fanhdi, and Wilate). Two budget scenarios were considered: a status quo scenario (no changes in uptake of products/strategy) and an alternative scenario (increased Haemate-*P* uptake). The model estimated total product costs and other medical costs for each budget scenario annually over 3 years. Total budget impact was estimated by subtracting the costs for the status quo scenario from the costs for the alternative scenario.

Results: The alternative Haemate-*P* uptake scenario was estimated to save the Spanish healthcare system €5.64 million over 3 years. Cost savings were driven primarily by a reduction in vWF product costs (–€5.62 million) resulting from a reduction in vWF product use (–€4.80 million) and bleed reductions (–€0.82 million). In univariate and multiway deterministic sensitivity analyses, the alternative scenario remained cost-saving.

Conclusion: Haemate-*P* was found to be a cost-saving strategy, and increased use of Haemate-*P* over Fanhdi, and Wilate is expected to reduce overall costs to the healthcare system in Spain.

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Introduction

von Willebrand disease (vWD) is an inherited bleeding disorder with a prevalence of over 100 per 1,000,000 individuals within the Spanish population[1]. The disease is characterized by excessive bleeding events and a significant economic burden[2–5], with an estimated €481 million in annual costs for healthcare in Spain[6].

Management of vWD involves von Willebrand factor (vWF) replacement therapy. Individuals with vWD receive either on-demand treatment (ODT) to manage bleeding events when they occur, or they receive regular administrations for long-term prophylaxis (LTP) to prevent bleeds. Treatment with LTP has been shown to greatly reduce bleed events compared with ODT[7–10], and as such LTP is recommended in individuals with severe and frequent bleeds[11]. In Spain, three vWF products are commonly used as LTP and ODT to manage vWD: Haemate-*P*, Fanhdi, and Wilate.

Estimating the costs of care for individuals with vWD is important for determining the most efficient allocation of resources from a healthcare system. To date, limited economic analyses have been conducted in

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vWD[4,12–14]. As such, the objective of the analysis was to assess the budget impact to healthcare systems with increased use of Haemate-*P* in individuals with vWD from a Spanish perspective.

Methods

Model structure

An economic model was developed in Microsoft Excel to estimate the Spanish national healthcare system budget impact of increasing the use of Haemate-*P* in patients with vWD from Spain. The model first estimated the number of individuals with vWD eligible for treatment with any of the modeled vWF products (defined below), using a funnel-down approach based on disease prevalence data and other epidemiologic parameters. Within this eligible population, the model estimated healthcare costs over a 3-year horizon for two budget scenarios. The status quo scenario assumed a constant mix of vWF products (e.g. Haemate-*P*, Wilate, and Fanhdi) and therapeutic strategies (LTP and ODT), representing the current treatment approach for vWD in Spain. The "alternative" scenario assumed a mix of treatments and therapeutic strategies with increasing use of Haemate-*P* in both LTP and ODT. The modeled risk of bleeds (major and minor) and the associated cost of treating those bleeds was dependent on therapeutic strategy considered. In addition, the model calculated the cost of prophylaxis based on the specific treatment that was received. Annual costs (pharmacy, other medical, and total) were estimated for each budget scenario over a 3-year period. Costs were then compared across the two scenarios to estimate the total potential cost savings annually and over a 3-year horizon.

The model estimated medical costs associated with incremental increases in the use of Haemate-*P* over a time horizon of 3 years; outcomes were estimated both annually and in total across the 3-year period. Costs were presented undiscounted[15] in 2022 Euros[16]. Based on this perspective, this analysis included only direct medical costs (e.g. pharmacy and other medical costs for treatment of major and minor bleeds and outpatient physician visits).

Model inputs and data sources

Population characteristics

Using the epidemiologic data and the funnel-down approach (Table 1)[17,18], the model estimated that 379 individuals with vWD were eligible for LTP within the full Spanish population (47,435,597 individuals)[19], which was assumed constant over the 3-year time horizon. The population was assumed to have a mean weight of 74.34 kg[20].

Market share and therapeutic strategy inputs

To estimate the overall budget for the status quo and alternative scenario, the market share of each vWF product for each year was applied. For the status quo scenario, market share data by product and distribution among LTP and ODT were provided by CSL Behring based on market research[21] and assumed constant over 3 years. For the alternative scenario, an increase in the use of Haemate-*P* in both LTP and ODT was assumed from 36% in year 1 to 43% in year 3, with uptake coming equally from the other two products. Details on the market share estimates can be found in Table 2.

Cost inputs

The model included the costs of vWF products, and the costs of medical resource use associated with the treatment of bleeds; these costs are presented in Table 1. Treatment acquisition costs per vWF unit for each product were based on the list prices obtained from BotPLUS, which provides a list price of €197.48 per vial for each product (Haemate-*P* 500 IU FVIII:1,200 IU vWF; Wilate 500 IU FVIII:500 IU vWF; Fanhdi 405 IU FVIII:600 IU vWF)[22]. For Wilate's prophylaxis regimen, dosing level and frequency were obtained from Wilate's summary of product characteristics (SmPC) and were assumed equivalent for all treatments[23]. For Haemate-*P* and Fanhdi, it was assumed that dosing was similar to Wilate (Table 1).

The model also considered costs of vWF products used to treat bleed events for individuals in both the LTP and ODT strategies. Dosing data to treat bleeds were based on the SmPC data[23,24]. The recommended

Table 1. Model input parameters.

Epidemiologic parameters		Estimate		Sources/assumptions
Total population		47,435,597		INE[19]
vWD prevalence		0.0092%		Batlle et al.[17]
Distribution by vWD type				
Type 1		74.1%		Batlle et al.[17]
Type 2		20.5%		
Type 3		5.4%		
Percentage eligible for prophylaxis				
Type 1		0.0%		Assumption
Type 2		16.0%		Laffan et al.[18]
Type 3		100.0%		
Dosing parameters	**Haemate-*P***	**Fanhdi**	**Wilate**	**Sources/assumptions**
LTP dosing (IU/kg/week)	75	75	75	Wilate SmPC[23] dosing for Haemate-*P* and Fanhdi assumed equal to Wilate
Dosing per bleed event (IU/kg)				
Minor bleed	30	30	30	Wilate SmPC[23]; Haemate-*P* SmPC
Major bleed	66.67	45	45	Wilate SmPC[23,24]; Dosing for Fanhdi assumed equivalent to Wilate
Bleed parameters		**Minor bleed**	**Major bleed**	**Sources/assumptions**
Severity of bleeds				Lissitchkov et al.[8]
ODT		61.84%	38.16%	
Prophylaxis		70.00%	30.00%	
Resource use per bleed event				Assumption
Outpatient		25%	0%	
Inpatient		50%	50%	
Cost				
Outpatient visit		€200.87	€200.87	Oblikue[25]
Inpatient stay		€1,203.95	€1,203.95	CMBD[26]

[a]Median of 11 bleeds per year prior to prophylaxis, median of 1 bleed per year with prophylaxis[9].
[b]Incidence rate of 1.41 per patient-month prior to prophylaxis and 0.34 after prophylaxis[10].
[c]Median of 3.3 bleeding events per month prior to prophylaxis and a median of 1.1 events per month on prophylaxis[7].
Abbreviations. IU, international unit; kg, kilogram; LTP, long-term prophylaxis; ODT, on-demand treatment; SmPC, Summary of Product Characteristics; vWF, von Willebrand factor.

Table 2. Strategy distribution.

Strategy	Year 1	Year 2	Year 3
Distribution of therapeutic strategies			
Status quo scenario			
LTP (%)	13.08	13.08	13.08
ODT (%)	86.92	86.92	86.92
Alternative Haemate-P uptake scenario			
LTP (%)	13.08	13.08	13.08
ODT (%)	86.92	86.92	86.92
Product market shares			
Status quo scenario			
Haemate-*P* (%)	30.00	30.00	30.00
Wilate (%)	22.00	22.00	22.00
Fanhdi (%)	48.00	48.00	48.00
Alternative Haemate-P uptake scenario			
Haemate-*P* (%)	36.00	40.00	43.00
Wilate (%)	19.00	17.00	15.50
Fanhdi (%)	45.00	43.00	41.50

Abbreviations. LTP, long-term prophylaxis; ODT, on-demand treatment.

duration of treatment for bleeds from Haemate-*P* and Wilate's SmPC was at least 1 day for early hemarthrosis and 3–4 days for more extensive hemarthrosis[23,24]. Thus, it was estimated that the duration of treatment was 1 and 4 days for minor and major bleeds, respectively. Dosing is recommended to be infused every 12–24 h for at least 1 day[23,24]; therefore, two daily doses were assumed for all bleeds. The same dosing for the treatment of major and minor bleeds was assumed, regardless of whether the individual was receiving LTP or ODT.

Costs of other medical resources used for treatment of major and minor bleeds were estimated from the published literature[25,26]. Costs of an outpatient physician visit were estimated to be €200.87[25], while costs to treat major bleeds were estimated to be €1,203.95[26]. It was assumed that 25% of minor bleeds and 50% of major bleeds would require an outpatient visit. Similarly, it was assumed that 0% of minor bleeds and 50% of major bleeds would require hospitalization (Table 1). Due to the uncertainty of these estimates, scenario analyses were performed using lower and upper bound assumptions.

The number of bleeding events incurred was dependent on therapeutic strategy and vWF product. For ODT, baseline annualized bleeding rates (ABRs; low, medium, and high) were obtained from published studies in patients with vWD which report the bleeding rates prior to the use of prophylaxis (e.g. when they are treated on-demand)[7–9]. The severity of these bleeds was classified as minor or major using the definitions in Lissitchkov et al.[8]. Here, the authors defined major bleeding events as joint, muscle, brain, or mucosal gastrointestinal bleeds; all other bleeding events were classified as minor. Table 1 presents the baseline (i.e. without prophylaxis) annual bleed rates for each bleed type and distribution among bleed severity. For each LTP regimen, a relative risk analysis of bleeds was applied and compared with the ODT ABR, based on the published literature (Table 1)[7–9].

Due to the rarity of adverse events and lack of product-specific data, we have assumed equivalence among all comparators and therapeutic strategies and excluded adverse events; as such, no adverse event costs were considered in the model.

Sensitivity analysis

Due to the uncertainty of input parameters, a series of sensitivity analyses were performed. First, a deterministic one-way sensitivity analysis was conducted to assess the impact of parameter variation on the total 3-year incremental budget impact. In this analysis, the baseline ABRs varied from a low of 11.0 and a high of 39.6, and the expected resource use per bleed event was varied to upper and lower bounds outlined in Table 3. All other parameters were varied by ±20%. In addition to these one-way sensitivity analyses, multiway deterministic analyses were conducted. First, we set the use of LTP as constant over time (i.e. held the combined percentage of LTP use of each product at 13.08% for each year). We also included analyses setting the percentage of bleeds with inpatient and/or outpatient visits to extreme bounds (0 and 100%). Additionally, we included two multiway analyses assuming no product-specific differences in ABR relative risk of bleed with LTP for all three products to 0.09, and another by setting the ABR relative risks with LTP for all three products equal to 0.33. The parameter estimates used in this multiway analysis are presented in Table 3.

Results

Based on the input estimates outlined above, the total 3-year costs for each scenario can be seen in Table 4. The alternative Haemate-*P* uptake scenario was estimated to save the Spanish healthcare system €5.64 million over 3 years; this was driven primarily by a reduction in vWF product costs (−€5.62 million). The model estimated both a reduction in prophylaxis costs (−€0.82 million) resulting from the higher vWF:FVIII ratio for Haemate-*P* (2.4:1) and a reduction in bleed-related product costs (−€4.80 million), resulting from a combination of reduced bleed rates when using Haemate-*P* LTP (−€0.36 million) and the reduction in product costs per bleed (−€4.44 million) associated with Haemate-*P* (again due to the higher vWF:FVIII ratio). Other medical costs represented only a small portion of the budget impact (−€18,386). Total annual costs for each scenario can be seen in Figure 1; overall, cost savings increased from Year 1 to Year 3.

Figure 2 illustrates the findings of the deterministic one-way sensitivity analyses, presenting the ten most influential parameters. Baseline ABR, price of products, Haemate-*P* ODT dose, number of daily

Table 3. Parameter estimates considered in multiway analysis.

Scenario description	Base case estimate	Lower bound estimate	Upper bound estimate
Baseline annual bleed rate*	26.5 bleeds	11.0 bleeds	39.6 bleeds
Resource use per event*			
Minor bleeds	Outpatient: 25% Inpatient: 0%	Outpatient: 0% Inpatient: 0%	Outpatient: 100% Inpatient: 100%
Major bleeds	Outpatient: 50% Inpatient: 50%	Outpatient: 0% Inpatient: 0%	Outpatient: 100% Inpatient: 100%
Percentage receiving LTP in the alternative scenario	13.08% each year	13.08% in Year 1; 15.08% in Year 2; 17.08% in Year 3	N/A
Relative risk of bleeds with LTP	Haemate-*P*: 0.09 Wilate: 0.33 Fanhdi: 0.24	Haemate-*P*: 0.33 Wilate: 0.33 Fanhdi: 0.33	Haemate-*P*: 0.09 Wilate: 0.09 Fanhdi: 0.09

Abbreviations. LTP, long-term prophylaxis; N/A, not applicable.
*Included as part of univariate sensitivity analysis. All other modeled parameters were varied by ±20% in the univariate sensitivity analysis.

Table 4. Base case results: 3-year budget impact results by budget scenario for individuals with von Willebrand disease in Spain over a 3-year period.

Parameter	Status quo	Alternative	Incremental
Drug costs	€122,196,274	€116,575,102	−€5,621,172
Prophylaxis costs	€12,684,654	€11,861,712	−€822,942
Bleed-related treatment costs for LTP	€3,212,022	€2,849,457	−€362,565
Bleed-related treatment costs for ODT	€106,299,598	€101,863,933	−€4,435,665
Bleed-related treatment costs for LTP and ODT in total	€109,511,620	€104,713,390	−€4,798,230
Other bleed event costs	€8,041,676	€8,023,290	−€18,386
Outpatient visits	€1,872,519	€1,867,637	−€4,882
Hospitalizations	€6,169,157	€6,155,653	−€13,504
Overall costs	€130,237,949	€124,598,392	−€5,639,558

Abbreviations. LTP, long-term prophylaxis; ODT, on-demand treatment.

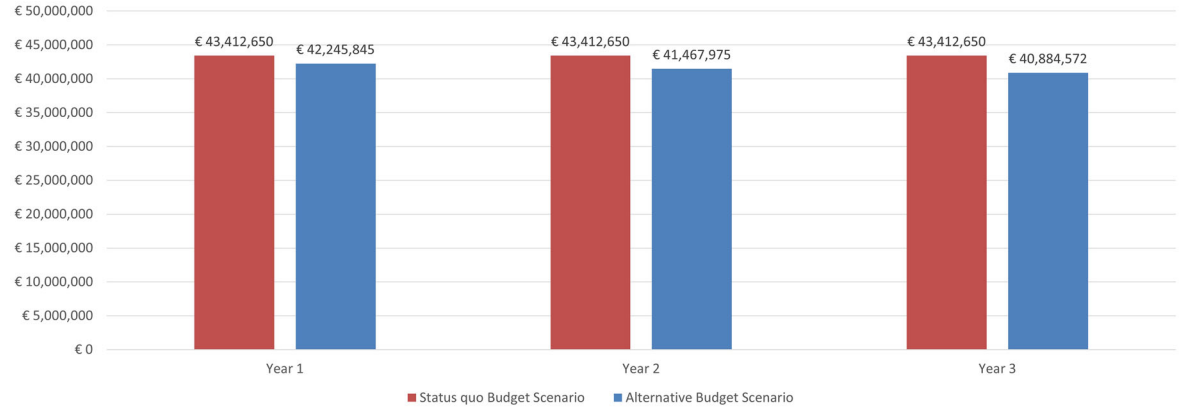

Figure 1. Base case results: total annual costs by year and budget scenario. Results represent the total vWD-related direct medical costs incurred by 379 individuals with vWD in Spain.

doses, duration of treatment for major bleeds, vWD prevalence, patient weight, and Wilate ODT dose were among the most influential model parameters. Baseline ABR had the largest impact on cost savings, with 3-year cost savings ranging from −€2.82 million (assuming an ABR of 11.0) to cost savings of −€8.02 million (assuming an ABR of 39.6). The alternative scenario remained cost-saving in all deterministic univariate sensitivity analyses.

Results of the multiway deterministic sensitivity analyses are presented in Table 5. Increasing the overall use of LTP over time was expected to result in a slight increase in total cost savings compared with holding the current distribution of ODT versus LTP treatment constant (−€5,904,512 vs. −€5,639,558). Setting the ABR relative risks in LTP equal between the three products decreased the cost savings slightly. However, the alternative scenario was the cost-saving strategy, with cost savings of at least €5.31 million across the 3-year period (Table 5) in all multiway deterministic sensitivity analyses.

Discussion

This analysis evaluated the potential budget impact for Spain's national healthcare system based on increasing the use of Haemate-*P* for LTP and ODT in individuals with vWD. This analysis suggests that increasing the use of Haemate-*P* to replace the use of Wilate and Fanhdi in LTP or ODT will result in cost savings to the healthcare system. Sensitivity analyses confirmed the base case findings, as the uptake of Haemate-*P* was cost-saving in all parameter variation. Increasing the assumed use of LTP resulted in greater healthcare system cost savings. Increasing the relative risk of bleeds with LTP reduced the overall cost savings. The model was most sensitive to the baseline ABR. Crucially, the increased use of Haemate-*P* resulted in substantial estimated cost savings, regardless of parameter variation.

While all three vWF products considered in this analysis have been found in clinical studies to reduce bleed risk in individuals with vWD, Haemate-*P* has a higher concentration of vWF to FVIII ratio (2.4:1) than either Wilate (1:1) or Fanhdi (1.48)[27]. As such, LTP and/or ODT for individuals with vWD, Haemate-*P* is likely to be cost-saving compared with the use of either Wilate or Fanhdi, by virtue of requiring fewer vials of

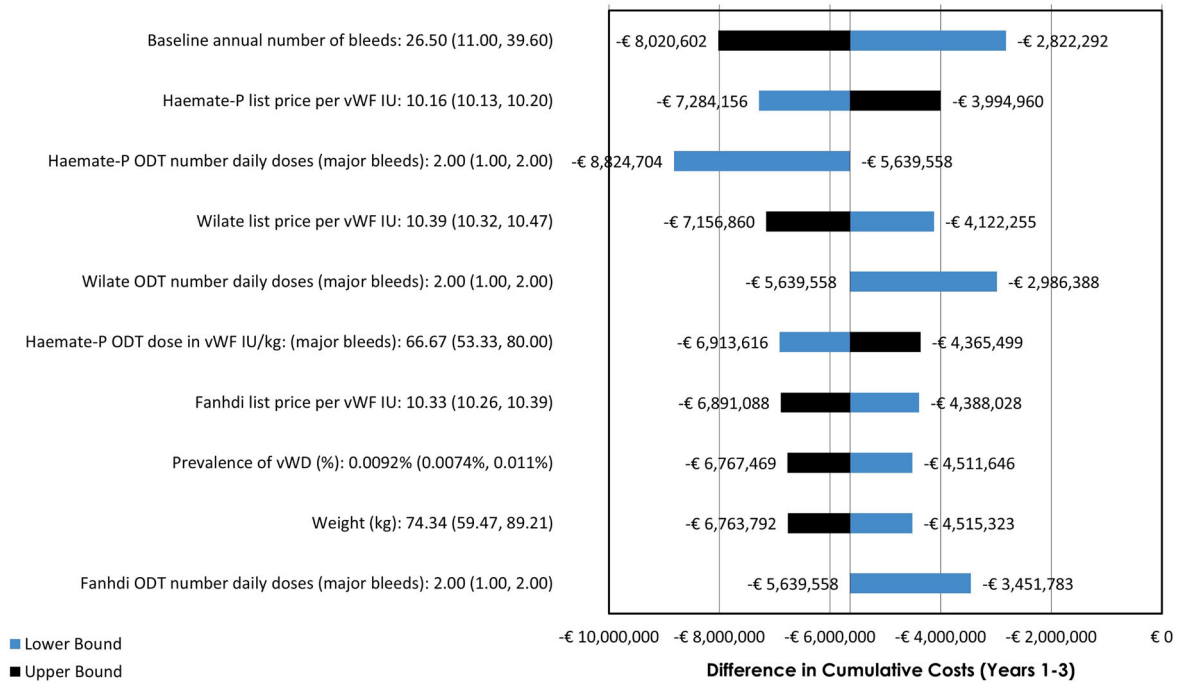

Figure 2. One-way sensitivity analysis results: impact of parameter variation on 3-year budget impact results. Results of the univariate deterministic sensitivity analysis are presented. Black bars represent the difference in 3-year budget impact results due to varying parameter estimates from the base case value to the upper bound value. Blue bars represent the difference in results from base case value to the lower bound value. The tornado diagram presents the results for the ten parameters whose variation had the largest effect on the overall budget impact. Abbreviations. IU, international unit; kg, kilogram; ODT, on-demand treatment; vWD, von Willebrand disease; vWF, von Willebrand factor.

Table 5. Multiway sensitivity analysis: incremental costs by year for the Spanish vWD population over a 3-year period.

Analysis	Year 1	Year 2	Year 3	Total
Base case	−€1,166,805	−€1,944,675	−€2,528,078	−€5,639,558
Increasing total share of LTP	−€1,166,805	−€2,027,335	−€2,710,372	−€5,904,512
Percentage of bleeds with inpatient and/or outpatient stays				
0% for all bleeds	−€1,163,001	−€1,938,335	−€2,519,835	−€5,621,171
100% for all bleeds	−€1,184,736	−€1,974,559	−€2,566,927	−€5,726,222
ABR relative risk estimate				
All LTP regimens ABR relative risk = 0.09	−€1,099,206	−€1,832,010	−€2,381,614	−€5,312,830
All LTP regimens ABR relative risk = 0.33	−€1,129,123	−€1,881,871	−€2,446,432	−€5,457,426

Base case analysis assumes baseline ABR of 26.5 and resource use as outlined in Table 1. Incremental costs are the total annual costs for the "alternative" scenario minus the "status quo" scenario. The "increasing total share of LTP" case represents a case where the total use of LTP increases from 13.08% in Year 1 to 15.08% in Year 2 and 17.08% in Year 3, holding all else from the base case constant.
Abbreviations. ABR, annualized bleed rate; LTP, long-term prophylaxis.

product to provide comparable increases in vWF (due to Haemate-P having a higher ratio of vWF to FVIII). This is demonstrated by this analysis, whereby an increased use of Haemate-*P* was shown to provide greater cost savings, based on the assumption of no differences in the impact on bleed risk between the vWF products.

To our knowledge, this analysis is the first to estimate the budget impact of vWF treatments, and the first to do so from a Spanish perspective. A previous study illustrated the economic value of prophylaxis from United States perspective[12], and found LTP to be a cost-saving strategy. Although the setting is different, our results (Table 4) are consistent with the previous study in its findings that LTP was found to provide the optimal cost-saving strategy. This study is the first to provide insight into the budget impact of vWF replacement therapy in Spain for patients with vWD and highlights the potential cost savings associated with the increased use of Haemate-*P* using LTP.

This analysis is subject to several limitations. First, the costs of all vWF products represented over 90% of the costs in the model. As such, assumptions regarding the dosing schedule for prophylaxis would have substantial implications on the budget impact results, as these parameters affect the total product use. Additionally, due to the absence of availability of the confidential negotiated prices for all

comparators, we used list price for each product. The actual prices to the Spanish government may differ. We included sensitivity analysis on these prices to assess the potential impact of price on results. Additionally, data for the baseline ABRs and bleed risk reduction for the prophylaxis regimens and the dosing required to treat bleeds are also limited. Studies used to obtain efficacy measurements had small sample sizes, which may be problematic in a vWD population as bleed risk is highly variable. Furthermore, the lack of direct head-to-head comparisons among clinical studies makes comparisons between products difficult. Lastly, substantial heterogeneity among the clinical trials, including potential variation in vWD severity, possible different definitions of bleeds across clinical studies, and different baseline bleed risks observed between study populations. Finally, there is uncertainty regarding the market share assumptions, which affect the drug costs both directly (as the price of each product varies substantially) and indirectly (the estimated reduction in bleed risk for prophylaxis differs among products).

Based on the findings of this analysis, due to the 2.4:1 vWF:FVIII ratio, annual costs for individuals treated with Haemate-*P* are anticipated to be lower than those receiving Fanhdi or Wilate. LTP with Haemate-*P* is expected to reduce the risk of bleeds experienced by individuals with vWD; as such, increasing the use of Haemate-*P* in LTP and ODT for individuals with vWD is expected to increase the cost savings to the healthcare system in Spain.

Transparency

Declaration of funding

This study was conducted by RTI Health Solutions under the direction of CSL Behring and was funded by CSL Behring.

Declaration of financial/other relationships

LVB, KK, PM, MP, and SY are employees of CSL Behring. MRW and CM are employees of RTI Health Solutions. JEMV reports these potential conflicts of interest: research grant, speaker's bureau, advisory board by CSL Behring. GE has received honoraria/consultant fees from Bayer, BMS, Boehringer Ingelheim, CSL Behring, Grifols, and Terumo (BCT). Peer reviewers on this manuscript have no relevant financial or other relationships to disclose.

Author contributions

MW, PM, and SY were responsible for study design, data extraction, data interpretation, analysis, interpretation of analysis, preparation of manuscript, and review/approval of manuscript; LVB was responsible for data extraction, data interpretation, and review/approval of manuscript; CM was responsible for study design, data extraction, analysis, preparation of manuscript, and review/approval of manuscript; and JEMV, GE, KK, and MP were responsible for data interpretation, interpretation of analysis, and review/approval of manuscript.

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