PBI25

COST OF STANDARD HALF-LIFE VERSUS EXTENDED HALF-LIFE RECOMBINANT COAGULATION FACTOR VIII IN PATIENTS WITH HAEMOPHILIA A IN SPAIN: RESULTS FROM A PROBABILISTIC MODEL

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OBJECTIVES

- A Real-World study in the United States (USA) found the median recombinant coagulation factor VIII (rFVIII) international units (IUs) dispensed to treat haemophilia A patients were between 10% and 45% higher with extended half-life (EHL) versus standard half-life (SHL) products.
- The objective was to compare the cost of SHL versus EHL rFVIII in Spain.

METHODS

- A probabilistic model (second-order Monte Carlo simulation) in a hypothetical cohort of 1,000 patients with haemophilia A and a time horizon of 1 year was carried out[1].
- The mean IUs dispensed quarterly with each rFVIII were taken from a Real-World study[2], conducted in two USA databases, which included 776 patients with haemophilia A (Table 1).
- The frequency of use of each recombinant rFVIII was obtained from sales estimation (IQVIA) of a 12-months period in Spain[3] (Table 2).
- rFVIII prices (€ 2019) were obtained from the Reference Price Order[4] by the National Health System.
- A base case analysis, considering the use of rFVIII in the clinical practice in Spain and two sensitivity analyses were conducted.
- Sensitivity analysis 1: The frequency of use of each rFVIII in the United States observed in the real-world analysis, was obtained from a Real-World study by Chhabra et al.[2] (Table 3).
- Sensitivity analysis 2: The IUs of rFVIII dispensed quarterly in clinical practice before (pre) and after (post) switching from SHL to EHL, were taken from the study by Chhabra et al.[2] (Table 4).

Table 1. Mean IUs of the rFVIII dispensed quarterly in clinical practice[2].

Cohort	Database	N*	Mean IUs per quarter	SD*	Distribution	Alpha**	Beta**
SHL	Optum	243	75,255	61,847	Gamma	1.48	50827.87
SHL	Truven	415	60,198	60,846	Gamma	0.98	61500.98
SHL	Total	658	135,453	122,693	Gamma	1.22	111135.02
EHL	Optum	33	73,696	40,875	Gamma	3.25	22671.05
EHL	Truven	89	72,746	35,254	Gamma	4.26	17084.71
EHL	Total	122	146,442	76,129	Gamma	3.70	39576.25

*Some individuals received an EHL and an SHL factor VIII replacement product during the study period and are included in both cohorts; **The SDs and the alpha and beta statistics were calculated from the Chhabra et al. study data[2]. Abbreviations: EHL: rFVIII with extended elimination half-life; IUs: international units; rFVIII: recombinant coagulation factor VIII; SD: standard deviation; SHL: rFVIII with standard elimination half-life.

Table 2. Frequency of use of rFVIII in the last 12 months in Spain[3].

rFVIII	Mean	Range		SD*	Distribution	Alpha*	Beta*
SHL							
Lonoctocog alfa CHO (Afstyla®)	2.2%	1.9%	2.4%	0.1%	Beta	375	16977
Moroctocog alfa CHO (Refacto AF®)	31.7%	28.5%	34.9%	1.6%	Beta	262	564
Octocog alfa CHO (Advate®)	32.7%	29.4%	35.9%	1.7%	Beta	258	532
Octocog alfa BHK (Kogenate®/Helixate®)	8.9%	8.0%	9.8%	0.5%	Beta	350	3564
Octocog alfa BHK (Kovaltry®)	18.0%	16.2%	19.8%	0.9%	Beta	314	1439
Simoctocog alfa HEK (Nuwiq®)	2.0%	1.8%	2.2%	0.1%	Beta	376	18640
Turoctocog alfa CHO (Novoeight®)	4.6%	4.1%	5.1%	0.2%	Beta	366	7602
EHL							
Efmoroctocog alfa HEK (Elocta®)	99.3%	89.4%	100%	2.7%	Beta	8.13	0.06
Rurioctocog alfa pegol CHO (Adynovi®)	0.70%	0.60%	0.75%	2.78%	Normal	384	0.00002

*The SDs and the alpha and beta statistics were calculated using the data provided by the market study[2]. Abbreviations: BHK: baby hamster kidney; CHO: Chinese hamster ovary; EHL: rFVIII with extended elimination half-life; HEK: human embryonic kidney; rFVIII: recombinant coagulation factor VIII; SD: standard deviation; SHL: rFVIII with standard elimination half-life.

Table 3. Frequency of use of rFVIII in the USA in the real-world[2].

rFVIII	Mean	Range		SD*	Distribution	Alpha*	Beta*
SHL							
Moroctocog alfa	36%	31%	41%	2.58%	Beta	125.42	219.95
Octocog alfa	59%	55%	63%	1.87%	Beta	407.22	284.90
Simoctocog alfa	3%	3%	3%	N/A	N/A	N/A	N/A
Turoctocog alfa	2%	0%	3%	0.80%	Beta	3.77	237.25
EHL							
Efmoroctocog alfa	83%	75%	91%	4.08%	Beta	69.47	14.23
Rurioctocog alfa pegol	17%	25%	9%	4.08%	Beta	14.23	69.47

*The SDs and the alpha and beta statistics were calculated using the data provided by the study[2]. Abbreviations: EHL: rFVIII with extended elimination half-life; N/A: not applicable; rFVIII: recombinant coagulation factor VIII; SD: standard deviation; SHL: rFVIII with standard elimination half-life.

Table 4. IUs of rFVIII dispensed quarterly in clinical practice before (pre) and after (post) switching from SHL to EHL[2].

Cohort	Database	No. of quarters	Mean IUs per quarter	SD*	Distribution	Alpha*	Beta*
PRE (SHL)	Truven	9	52,396	7,143	Gamma	53.8	973.7
POST (EHL)	Truven	9	75,486	23,723	Gamma	10.1	7,455.2

*The SDs and the alpha and beta statistics were calculated from the Chhabra et al. study data[2]. Abbreviations: EHL: rFVIII with extended elimination half-life; IUs: international units; rFVIII: recombinant coagulation factor VIII; SD: standard deviation; SHL: rFVIII with standard elimination half-life.

RESULTS

- In the base case analysis, an average annual saving per patient treated with SHL of € 11,227 was obtained, with a 75.5% saving probability (Table 5).
- The results were stable for sensitivity analyses performed (Table 5).
- In the event that there was no change in treatment from the SHL to the EHL, there would be a greater annual saving per patient (€ 53,079) with a 99.9% saving probability (Table 5).
- When applying the rFVIII frequency of use in the clinical practice in the USA, the annual savings per patient treated with SHL instead of EHL would amount to € 16,350, with a saving probability of 79.9% (Table 5).

Table 5. Average annual costs (± SD) per patient with haemophilia A treated with SHL or EHL and saving probability with SHL.

Analysis	Characteristics	SHL	EHL	Saving with SHL	Saving probability
Base case	Frequency of use of rFVIII in Spain	€159,629 ± €157,205	€170,856 ± €89,087	-€11,227	75.5%
Sensitivity analysis 1	Frequency of use of rFVIII in the United States	€160,106 ± €158,377	€176,456 ± €117,244	-€16,350	79.9%
Sensitivity analysis 2	Pre (SHL) vs post (EHL) analysis	€124,097 ± €23,372	€177,176 ± €56,033	-€53,079	99.9%

*Abbreviations: EHL: rFVIII with extended elimination half-life; rFVIII: recombinant coagulation factor VIII; SD: standard deviation; SHL: rFVIII with standard elimination half-life.

CONCLUSIONS

The probabilistic model found higher expenditures over one year for haemophilia A patients using EHL versus SHL products. The use of SHL instead of EHL, would amount to cost savings to the National Health System in Spain. The findings of this analysis could be considered alongside a thorough clinical evaluation when determining an optimal therapeutic strategy.

REFERENCES

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