EE 569

Cost-Effectiveness of Eftrenonacog Alfa in Treatment of Severe Haemophilia B Patients in Turkey

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INTRODUCTION

Hemophilia B is a rare genetic disorder in which blood does not coagulate normally due to a deficiency or absence of clotting factor IX (FIX) (Srivastava et al, 2013). The tendency to bleed spontaneously or following a trauma or surgery is the characteristic symptom of the disease. Treatment involves replacing the missing FIX and can be both on prophylaxis or on-demand basis. International guidelines recommend the use of prophylaxis with both recombinant or plasma derived FIX (Council of Europe, 2017; Srivastava et al, 2013).

OBJECTIVES

The objective of this study is to assess the cost-effectiveness of rFIXFc (recombinant factor IX Fc fusion protein) in prophylactic treatment of severe haemophilia B patients in comparison to recombinant FIX (rFIX) treatment from the Turkish healthcare payer perspective.

METHODS

A cost-effectiveness model was developed to capture the long-term costs and outcomes of treatment of severe haemophilia B with rFIXFc and rFIX (Figure 1). Efficacy data for rFIXFc were obtained from the B-LONG (Powell et al, 2013), Kids B-LONG (Fischer et al, 2017) and B-YOND clinical trials (Pasi et al, 2017). An indirect comparison was made to compare the effectiveness data of rFIXFc and rFIX. QALYs and Pettersson Score were used as the primary outcomes. The cost components in the model were factor acquisition costs and cost of resolving bleeding episodes. The time horizon of the model was 70 years and all outcomes and costs were discounted by 3%. The cohort start age is 6 years.

Figure 1: Cost Effectiveness Model

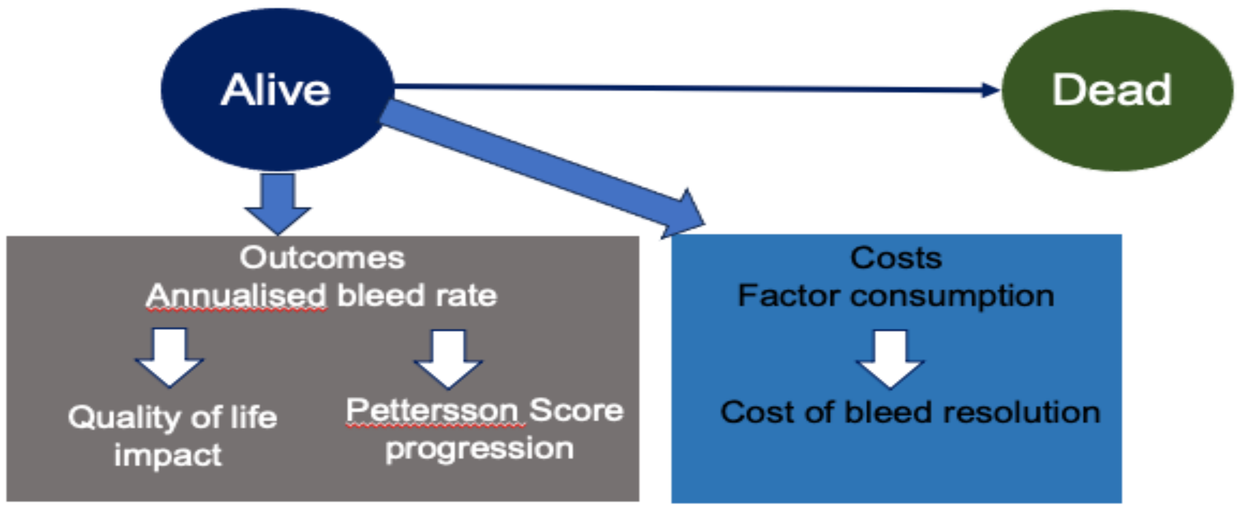


Table 1: Model Inputs

Parameter	Value
Discount rate	3%
Time horizon	70 years
Mean annual bleeding rate rFIXFc (Powell et al, 2013)	3.07
Mean annual bleeding rate rFIX (Pooled analysis)	3.79
Baseline Pettersson Score	6.50
Proportion bleeds joint bleeds (Berntorp et al, 2014)	
Aged 0-12	0.21
Aged 12-18	0.50
Aged 12+	0.62
Prophylaxis, weekly mean dose, IU/kg	
rFIXFc (Powell, 2013)	46.26
rFIX (Roth, 2001)	100.75
rFIX (Lambert, 2007)	129.68
Bleed resolution (mean total dose per bleed, IU/kg)	
rFIXFc (Powell, 2013)	53.5
rFIX (Roth, 2001)	71.49
rFIX (Lambert, 2007	101.68
Dosing frequency (weekly)	
rFIXFc (Powell, 2013)	1.00
rFIX (Roth, 2001)	2.50
rFIX (Lambert, 2007	2.00

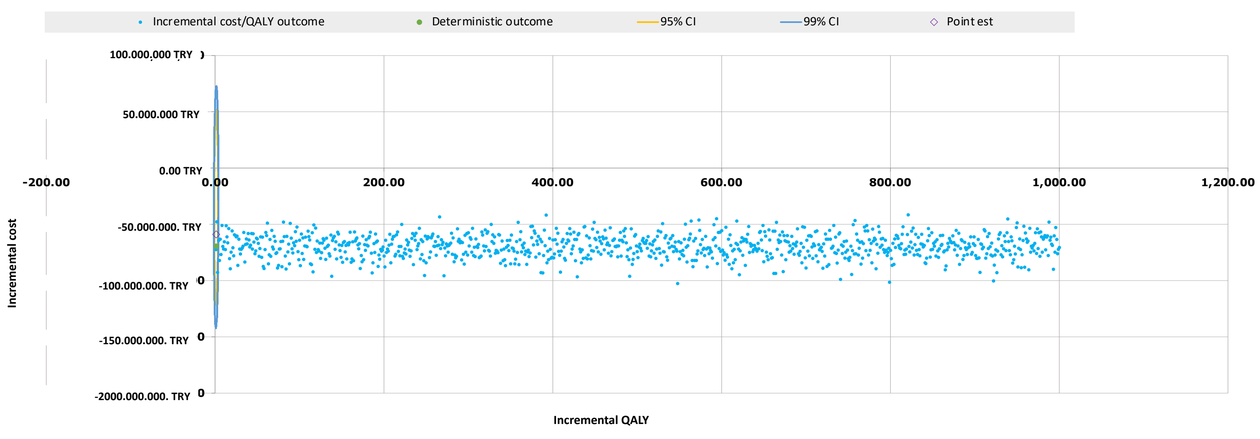
RESULTS

Table 3: Cost-Effectiveness Analysis Results

Parameter	rFIXFc	rFIX	Incremental
Total cost of bleeding episodes (TRY)	4,727,295	6,725,624	-1,998,329
Total cost of prophylaxis (TRY)	58,129,779	95,147,541	-37,017,762
Total cost (TRY)	74,200,657	133,251,610	-59,050,952
Total QALY	27.14	25.90	1.25
Final Pettersson Score	15.50	18.50	3.00
ICER	**Dominant**		

The final Pettersson score was 15.50 for rFIXFc and 18.50 for rFIX indicating better outcomes for rFIXFc The score increases one unit for every 12.6 joint bleeds. rFIXFc yielded 27.14 QALYs whereas the QALY for rFIX was 25.90 (incremental QALY: 1.25). The total cost of prophylaxis treatment was 74,200,657 TRY for rFIXFc and 133,251,610 TRY for rFIX.

Figure 2: Scatter Plot of the Probabilistic Sensitivity Analysis



Both one-way (OWSA) and probabilistic sensitivity analyses were undertaken to evaluate the robustness of the results. The probabilistic sensitivity analysis was run for 2000 iterations and the dominance of rFIXFc was maintained in all cases. Figure 2 presents the scatter plot of these results.

CONCLUSION

The cost-effectiveness analysis results have shown the dominance of rFIXFc over rFIX in treatment of severe haemophilia B patients in Turkey. In other words, rFIXFc achieved better outcomes with lower costs. The sensitivity analysis results have indicated that the findings were robust. rFIXFc is a cost-effective option in prophylaxis treatment of severe haemophilia B patients in Turkey.

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Disclosures: MT has received consultancy fees from Sobi, KE is employee of Proceutica, AE is employee of Sobi, AM is employee of Sobi

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