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Budget Impact of Efmoroctocog Alfa in Treatment of Severe Hemophilia A Patients in Turkey

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INTRODUCTION

Hemophilia A is a rare, inherited, life-long bleeding disorder characterized by bleeding due to reduced levels of clotting factor VIII (FVIII) (Srivastava et al, 2013). Replacing FVIII is the current standard of care supported by major clinical guidelines (Keeling et al, 2008; Rocino et al, 2014; Teitel, 1998). Patients can receive both prophylactic and on-demand therapy. Efmoroctocog alfa is a recombinant fusion protein (recombinant VIII-Fc fusion protein referred to herein as rFVIIIFc) that provides extended half-life factor therapy and thus remain in the body longer. The efficacy and safety of rVIIIFc is established in clinical studies involving adults and children with hemophilia A [A-LONG (Mahlangu et al, 2014), Kids A-LONG (Young et al, 2015), ASPIRE (Nolan et al, 2016). Clinical study results have shown lower annual bleeding rates (ABR) with reduced number of injections compared to rFVIII products (Mahlangu et la, 2014; Nolan et al, 2016; Young et al 2015).

OBJECTIVES

The objective of this study is to explore the budget impact of efmoroctocog alfa (rFVIIIFc) prophylaxis treatment in severe hemophilia A patients in comparison to recombinant FVIII (rFVIII) treatments in Turkey.

METHODS

A budget impact model based on the clinical effectiveness data for rFVIIIFc and rFVIII was designed. Number of patients with severe hemophilia A, their age distribution and number of patients in prophylaxis treatment were estimated from published epidemiological data. Annual bleeding rates and number of units needed to treat a bleeding episode were taken from literature. The analysis was made from the Turkish healthcare payer perspective (SSI). Only reimbursement prices of drugs were used in the analysis. The following steps were followed in the model:

1. Average per IU reimbursement prices of rFVIII and rFVIIIFc were calculated.
2. Number of patients, number of severe patients, their age distribution and number of patients under prophylactic and on-demand therapy were calculated from the epidemiological data published in World Federation of Hemophilia reports (Table 1).
3. Annual bleeding rates for rFVIIIFc and rFVIII on prophylaxis treatment were taken from Bullement et al (2020) (Table 2).
4. Number of annual bleedings for <18 and >18 age groups were calculated (Table 3).
5. Average weight of patients were estimated from a study in Turkey (Malhan et al, 2021) (74.1 kg for adults, 37.9 kg per paediatric patients).
6. Number of IUs needed to treat the bleedings under prophylactic therapy were calculated based on findings of Bullement et al (2019) (42.75 IU/kg per adults, 57.99 IU/kg per paediatric patients) (Table 4).
7. Total cost of prophylactic treatment for rFVIIIFc and rFVIII were calculated based on the findings of above stages (Table 5)

RESULTS

Table 1: Number of Hemophilia Patients in Turkey*

	2022
Total population	85,410,000
Number of hemophilia A patients	5,082
Number of severe patients by age	
0-4	213
5-13	711
14-18	462
19-44	1,672
45+	498
Total number of severe patients	3,557
Number of patients on prophylaxis	
<18	1,110
>18	1,627
Number of patients on-demand	
<18	277
>18	542

* All epidemiological data are from World Federation of Hemophilia, 2015

Table 2: Annual Bleeding Rates by Age Group (Prophylaxis Treatment)

	<18	>18
rFVIII	4.0	3.25
rFVIIIFc	1.96	2.90

Bullement et al, 2020

Number of severe hemophilia A patients was estimated as 3,557 for 2022. This population was divided into <18 and >18 age groups and then was further divided into patients under prophylaxis and on-demand therapy. There were 1,110 patients in the <18 prophylaxis group and 1,627 patients in the >18 prophylaxis group. Annualized bleeding rates for each group were taken from the literature.

Table 3: Number of Annual Bleedings by Age Group in Turkey (Prophylaxis Treatment)

	<18		>18	
	rFVIIIFc	rFVIII	rFVIIIFc	rFVIII
Number of Annual Bleedings	2,175	4,440	4,720	5,289

There were 2,175 and 4,440 annual bleedings in the <18 age group for rFVIIIFc and rFVIII respectively. The number of bleedings for >18 was 4,720 (rFVIIIFc) and 5,289 (rFVIII). Required dose for treatment of a bleeding for adults and children were taken from the literature (for adults 41.75 IU/kg, for children 57.99 IU/kg). Average weight for adults was taken as 74.1 for adults and 37.9 for children from a recent survey in Turkey. Based on this data, total annual cost saving by using rFVIIIFc was estimated

Table 4:IU Usage Needed to Treat Bleedings During Prophylaxis Treatment

	<18		>18	
	rFVIIIFc	rFVIII	rFVIIIFc	rFVIII
Usage Comparison	0.49	1	0.89	1

Table 5: Annual Total Cost of Prophylactic Treatment of Hemophilia A Patients to the SGK by Age Group (TRY)

	<18		>18	
	rFVIIIFc	rFVIII	rFVIIIFc	rFVIII
Total Cost	14,141,399	30,437,600	43,187,014	51,044,934
Budget Impact	-16,296,201		-7,857,920	
Total Saving	**24,154,121**			

CONCLUSION

Use of rFVIIIFc in prophylaxis treatment of severe hemophilia A is a cost saving option in Turkey. This saving arises from lower ABR, reduced annual consumption and lower number of IUs needed to treat bleedings that occur during treatment

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