PSY112: Factor Utilization Analysis of Extended Duration and Standard-Acting Recombinant FVIII Treatments for Severe Hemophilia A in Sweden

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OBJECTIVE

To compare the rFVIII utilization in international units (IU) of BAY 94-9027 (Jivi®) with other extended duration or standard-acting recombinant FVIII products used for prophylactic treatment of hemophilia A in Sweden

CONCLUSIONS

Assuming no difference in clinical outcomes, treatment with BAY 94-9027 utilizes the fewest units of clotting factor over a lifetime, compared to other relevant treatments in Sweden. This has implications for the healthcare budget.

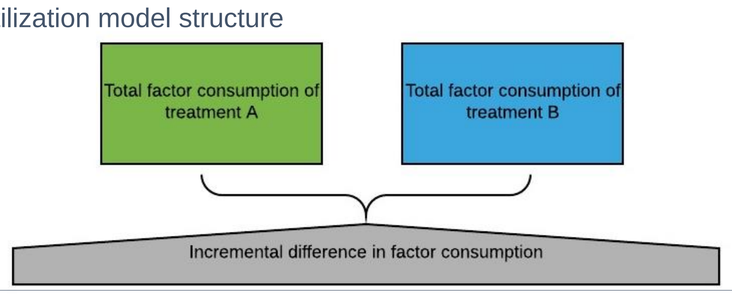
INTRODUCTION

- Hemophilia A is a congenital bleeding disorder resulting from deficiency of the plasma coagulation factor VIII, which occurs with an incidence of 1 in 5000 male births.[1] Management of hemophilia A has undergone a paradigm shift with the introduction of routine prophylaxis as a means of increasing plasma FVIII levels to prevent spontaneous bleeding and resulting joint damage.[2] Despite major advances in the management of hemophilia A, conventional rFVIII products have virtually indistinguishable pharmacokinetic profiles with a relatively short half-life of approximately 12 hours.[3] More recently, extended duration rFVIII products have been introduced to offer comparable factor VIII activity levels with a lower dose or less frequent dosing than earlier generation rFVIII replacement products.[4]
- BAY 94-9027 is a new prolonged half-life site specifically PEGylated B domain deleted (BDD) recombinant factor VIII (rFVIII) product. The PROTECT VIII study showed that BAY 94-9027 provides effective protection against bleeds and is well tolerated with twice-weekly, every 5-day, and every 7-day prophylaxis in patients with severe hemophilia A.
- The Swedish HTA body, the Dental and Pharmaceutical Benefits Agency (TLV) have concluded that all standard half-life (SHL) products are comparable as to effect and consumption. The EHL products, rFVIIIFc and BAX 855 however, were differentiated.[5]

METHODS

- A rFVIII utilization analysis was conducted from the Swedish perspective using a utilization model (Figure 1). The analysis assumed equivalent efficacy between treatments informed by an indirect comparison against other FVIII treatments.[6]
- The model captures factor utilization for the prophylactic treatment of severe hemophilia A with a median annual bleed rate (ABR) for joints of 0.[7]
- A scenario analysis was conducted to assess the factor utilization with a median ABR of 3.7 as reported in the indirect comparison as this might be more relevant internationally.[6]
- Prophylaxis utilization was modelled as the midpoint of the range of all dosing regimens as informed by products' summary of product characteristics. Swedish weight data were also retrieved to calculate the utilization for the population of interest.[8]
- Patients enter the model at treatment initiation and are assumed to remain on treatment for the duration of their lifetime. Individuals with hemophilia in Sweden have a similar life expectancy to those without hemophilia, therefore background mortality risk for patients within the model was derived from Swedish lifetable data.[9,10]
- Patients entered the model aged 12 years, and underwent a prophylaxis dose reduction (assumed to be 10%) at age 20, in accordance with expert opinion[11,12]. The model outcome was lifetime factor utilization.

Figure 1. Utilization model structure



RESULTS

- There is a reduction in the total number of factor units utilized over a lifetime horizon by those treated with BAY 94-9027 when compared to other extended duration products and to standard half-life (SHL) rFVIII products (Table 1). Over a lifetime horizon, a patient treated with BAY 94-9027 was estimated to utilize 17,972,360 IU compared to 23,325,727 IU and 22,429,505 IU for the other extended duration products, rFVIIIFc and BAX 855 respectively. These figures account for both prophylactic treatment and, in the scenario, the treatment of breakthrough bleeds. As the TLV does not consider there to be any difference in consumption between SHL-products, they are presented as a group. Patients treated with SHL products were estimated to utilize 23,234,667 units over their lifetime.

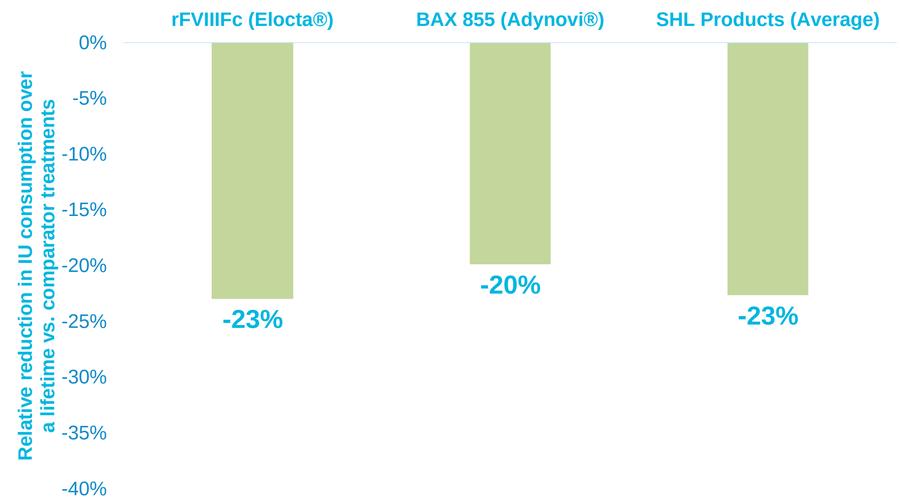
Table 1. Lifetime factor utilization across rFVIII products

Treatment	Lifetime factor utilization	Incremental reduction in factor utilization with Jivi vs. comparator
Extended duration		
BAY 94-9027 (Jivi®)	17,972,360	
rFVIIIFc (Elocta®)	23,325,727	5,353,367
BAX 855 (Adynovi®)	22,429,505	4,457,145
Standard half-life		
Average*	23,234,667	5,262,307

*The average lifetime factor utilization of all available SHL products

- Figure 2 illustrates that, across all available rFVIII products in Sweden, BAY 94-9027 was associated with the lowest factor utilization compared to other extended duration products, ranging from a 20% reduction vs BAX 855 to 23% vs rFVIIIFc, and to SHL rFVIII products (23% reduction) (Figure 2).

Figure 2. Factor utilization reduction expected with BAY 94-9027 compared to other rFVIII products



- In a scenario with an ABR of 3.7[6], BAY 94-9027 is also associated with the lowest utilization.

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Disclosure

Disclosure text to be inserted here

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