# Preventing bleeds in pediatric patients with hemophilia A: which factor replacement therapy offers the best protection and on what cost?

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## CONCLUSIONS

- **In both the US and Germany, treatment with Damoctocog alfa pegol (Jivi®) resulted in the lowest costs of managing breakthrough bleeds.**
- **The lowest number of bleeds per year are estimated for patients treated with damoctocog alfa pegol (Jivi®) and Efanesoctocog alfa (Altuviiio®/Altuvoct®).**

## OBJECTIVES

1. **To estimate the number of bleeding episodes over a 5-year period in patients aged 7-11 years, who receive prophylactic treatment for hemophilia A.**
2. **To estimate the economic burden associated with bleeds in the US and in Germany**

## INTRODUCTION

- Hemophilia A is a rare hereditary condition characterized by the absence or insufficiency of coagulation factor VIII (FVIII)[1], leading to frequent or spontaneous bleeding into joints and muscles, and in severe cases, life-threating internal hemorrhages, if left untreated.[2]
- The current standard of care is prophylaxis, including FVIII-replacement, focusing on bleed prevention[20] – which is particularly crucial in the pediatric population, to protect developing joints of children from irreversible damage and chronic pain
- Breakthrough spontaneous bleeds may happen despite regular prophylaxis, and pediatric patients can be particularly prone to bleeds due to trauma as consequence of active lifestyle. Any bleed might be putting children's long-term health at risk.
- Selecting a treatment option associated with lowest possible bleeding rates can support the physical development of these high-risk patients.

## METHODS

Efficacy and factor consumption

- The model applied a 5-year time horizon, with patients entering at age 7
- Nine factor replacement therapy options were compared (Table 1).
- Annualized bleeding rate (ABR) for each therapy was derived from their respective clinical trials (Table 1)
- Bleeding episodes were assumed to occur while patients received prophylaxis therapy (prophylaxis costs not modelled)
- Factor consumption used for the treatment of a bleed (IU/kg) was derived from clinical trials where available, and from the recommendations in the Summary of Product Characteristics when trial data was not available
- For the two newly available treatment options, Jivi® and Altuviiio®/Altuvoct® , most recent data from their extension studies (Alfa PROTECT Extension and XTEND-ed, respectively) were used to derive ABRs and consumption data
- Annual factor consumption to treat bleeds was calculated with the following formula:

*Annual consumption = ABR * IU consumption per bodyweight per bleed * weight in kg*

Calculation of costs

- In the US, WAC prices per IU were used from Data.Medical.gov (Pricing Comparison for Blood Disorder Treatments) (Table 2). [18]
- In Germany, list prices were available for several dosage strengths for each treatment. The model used the mean cost per IU (Table 2).[19]
- Weight of patients was based on National Center for Health Statistics[5] in the US, and the German weight data were derived from the nationwide German Health Interview and Examination Survey for Children and Adolescents (KiGGS)[6]

**Table 2: DRUG PRICE PER IU**

| Treatment | US | Germany |
|---|---|---|
| Jivi® | $ 3.02 | € 0.62 |
| Altuviiio®/Altuvoct® | $ 5.47 | € 0.92 |
| Elocta® | $ 2.68 | € 0.51 |
| Esperoct® | $ 2.67 | € 0.51 |
| Advate® | $ 1.96 | € 0.57 |
| NovoEight® | $ 2.26 | € 0.43 |
| Nuwiq® | $ 1.90 | € 0.52 |
| Afstyla® | $ 1.96 | € 0.51 |
| Kovaltry® | $ 2.41 | € 0.58 |

## RESULTS

- Damoctocog alfa pegol (Jivi®) was associated with the lowest number of bleeds, over a 5-year horizon, in Germany, where the total number of bleeds was from 3.70 (Table 3).
- In the US, efanesoctocog alfa (Altuviiio® /Altuvoct®) was associated with the lowest total number of bleeds, with damoctocog alfa pegol (Jivi®) yielding the next lowest value (Table 3).
- Higher frequency of bleeds was estimated with shorter half-life products, such as Advate® (18.75) Afstyla® (18.45) and Kovaltry® (16.85) over 5 years (Table 3).
- The lowest FVIII consumption in Germany was estimated with Jivi®. In the US, Altuviiio®/Altuvoct® showed the lowest consumption, followed by Jivi® (Figure 2).
- Advate® shows the highest level of consumption in both countries – US and Germany (Figure 2).
- Cost of treating bleeds was estimated to be ranging from €4,479 (Jivi®) to €23,243 (Advate®) in Germany (Figure 3).
- In the US, Jivi® represented the least costly option, whereas Esperoct® was associated with the highest costs (Figure 1).

**Table 3: TOTAL NUMBER OF BLEEDS OVER 5 YEARS**

| Drug | US | Germany |
|---|---|---|
| Jivi® | 3.7 | |
| Altuviiio®/Altuvoct® | 3.3 | 3.9 |
| Elocta® | 9.8 | |
| Esperoct® | 9.85 | |
| Advate® | 18.75 | |
| NovoEight® | 9.85 | |
| Nuwiq® | 11.00 | |
| Afstyla® | 18.45 | |
| Kovaltry® | 16.85 | |

**Figure 2: TOTAL FVIII CONSUMPTION FOR THE TREATMENT OF BLEEDS, OVER 5 YEARS, IN IU**

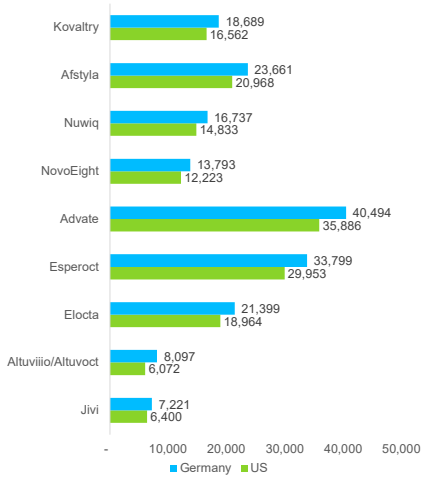



**Table 1: CHARACTERISTICS OF FVIII THERAPIES IN COMPARISON**

| | Brand name | Active substance | Total ABR | IU/kg per dose per bleed |
|---|---|---|---|---|
| Extended half-life products | Jivi® | Damoctocog alfa pegol | 0.74[7] | 56.31[†] |
| | Altuviiio®/Altuvoct® | Efanesoctocog alfa | 0.66* / 0.78** | 59.9[4] |
| | Elocta® | Efmoroctocog alfa | 1.96[8] | 63[14] |
| | Esperoct® | Turoctocog alfa pegol | 1.97[9] | 99[9] |
| Short half-life products | Advate® | Octocog alfa | 3.75[10] | 62.31[10] |
| | NovoEight® | Turoctocog alfa | 1.97[9] | 40.4[15] |
| | Nuwiq® | Simoctocog alfa | 2.2[11] | 43.9[16] |
| | Afstyla® | Lonoctocog alfa | 3.69[12] | 37[12] |
| | Kovaltry® | Octocog alfa | 3.37[13] | 32[17] |

*Mean dose per bleed as reported in Alfa-PROTECT Extension, age 7 to <12
*ABR in the United States is 0.66, reflecting ABR reported for the overall population, age 6 to <12[3]
**ABR in Germany is 0.78, reflecting ABR reported for the European population, age 6 to <12[3]

**Figure 1: TOTAL COST OF TREATMENT OF BLEEDS – US, USD**

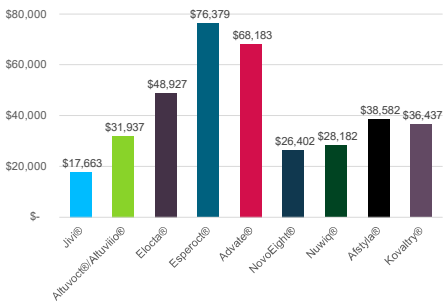
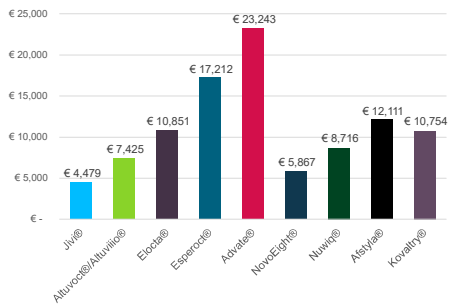



**Figure 3: TOTAL COST OF TREATMENT OF BLEEDS – GERMANY, EUR**




Disclosures
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