

The Economic Burden of Hemophilia B – A Lifetime Decision Analytic Model

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INTRODUCTION

- Hemophilia B (HB) requires lifelong treatment to prevent or manage bleeding and associated morbidity.¹
- HB is managed by factor IX (FIX) replacement therapy, including standard half-life [SHL] FIX prophylaxis, extended half-life [EHL] FIX prophylaxis, and FIX on-demand.^{2,3}
- Frequent intravenous administration of FIX can be burdensome to people with HB (PwHB) and is associated with a significant cost to the health care system.⁴

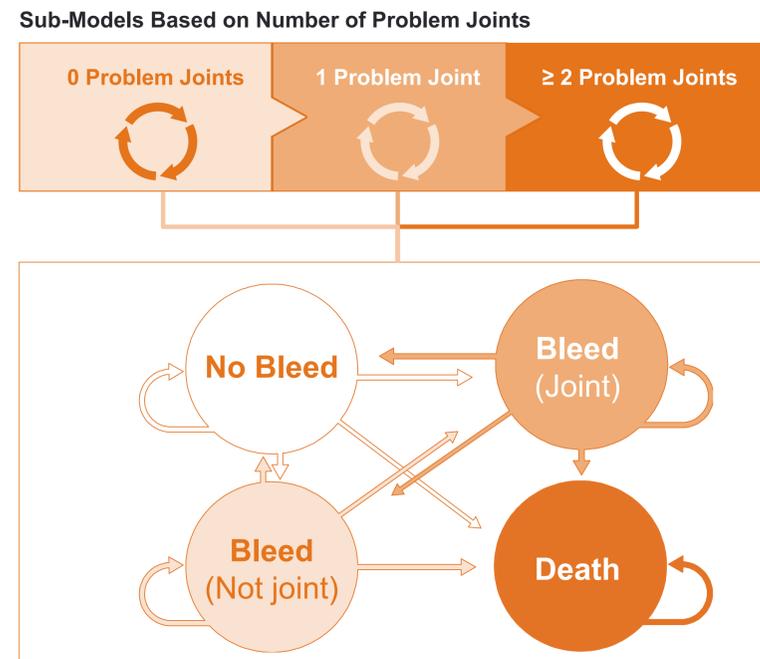
OBJECTIVES

- To develop a decision analytic model that estimates the adult lifetime costs associated with HB treatment options: SHL FIX prophylaxis, EHL FIX prophylaxis, and FIX on-demand.
- To identify the key drivers behind the overall cost of HB management.

MATERIALS & METHODS

- An expert panel consisting of clinicians, health technology assessment specialists, and patient advocacy representatives evaluated and reached a consensus on the model framework.
- A Markov model (**Figure 1**) was developed to reflect the natural course of the disease for adult patients with severe and moderately severe HB. The model consists of four health states: “no bleed”, “bleed (not joint)”, “bleed (joint)” and “dead”.
- Sub-models were based on the number of problem joints (PJs) acquired by PwHB: 0, 1, and 2+. This allowed different patterns of healthcare resource utilization due to joint deterioration to be factored in.
- Both societal and US third-party payer perspectives were considered, with lifetime horizon as the base-case and shorter time horizon of three, five, and ten years as sensitivity analyses.
- All costs were in 2019 USD(\$) and the discount rate was 3%.
- Model inputs were tested in one-way sensitivity analysis (OWSA) primarily based on their 95% confidence interval.

Figure 1: Model Structure

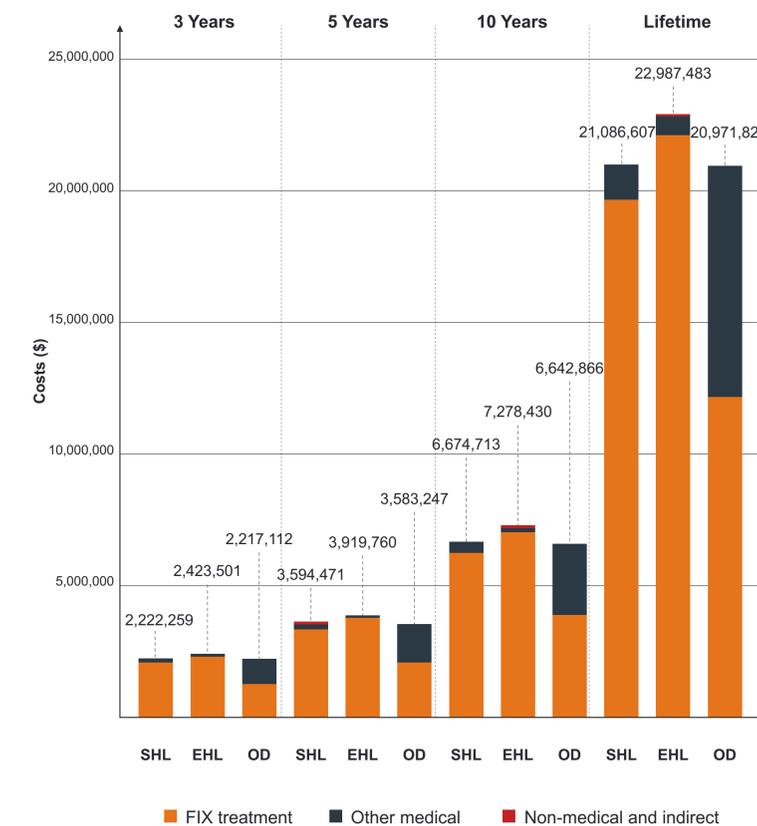


Bleed Events Markov Model (Same for Each Problem Joint Sub-Model)

RESULTS

- Model results showed a substantial cost of HB management associated with all three treatment options. The adult lifetime total cost per patient was \$21,086,607 for SHL FIX prophylaxis, \$22,987,483 for EHL FIX prophylaxis, and \$20,971,826 for FIX on-demand treatment (**Figure 2**).
- Most of the direct medical cost for HB management is driven by FIX treatment, estimated at \$19,754,862 and \$22,202,092 for prophylaxis with SHL and EHL FIX prophylaxis, respectively (both over 90% of direct medical cost) and at \$12,179,003 for FIX on-demand treatment (close to 60% of direct medical cost).
- At shorter time horizons, the total cost per patient ranged from \$2,222,259 to \$2,423,501 for 3-year, \$3,583,247 to \$3,919,760 for 5-year, and \$6,652,866 to \$7,278,430 for 10-year across all three treatment arms.

Figure 2: Summary of Model Results

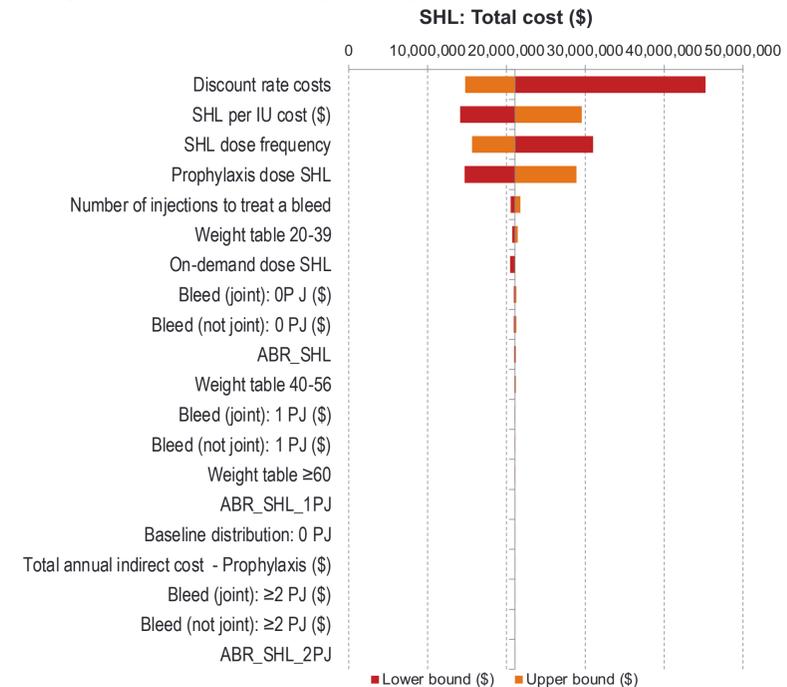


- OWSA results were generally consistent with the base-case results. Total adult lifetime cost of HB management was most sensitive to variation in the unit cost of FIX, discount rates, and the number of injections needed to treat a bleed, regardless of the treatment arm (see **Figure 3** for representative results of SHL FIX prophylaxis).

LIMITATIONS

- Limitations of this study include the assumptions used in economic modelling, for example, the baseline distribution of proportion of patients with 0, 1, and 2+ PJs. However, extensive sensitivity analyses were conducted to test model inputs.
- The model also did not capture the impact of HB on caregivers.

Figure 3: Sensitivity analysis results



CONCLUSIONS

- The model results show a substantial economic burden at over \$20 million per patient among patients with severe and moderately severe HB, regardless of the treatment strategy used.
- Cost of FIX treatment is the leading cost driver.
- These findings highlight the unmet medical need for PwHB.

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