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.

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 (Figure 2).
- Most of the direct medical cost for HB management is driven by FIX treatment, estimated at $19,754,862 and $22,090,093 for prophylaxis with SHL and EHL FIX prophylaxis, respectively (both over 90% of direct medical cost) and at $12,179,03 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.

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.

REFERENCES


ACKNOWLEDGEMENT

This work was supported by uniQure Inc.