ESTIMATING THE CLINICAL AND ECONOMIC BENEFITS OF OPTIMISING THE MANAGEMENT OF PATIENTS WITH HEMOPHILIA A IN DEVELOPING COUNTRIES

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INTRODUCTION

- Hemophilia A (HA) is a debilitating disorder associated with a high healthcare burden[1,2,3,4].
- Prophylaxis (Px) treatment helps reduce these associated costs and healthcare resource utilization (HCRU) by reducing the number of bleed-related clinical outcomes[1,2,4].
- Whilst lifelong high-dose Px is considered the optimal standard of care (SOC) for patients with HA, the perceived high costs and limited access and availability of factor FVIII could limit the adoption of high-dose Px[3,5].
- As such, many developing countries adopt less costly Px management alternatives, including low- and intermediate-dose Px treatment[3,5].
- Figures 2 and 3 show the country demographics of patients with HA in Chile and Malaysia, respectively[1].

OBJECTIVE

- This study explores the potential clinical and economic benefits of optimizing Px treatment strategies to improve the SOC in patients with severe HA in Chile and Malaysia

CLINICAL OUTCOMES		ECONOMIC OUTCOMES
Bleeds Target Joints Surgeries	Hospitalizations Deaths	Missed Days Direct Costs Indirect Costs

Figure 1: Clinical and economic outcomes of patients with Hemophilia A in Chile and Malaysia

Current HA landscape in Chile

Figure 2: Country demographics of Chile

Current HA landscape in Malaysia

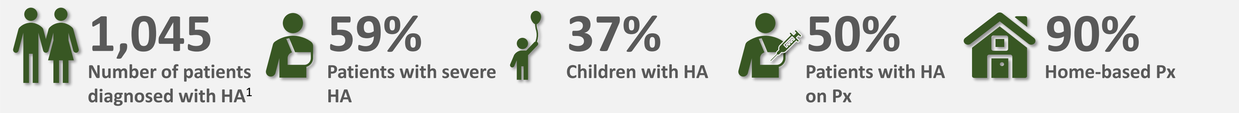

Figure 3: Country demographics of Malaysia

METHODS

- A **burden-of-illness** (BOI) analysis was undertaken to understand the clinical and economic landscape of HA in developing countries. A de-novo BOI-model was developed, with inputs derived from local literature and clinical expert discussions in two developing countries – Malaysia and Chile
- Annualized bleeding rates (ABRs) were calculated for each treatment strategy, based on relative risk reductions derived directly, or based on published international long-term clinical studies.
- Epidemiology, unit costs and HCRU data were obtained via local publications or expert interviews.
- Bleed treatment regimens, including on-demand treatment and low-, intermediate- and high-dose Px treatment strategies were considered, as illustrated in Figure 4.
- **Regimens were compared in terms of:**
  - **Clinical outcomes** – including the number of bleeds, target joints and disabilities, as well as direct and indirect costs associated with HA
  - **Net clinical and cost outcomes** associated with moving patients to optimized Px treatment
- The analysis was conducted from a payer perspective, over a 5-year time horizon.

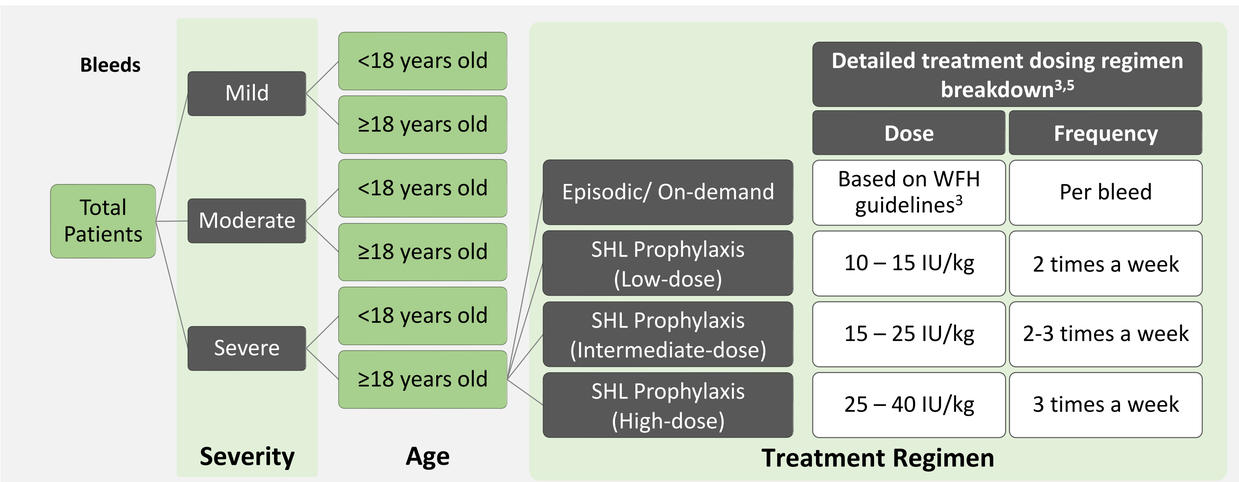

Figure 4: Detailed treatment dosing regimen breakdown for patients with HA

RESULTS

- Optimizing Px treatment strategy in patients could lead to a ≥15% improvement in clinical outcomes, including bleeds, target joints and disabilities, over five years depending on the level of Px adopted.
- Whilst Px-related factor cost is expected to increase with higher Px dosing, this cost is partially offset by reduced HCRU costs, bleed-related treatment factor VIII costs and indirect costs due to loss of productivity and school/work absenteeism.
- Results reported varied according to baseline SOC and the level of Px adopted.

	MODEL OUTCOMES	Chile	Malaysia
	Increase in Px uptake	**+6.0%**	**+16.0%**
Bleed-related EVENTS	**Bleeds**	-5,568 **(-18.5%)**	-2,790 **(-12.8%)**
	Target Joints	-1,545 **(-19.3%)**	-1,698 **(-14.9%)**
	Disabilities	-146 **(-19.9%)**	-264 **(-21.7%)**
	Surgeries	-67 **(-8.6%)**	-402 **(-15.0%)**
	Hospitalizations	-5,039 **(-18.5%)**	-2,494 **(-13.0%)**
	Missed Days	-79,629 **(-19.1%)**	-32,484 **(-14.3%)**
	Deaths	-1 **(-16.1%)**	-0 **(-0.0%)**
Costs (USD Mn)	**Direct Cost (excl. factor)**	-USD 0.3Mn **(-12.0%)**	-USD 2.7Mn **(-13.9%)**
	Indirect Cost	-USD 3.0 **(-22.1%)**	-USD 1.0Mn **(-15.3%)**
	Cost of Care (excl. factor)	-USD 3.3Mn **(-20.4%)**	-USD 3.7Mn **(-14.2%)**

Table 1: Net clinical and economic outcomes of patients in Chile and Malaysia after initiating patients to intermediate Px

Tx: treatment; Px: prophylaxis

Chile

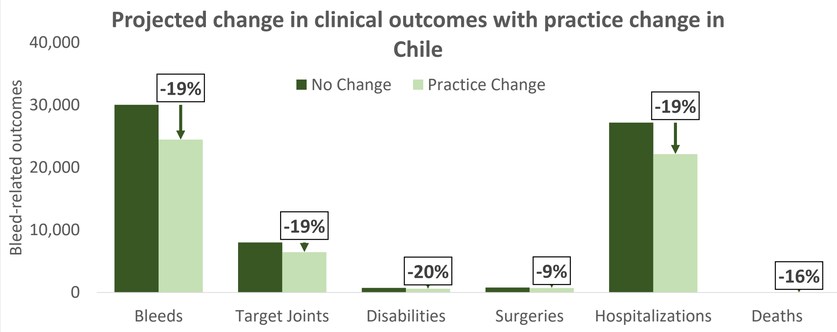

Figure 5: Clinical outcomes of patients in Chile after transitioning patients to intermediate Px

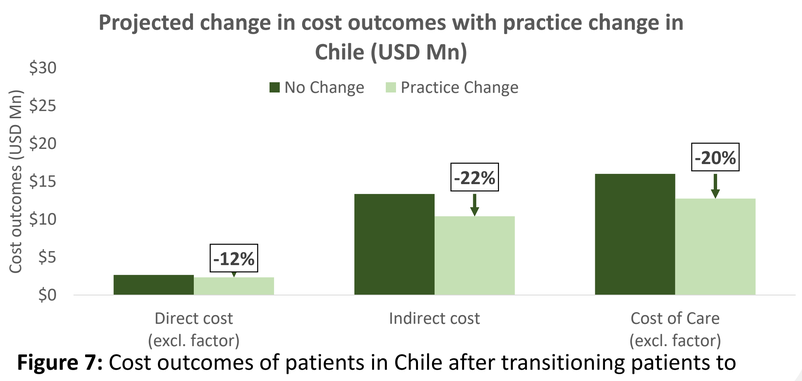

Figure 7: Cost outcomes of patients in Chile after transitioning patients to intermediate Px

Malaysia

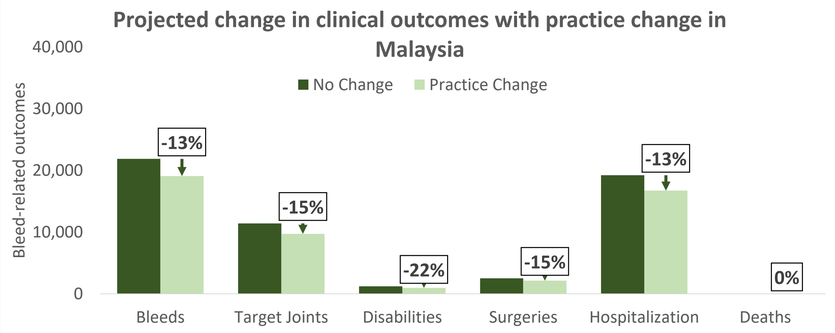

Figure 6: Clinical outcomes of patients in Malaysia after transitioning patients to intermediate Px

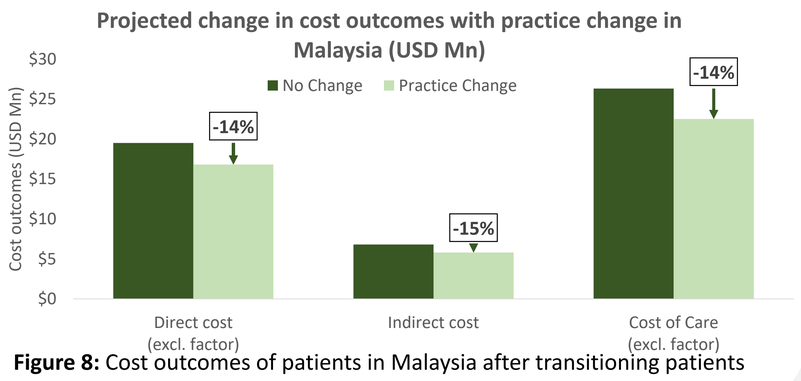

Figure 8: Cost outcomes of patients in Malaysia after transitioning patients to intermediate Px

SENSITIVITY ANALYSIS

- The robustness of the model was assessed using one-way sensitivity analysis (OWSA)
- Sensitivity analyses were performed on all three key parameters: clinical outcomes, healthcare resource use and costs.
- The model was most sensitive to assumptions on the switch rate of patients to Px and the cohort size in both countries. In Chile, the model was also sensitive to daily wage and resource utilization while in Malaysia, other influential factors include the cost of longer term disability and the baseline ABRs.

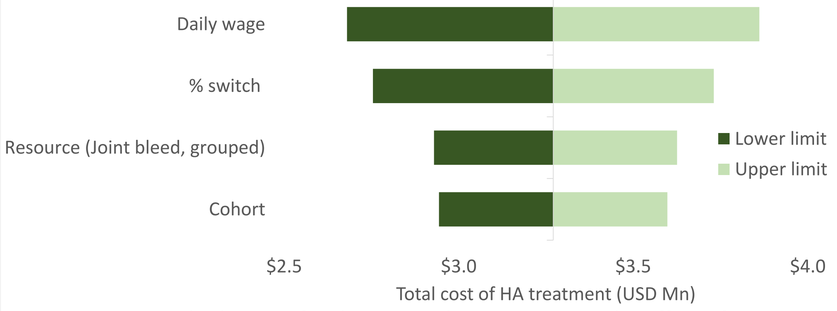

Figure 9: Tornado diagram for the OWSA that summarizes the effect of variation in key model parameters in Chile

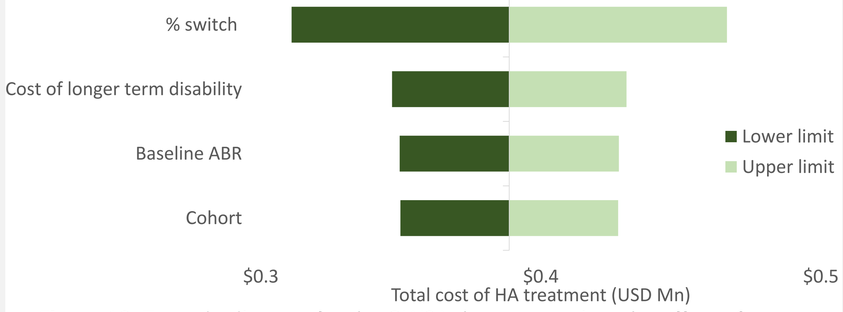

Figure 10: Tornado diagram for the OWSA that summarizes the effect of variation in key model parameters in Malaysia

LIMITATIONS

- The BOI model considers only patients diagnosed with HA; the burden of hemophilia arising from undiagnosed patients has not been considered in this simulation.
- The current model assumes all bleed episodes are treated optimally, although real-world practice may differ.
- Clinical and cost inputs are highly dependent on clinical expert information where limited published research is available.
- Inputs should be considered exploratory, with updates expected as country datasets mature.

CONCLUSIONS

- A flexible model considering baseline SOC and comparing various Px regimens can provide insights into the long-term benefits of different HA management strategies
- These findings can inform healthcare policy decisions to improve the SOC for patients with HA, especially in countries such as Malaysia and Chile, where the perceived high cost, and limited access and availability of factor FVIII could limit adoption of high-dose Px.

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ABBREVIATIONS

BOI: burden-of-illness; HA: hemophilia A; HCRU: healthcare resource use; OWSA: one-way sensitivity analyses; Px: prophylaxis; SHL: standard half-life; SOC: standard of care; WFH: World Federation of Hemophilia

DISCLOSURES

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This poster is intended for healthcare professionals.