EVALUATING THE HOLISTIC VALUE OF INNOVATIVE THERAPIES VIA THE TREATMENT IMPACT MODEL: A CASE STUDY ON EMICIZUMAB FOR HAEMOPHILIA A IN SINGAPORE

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Chloe Lee[1], Melina Arnold[2], Mariana Al-Adwan[2], Eng Soo Yap[3,4], Pei Lin Koh[5,6], Joyce Ching Mei Lam[7,8], Sim Leng Tien[9,10]

1. Roche Singapore Pte Ltd, Singapore 2. Hoffmann-La Roche, Switzerland 3. Department of Laboratory Medicine, National University Hospital, Singapore 4. Department of Medicine, Yong Loo Lin School of Medicine, National University of Singapore, Singapore 5. Department of Paediatrics, Khoo Teck Puat - National University Children's Medical Institute, National University Hospital, Singapore 6. Department of Paediatrics, Yong Loo Lin School of Medicine, National University of Singapore 7. Paediatric Haematology / Oncology Service, KK Women's and Children's Hospital, Singapore 8. SingHealth Duke-NUS Paediatrics Academic Clinical Programme, Singapore 9. Department of Haematology, Singapore General Hospital, Singapore 10. SingHealth Duke-NUS Medical, Pathology and Oncology Academic Clinical Programme, Singapore

Background

While cost-effectiveness assessments provide per-patient metrics, the Treatment Impact Model (TIM) offers a broader population-level perspective. It complements existing frameworks by offering a method to quantify the holistic value medical innovations bring to patients, healthcare systems, and economies in a consistent manner.

Proposed Case Study – emicizumab in the prophylactic treatment of people with Haemophilia A (PwHA)

- Emicizumab is a prophylactic treatment for people with haemophilia A (PwHA) and combines the advantages of an extended interval between doses plus subcutaneous administration versus frequent intravenous FVIII infusions.[1] The effectiveness and safety of emicizumab has been demonstrated in several clinical trials and real-world studies,[2,3] including a reduction in bleeds when compared with FVIII replacement or bypassing agent (BPA) prophylaxis.[4,5]
- Prior to the introduction of emicizumab in Singapore, intravenous (IV) Factor VIII (FVIII) and bypassing agents (BPAs) were the standard of care in the reduction and treatment of bleeds in PwHA.
- Since its approval in late 2018, Singapore has seen adoption of emicizumab as a standard for PwHA with FVIII inhibitors. At the same time, we have also observed that while FVIII prophylaxis reduces bleeding episodes in PwHA without FVIII inhibitors, treatment burdens remain.[1,6] The local uptake and clinical outcomes can form the basis of a rich case study for a population-impact perspective.

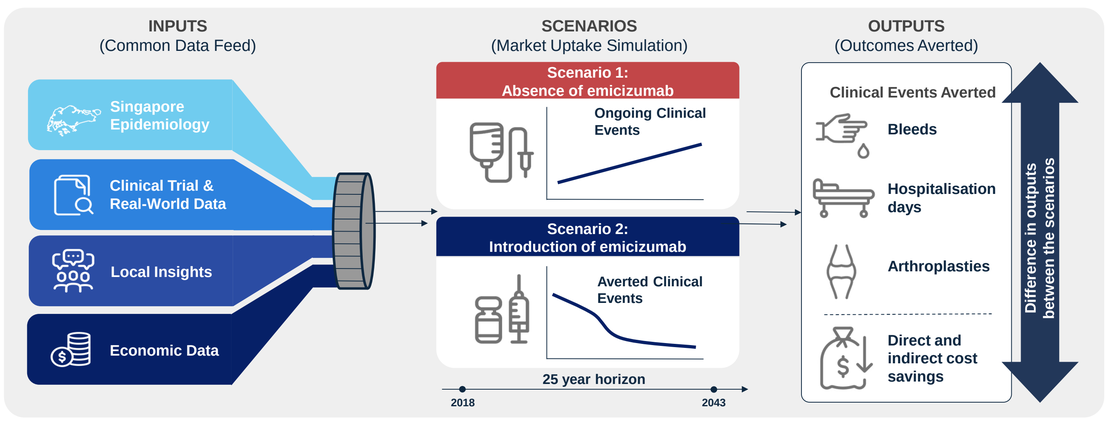
Objective

This study evaluates and quantifies the population-level impact of introducing emicizumab as a prophylactic treatment for PwHA in Singapore, focusing on averted clinical events, increased quality-adjusted life years (QALYs), and reduced direct medical and indirect productivity costs over a 25-year period.

Methods

- A dynamic epidemiologic model was used to project outcomes over a 25-year time horizon by comparing an "absence of emicizumab" scenario (treatment with only on-demand or prophylactic factor VIII replacement and bypassing agents) against an "introduction of emicizumab" scenario (simulating gradual market uptake). **Figure 1** provides a simplified schematic of TIM study design.

Figure 1. Simplified Schematic of TIM Study Design



- Some **key assumptions / limitations** of the model:
  - a constant treatment environment with no new therapies entering the market, hence excluding potential impacts from newer emerging therapies.
  - once a patient has switched from prophylactic or on-demand FVIII/BPA to emicizumab, they remain on emicizumab throughout the model duration
  - the inhibitor status of the patient does not change during the model duration
- The impact of introducing emicizumab as a treatment was established by comparing the number of clinical events (e.g., bleeds, arthroplasties), QALYs, direct medical costs associated with bleeds, indirect costs (e.g. productivity losses) between the two scenarios. **The model does not take into consideration the acquisition cost of the prophylactic treatment.**
- The model differentiated ABRs by treatment regimen and age group, and ABRs were derived based on a combination of HAVEN 3 trial and local published data.[2,3,4,5] Progressive joint damage was simulated via a Pettersson Score (PS)-based algorithm where occurrence of arthroplasties was calculated in PwHA reaching a PS threshold of 28.[7] Hospitalization days by treatment regimen and age group were retrieved from the HAVEN 1 and 3 trials.[4,8]
- The model incorporated Singapore-specific epidemiological and economic data. **Table 1** outlines the Singapore-specific epidemiology input for the model.

Table 1. Epidemiology data input for Singapore

Epidemiology	Singapore[9]
Number of PwHA eligible for emicizumab	137
Age distribution, %	
Infants and toddlers <2 years	3.0
Children 2–<12 years	13.5
Adolescents 12–<18 years	6.7
Adults 18–<65 years	42.9
Elderly 65+ years	33.9
PwHA with FVIII inhibitors, %	4.0
Ratio of prophylaxis vs on-demand prior to emicizumab introduction	0:1

References

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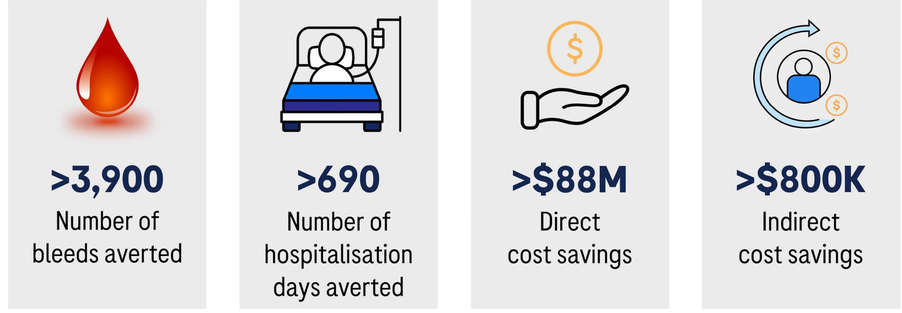
9. Epidemiology data were derived from expert clinician consultations and aggregated local patient cohort records across treatment centers.

Results

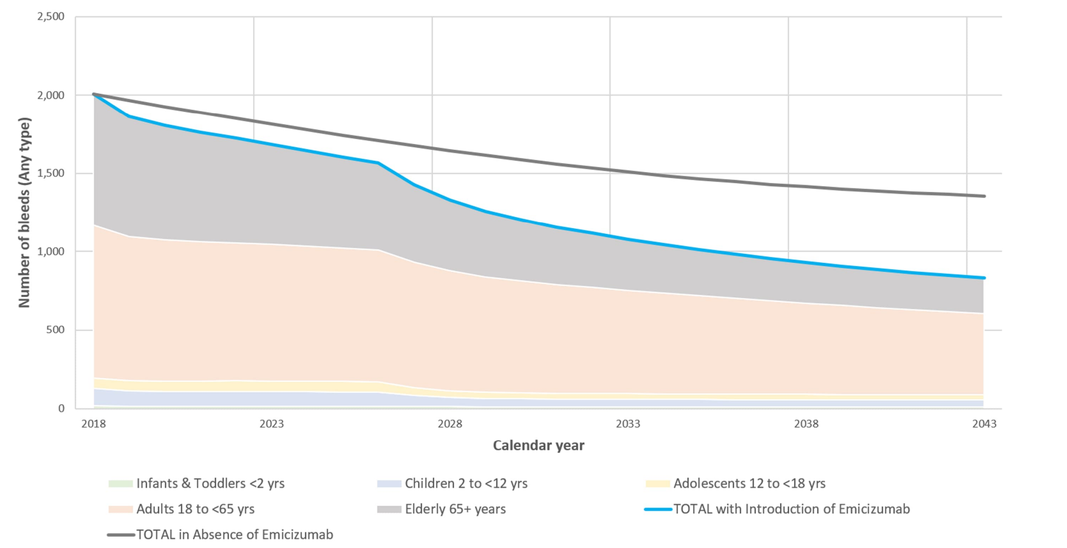
- With the introduction of emicizumab and at its projected market share uptake peaking at 94% and 65% of the eligible paediatric and adult PwHA, the model estimated in over 3,900 bleeds averted and 690 hospitalisation days avoided, leading to total savings exceeding S$88 million in direct and indirect costs over a 10-year period. See **Figure 2**

Figure 2. Projected 10-year (2027–2036) reduction in clinical events and cost savings in Singapore following emicizumab introduction



- The projected number of bleeds averted corresponds to a 25.3% reduction in total bleeds over the next decade (2027-2036) in Singapore. See **Figure 3**

Figure 3. Bleeds (any type) in PwHA with or without FVIII inhibitors, by age group and scenario (introduction or absence of emicizumab)



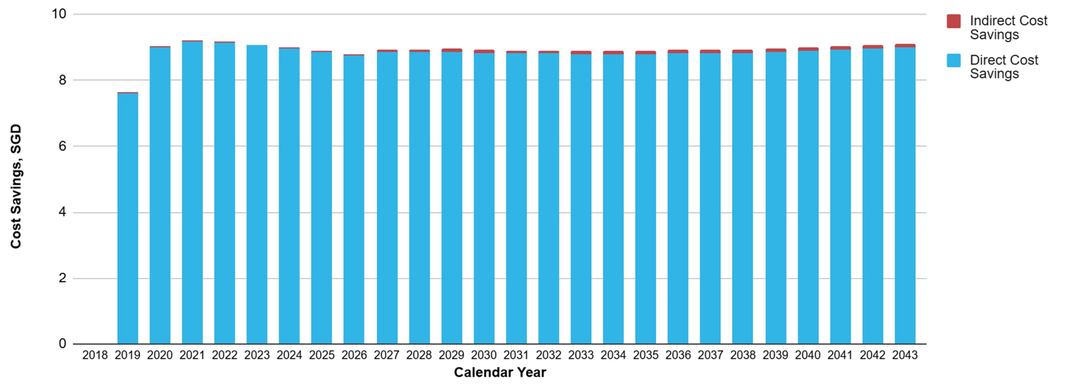
- **Table 2** compares the projected outcomes between the "introduction of emicizumab" and "absence of emicizumab" scenarios. The improved outcome in bleeds, arthroplasties and hospitalization days associated with the introduction of emicizumab is estimated to deliver a total savings exceeding $88 million in direct and indirect costs.

Table 2. Projected outcomes with emicizumab versus on-demand or prophylactic FVIII replacement/BPAs in PwHA with or without FVIII inhibitors

Year of emicizumab introduction	2019			
	Introduction of emicizumab	Absence of emicizumab	Absolute change, n	Relative change, %
2027-2036				
All bleeds	11,600	15,527	-3,925	-25.3
Joint bleeds	4,082	5,880	-1,797	-30.6
Arthroplasties	10	10	0	-
Hospitalisation days	667	1,356	-693	-51.1
QALY, average	90	87.2	2.8	+3.1
Direct Costs, SGD	23,342,451	111,686,049	-88,343,599	-79.1
Indirect Costs, SGD	2,233,323	3,073,602	-840,280	-27.3

- Figure 4 shows that the savings remains consistent with most of the savings resulting from direct cost savings (>90% of the total cost savings). Drug acquisition costs for prophylactic treatment are excluded.

Figure 4. Total Cost Savings, presented annually (with introduction of emicizumab)



Conclusion

- The TIM offers a consistent and structured method to quantify the comprehensive value that medical innovations can bring to patients, healthcare systems, and broader economies.
- Based on the modeling of emicizumab prophylaxis in Singapore, our case study demonstrates significant socioeconomic value at the population-level. This value is primarily derived from substantial reductions in clinical events, including a projected 25.3% decrease in total bleeding episodes and a 51.1% reduction in hospitalisation days over a decade that is estimated to generate cost savings exceeding S$88 million.
- The underlying TIM methodology is highly adaptable and can be customized to various diverse disease areas, providing essential evidence to quantify holistic socioeconomic value that extends beyond clinical efficacy measurements.
- Our study suggests that the TIM can serve as a valuable complementary tool for healthcare system decision makers, supporting value-based evaluation with a needed, broader population-impact perspective during the comprehensive assessment of innovative medical technologies.