Budget impact analysis of Emicizumab in pediatric Hemophilia A patients with inhibitors in Kingdom of Saudi Arabia (KSA)

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INTRODUCTION

- Hemophilia A (HA) is a rare congenital disorder caused by an inherited deficiency in blood clotting factor VIII and predominantly occurs in males.[1] HA patients have an increased risk of bleeding following surgery, trauma or bleed may even occur spontaneously. HA occurs in 1 in 5,000 live male births worldwide.[2]
- Bleeding risk in HA is managed with intravenous administration of Factor VIII replacement therapies (either recombinant or plasma-derived).[3] However, exposure to Factor VIII concentrate leads to the development of neutralizing alloantibodies (known as inhibitors) in ~30% of persons with HA.[3]
- Annual Global Survey Report 2016, published by World Hemophilia Federation, reported the total number of HA patients and HA patients with clinically identified inhibitors to be 149,764 and 4,711 respectively in 113 countries.[4]
- In 2016, Kingdom of Saudi Arabia (KSA) reported a total of 334 HA cases. Among these, the number of prevalent and incident cases of HA with clinically identified inhibitors were 48 and 4, respectively.[4]
- Hemophilia patients with inhibitors are twice as likely to be hospitalized for a bleeding complication, and treatment costs associated with inhibitors can be 5 times greater than for those without inhibitors (mean cost: $697,000 vs $144,000, median costs: $330,835 vs $73,321, respectively).[5]
- Bleeding in HA patients with inhibitors is treated with activated prothrombin complex concentrate (APCC) or recombinant activated FVII (rFVIIa) given either on-demand at the time of bleed or prophylactically. Immune therapy induction (ITI) is used to reduce the inhibitor load in these patients. These therapies are associated with a very high cost.[6,7]
- Emicizumab is a monoclonal antibody with dual targets (“bispecific”) that allow it to bridge activated factor IX and factor X, the role normally played by factor VIII in the clotting cascade. It offers advantage of reduced bleeding rates and lower injection frequency over currently available treatments (aPCC, rFVIIa) and immune therapy induction (ITI).[8]

OBJECTIVE

- This study estimated the budgetary impact of adopting Emicizumab in pediatric Hemophilia A patients with inhibitors in the ministry of health (MOH), Kingdom of Saudi Arabia (KSA) over a 5-year time horizon (2018-2022).

METHODOLOGY

- A Budget Impact Model (BIM) was developed to assess the economic effect of adopting Emicizumab in KSA.
- The model consisted of following variables:
  - Model structure & perspective: Microsoft® Excel based model was developed to access payer’s perspective.
  - Model Inputs: Various inputs such as current market share, direct costs based on drug costs, administration costs, travel costs and cost of management of serious adverse events were sourced from expert interviews.
  - Model Outputs: The model assessed the impact of adopting Emicizumab on payer’s budget as a prophylaxis in addition to aPCC, rFVIIa and ITI. These were calculated in form of two scenarios:
    - › Scenario 1 – Without prophylactic Emicizumab
    - › Scenario 2 – Anticipated scenario With prophylactic Emicizumab.
- Sensitivity Analysis: A one-way sensitivity analysis was performed to understand the impact on budget when model inputs were varied by 20%.

Step 1 **Understanding KSA Market**

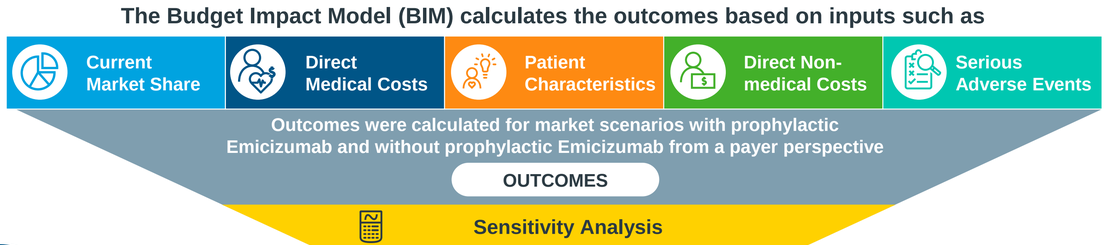
Step 2 **Discussion/Guide Questionnaire**

Step 3 **Expert Interviews**

Step 4 **Model Development/ Adaptation**

Result and Manuscript

The Budget Impact Model (BIM) calculates the outcomes based on inputs such as



RESULTS

- Over a 5-year time horizon, switching 100% of HA patients with inhibitors from prophylactic and on-demand recombinant activated factor VII (rFVIIa), and activated prothrombin complex concentrate (aPCC) therapies or immune tolerance induction (ITI) to prophylactic Emicizumab resulted in an a cumulative total savings of 21.5% (Fig.1).
- The main driver of these savings over the years were the superior efficacy of Emicizumab leading to a reduction in bleeding rates from 21% to 0.20% and a reduction in total number of hemorrhagic events from 549 to 2 (Fig. 2 and Fig. 3). Reduction in hemorrhages led to decrease in number of surgeries and hospitalizations required for management of hemorrhagic events by 98.9% each over time horizon of 5 years (Fig. 3).
- Introduction of Emicizumab also led to reductions in the overall drug cost, in administration cost and travel cost by 4.8%, 52.6% and 53.2% respectively over 5 years (Fig. 4). Reduction in administration costs were due to reduced need for on-demand administrations with Emicizumab.

RESULTS (contd.)

Figure 1: Total Cost Across 5 Years & Cumulative Cost: Without Emicizumab vs. with Emicizumab

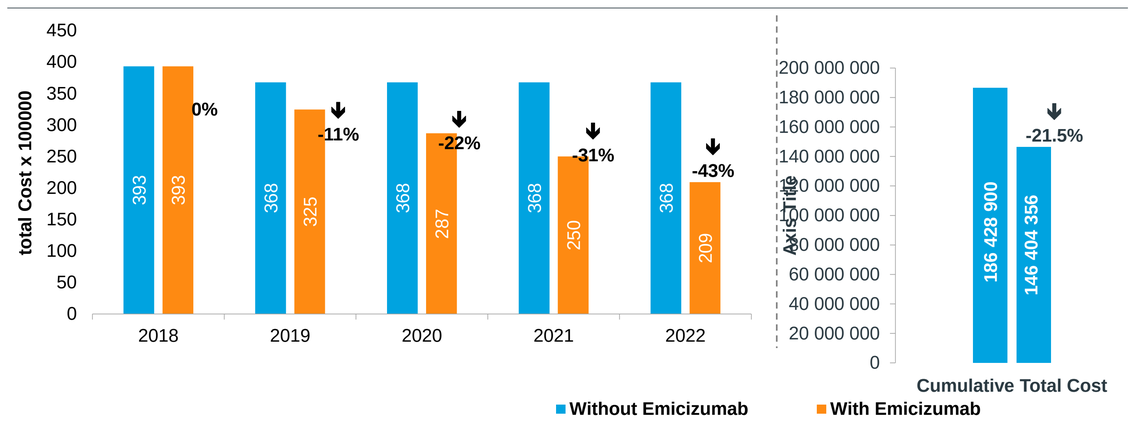


Figure 2: Annual Bleed Rate & Hemorrhagic Events: Without Emicizumab vs. with Emicizumab

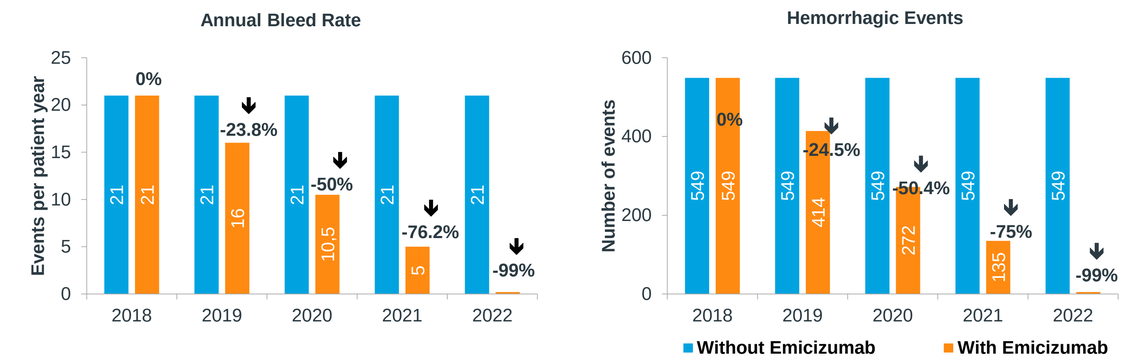


Figure 3: Surgeries and Hospitalization (for Hemorrhages): Without Emicizumab vs. with Emicizumab

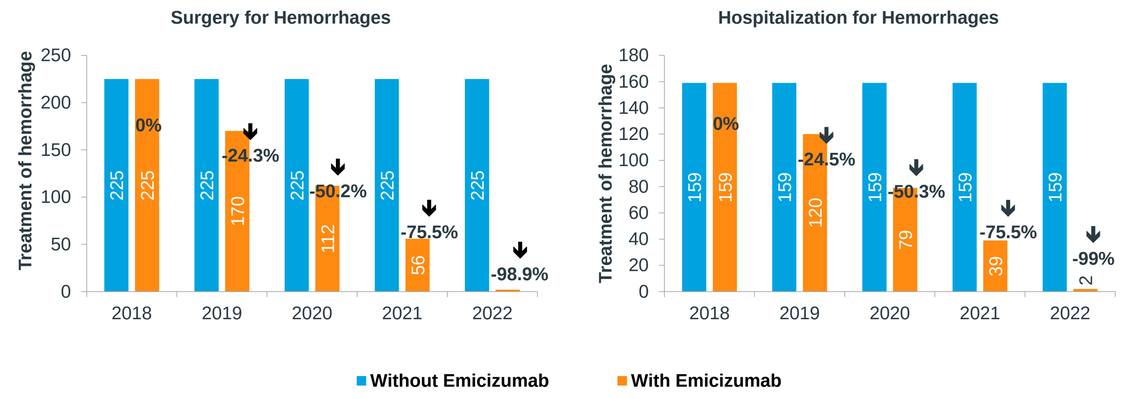
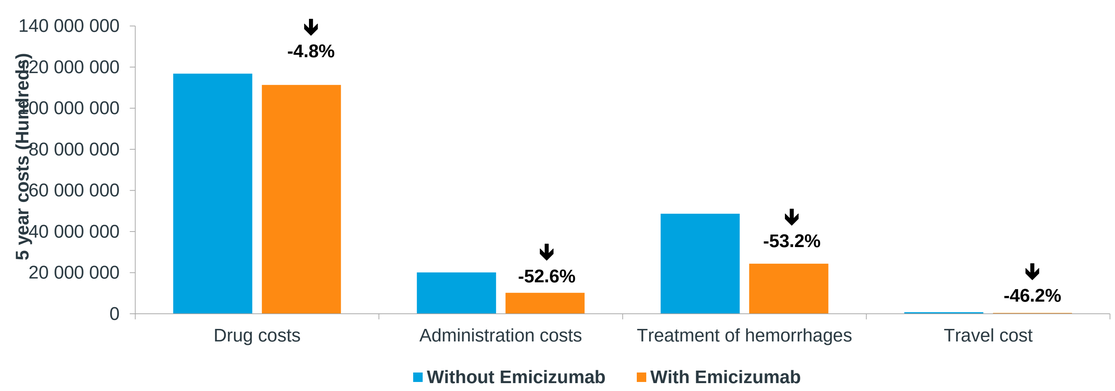


Figure 4: Cumulative 5-year Costs by Category: Without Emicizumab vs. with Emicizumab



CONCLUSION

- The current budget impact analysis estimated the potential budget impact of switching HA pediatric patients with inhibitors to prophylactic Emicizumab in the Saudi MOH.
- Prophylactic Emicizumab was found to be more cost saving as compared to current treatment strategies (prophylactic and on-demand aPCC, rFVIIa and ITI) in KSA. The cost savings were mainly driven by lower drug cost, administration cost, travel cost and lower rates of surgeries and hospitalizations for bleeds management.
- A 100% shift to Emicizumab resulted in 21.5% reduction in the overall cost of HA patients with inhibitors over 5 years.
- These findings provides a comprehensive summary of economic implication of adopting Emicizumab in KSA. It can also act as an important evidence for policy makers, budget holders and health advisors, while making treatment coverage decisions in the treatment of HA patients with inhibitors.

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