

# BUDGET IMPACT ANALYSIS OF THE USE OF EMICIZUMAB IN PROPHYLAXIS FOR SEVERE HEMOPHILIA A AND INHIBITORS IN THE PEDIATRIC POPULATION OF THE DOMINICAN REPUBLIC

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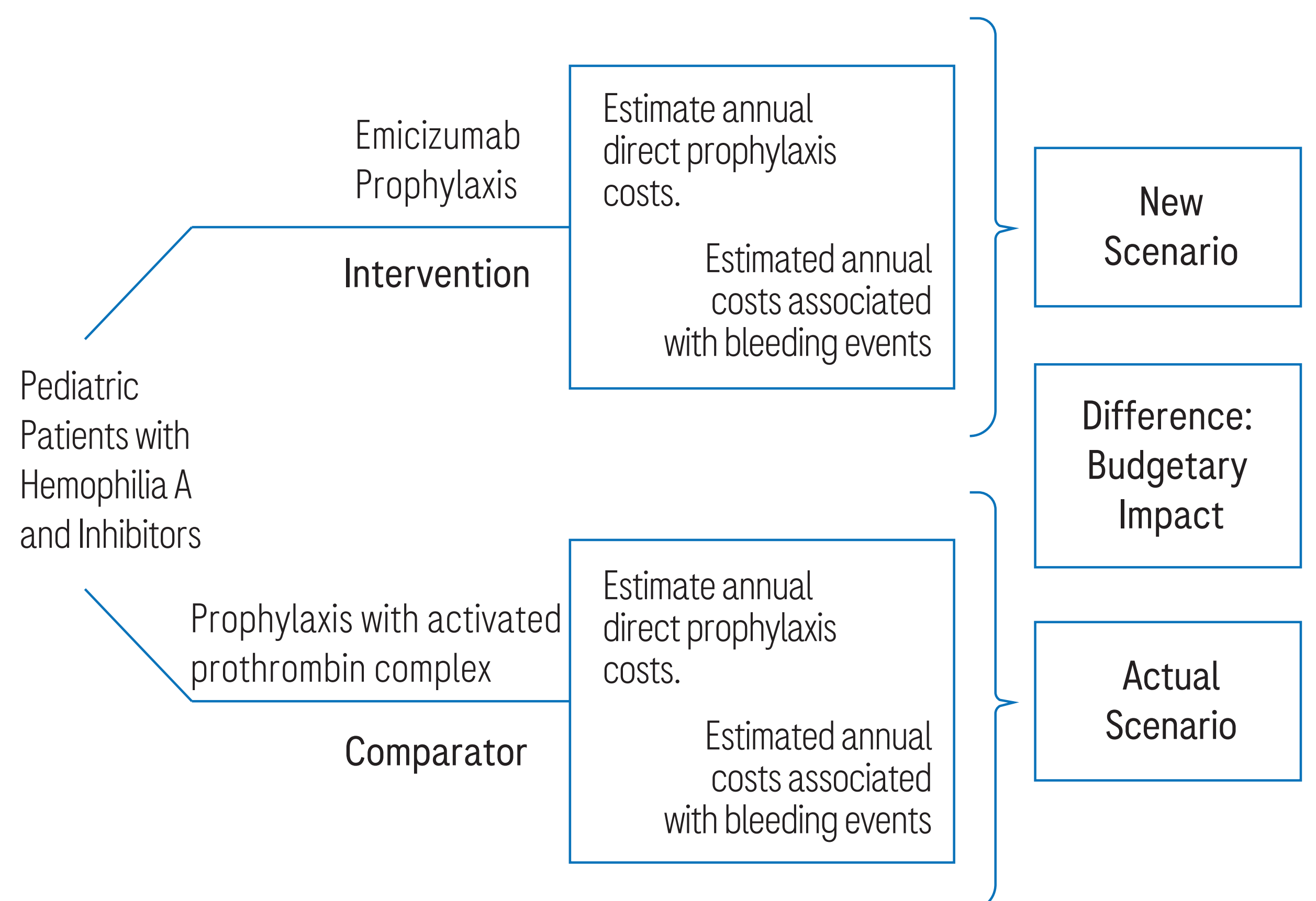
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## OBJECTIVES

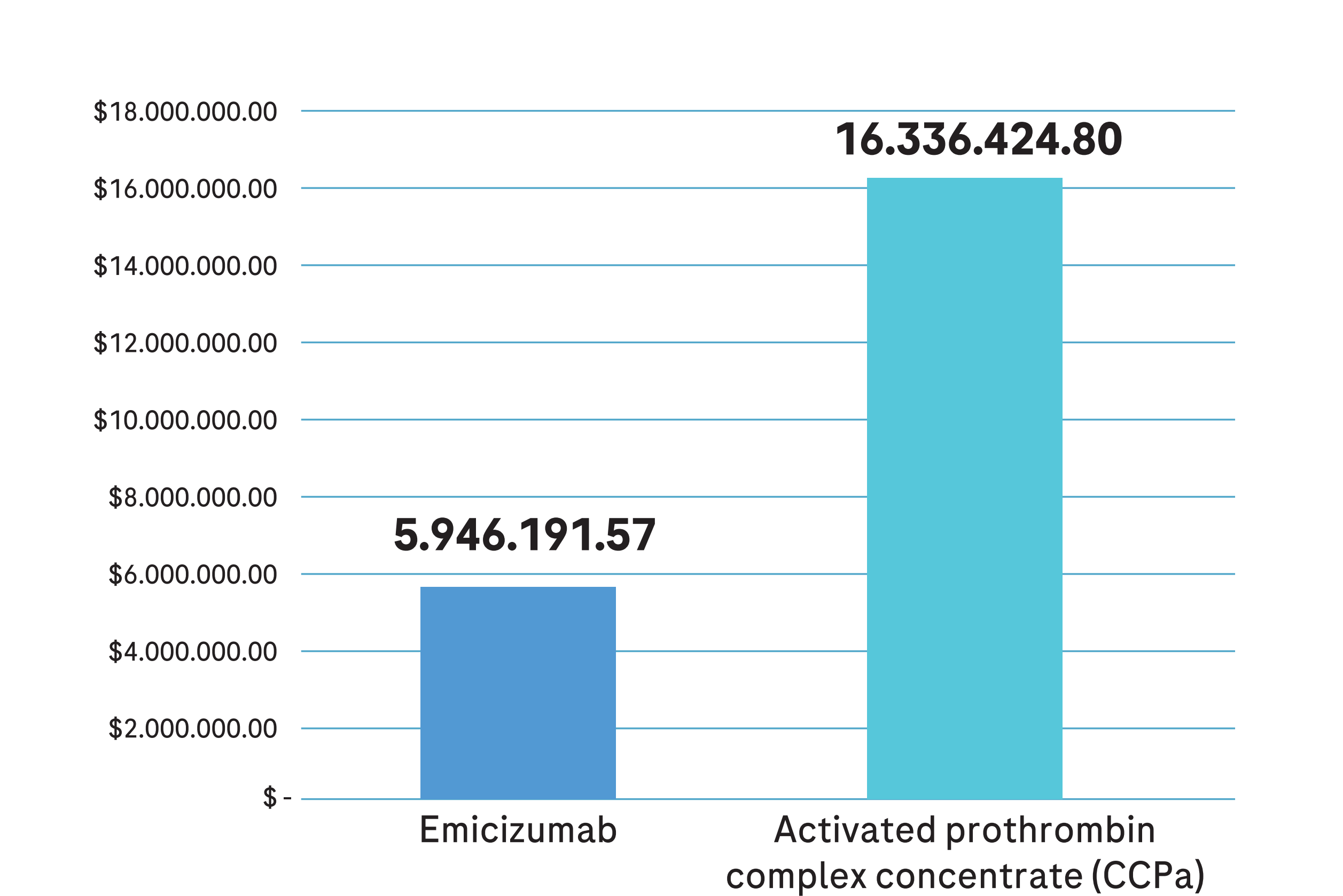
To estimate the impact on annual costs of the use of Emicizumab compared to activated prothrombin complex - aPCC in prophylactic treatment for pediatric patients with severe Hemophilia A and inhibitors in the health system of the Dominican Republic.

**Figure 1.** Conceptual framework of the budget impact analysis model of the use of Emicizumab in severe Hemophilia A prophylaxis and inhibitors in the pediatric population of the Dominican Republic.



## RESULTS

The annual cost of a pediatric patient in the current scenario with aPCC is USD \$286,603 in the future scenario with emicizumab is USD \$114,650. The cost of three years of care in the current scenario is USD \$16,336,424 and in the future scenario USD \$5,946,191



## METHODS

A BIA is performed where the current scenario is prophylaxis with aPCC and the future scenario is prophylaxis with emicizumab. The population is 19 patients with Hemophilia A and inhibitors reported by the Dominican Republic in the WFH global annual survey, the time horizon was 3 years. Assumptions: aPCC dose 50 IU/kg 3 times per week, emicizumab 3mg/kg loading dose once per week for 4 weeks, then 1.5 mg once per week in maintenance dose. 100% of the cases will make the switch in the first year. An average of 3 annual bleeding events per patient is estimated in the current scenario and the results of the Haven II trial for the future scenario as a reference. In both scenarios, a standard case of a 20-kg patient is used as the basis and the entire cohort is projected. No discount rate is applied, a deterministic analysis is performed regarding the price of each product.

## DISCUSSION

When comparing the present impact analysis budget with the one made in Chile in 2020 regarding patients with hemophilia A and inhibi-

tors regarding the use of CCPa, similarities are evident, in the Chilean analysis a savings of 8.7 million dollars was established, although they are different contexts due to the fact that the analysis of Chile included adult patients, the finding of cost reduction coincides with the present analysis with savings for 10.3 million dollars for the health system, it was also found cost reduction of emicizumab versus CCPa in prophylaxis with available in France, Italy, Spain, Australia and South Korea, according to these publications it would not be enough to reduce the cost of the comparator to compensate for the cost savings of the intervention, in the case of the present analysis, to affirm that CCPa should reduce its cost by 63.6% to equalize the direct costs of prophylaxis is not adequate because the impact of the most important clinical variable according to clinical trials that represents the decrease in the Annual Bleeding Rate would be unknown,it is necessary to establish the clinical benefit in the reduction of disability with each avoided bleeding provided by emicizumab and its impact on the quality of life as reported by international analyzes.

## CONCLUSIONS

The BIA shows that emicizumab could reduce prophylaxis costs in pediatric patients with Hemophilia A in the Dominican Republic by USD \$10,390,233 over a 3-year time horizon, reducing annual health care costs by 67.5%. It is recommended in future analyzes to include indirect costs associated with less frequent application and a decrease in the Annual Bleeding Rate.

## RECOMMENDATIONS

The present analysis was carried out in 2021, currently as of September 2022, the Dominican Republic has experience of using emicizumab in patients with and without inhibitors, therefore, they could obtain information on the therapeutic implementation and its clinical and economic impact on their patients in real scenario. The implementation of a registry of patients with Hemophilia A and inhibitors in the Dominican Republic can improve the cost estimation by having minimum variables such as the weights in kilograms of each patient, consumption of hemostatic agents and annual bleeding rate. Currently, the Dominican Republic lacks a registry of patients with hemophilia and detailed information on consumption of hemostatic agents.

Considering that Emicizumab is a subcutaneous technology that decreases the number of applications (16 times per year) compared to CCPa that requires travel to the infusion center for each application (156 intravenous infusions per year), in future analyses, the decrease in out-of-pocket expenses associated with reduced transportation costs in the pediatric patient's caregiver from home to the drug infusion center could be estimated from the social perspective, also considering the travel and associated expenses during hospital care for acute bleeding events.

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