

Reduction of Costs Associated With the Use of Emicizumab in Prophylaxis in Pediatric Patients Diagnosed With Severe Hemophilia A and Inhibitors in El Salvador

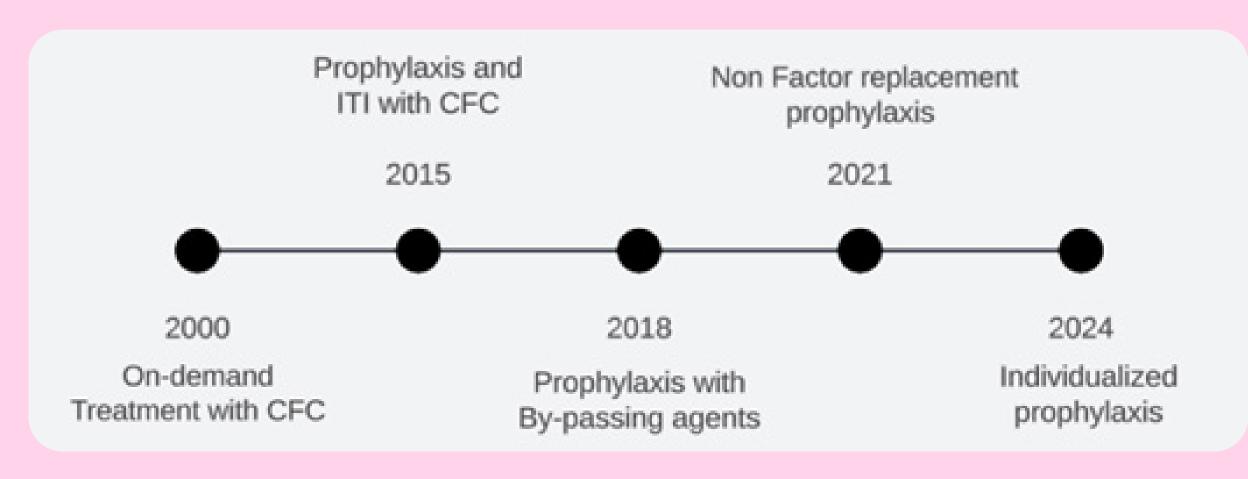
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Context

Hemophilia A is a disease with a high economic impact on health services, especially in those patients who develop Factor VIII inhibitors who have a higher risk of morbidity and mortality associated with different bleeding events. Additionally, the management of patients with Hemophilia A and inhibitors presents greater direct and indirect costs for the health system. Currently, the different modalities of on-demand therapies, prophylaxis and immunotolerance, are based on the use of treatment with replacement agents: clotting factor concentrates (CFC) or bridging agents and prophylaxis with mimetic or non-replacement agents: emicizumab.

At Benjamin Bloom Childrens Hospital, since 2015, patients with hemophilia have received prophylaxis and immunotolerance with replacement agents and recently prophylaxis with mimetic agents. Access to treatment is provided by the partnership between the state and the humanitarian aid program of the World Federation of Hemophilia.

Figure 1 Hemophilia A treatment timeline. Benjamín Bloom Children's Hospital



At Benjamín Bloom Children's Hospital, the annual cost of treatment for a patient with Hemophilia A weighing 20 kg is as follows:

Prophylaxis	Dose	Unit Cost	Annual Cost
FVIII DP	50 UI x Kg	US\$ 0.11/UI	US\$ 17,160
	three times per week		
aPCC	75 UI x Kg	US\$ 1.38 /UI	US\$ 332, 920
	three times per week		
FVII R	90 μg x Kg per day	US\$ 911/ mg	US\$ 665,030
Emicizumab	6 mg / Kg	US\$ 76.66/ mg	US\$ 110,400
	every four weeks		

Objectives

To perform a direct cost comparison between pediatric patients who received immunotolerance with FVIII and bypass agent versus subsequent change of therapy to emicizumab.

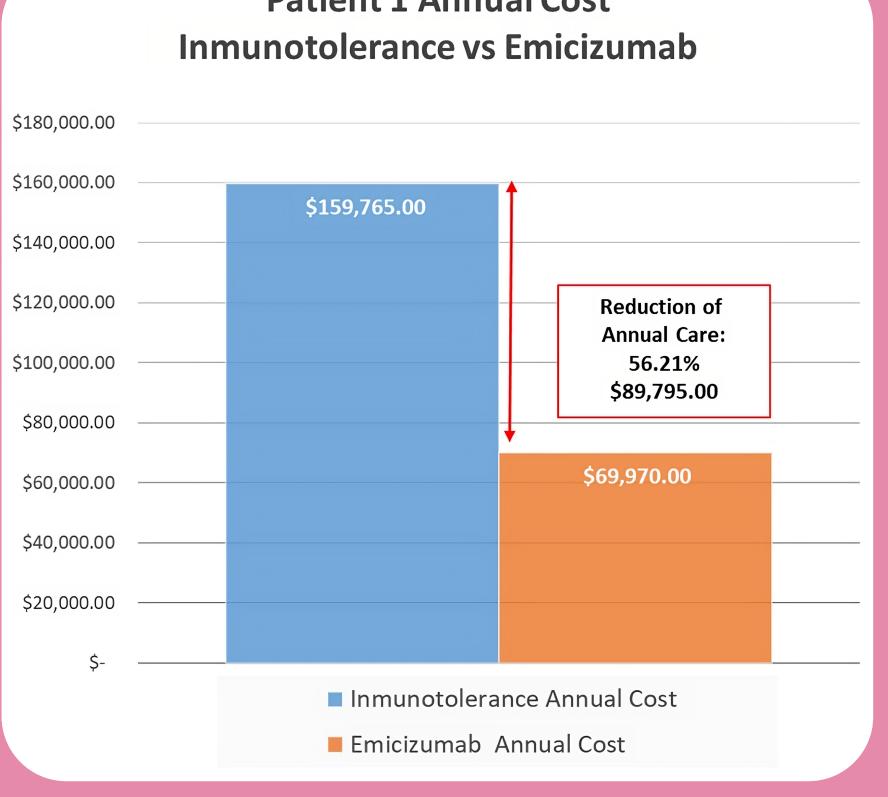
Methods

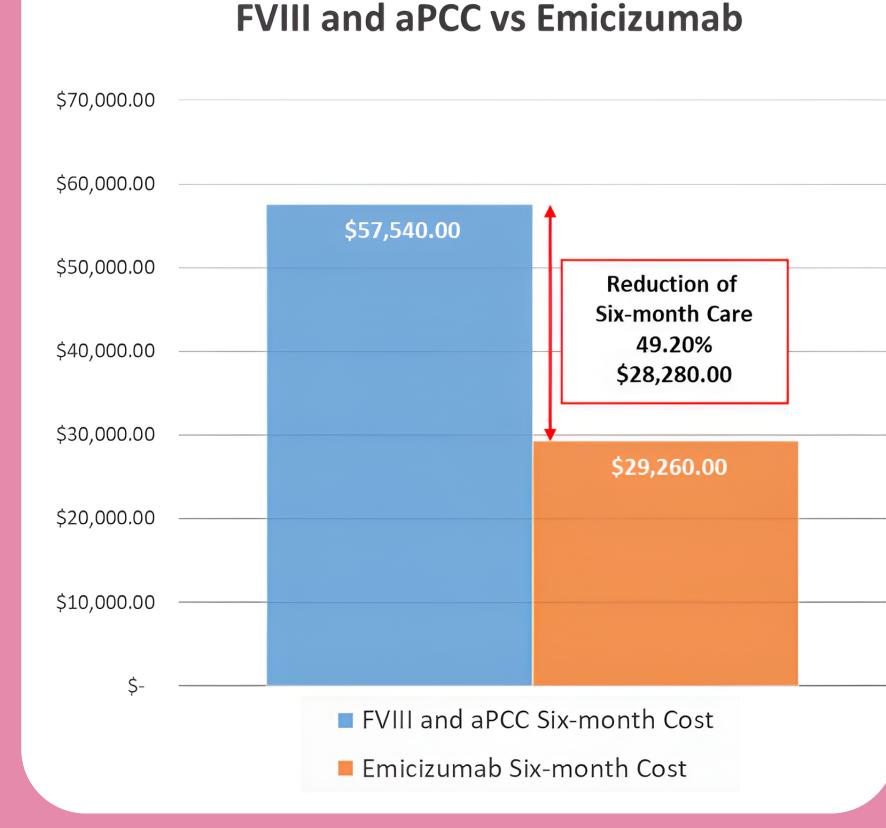
Clinical records of two pediatric patients with Severe Hemophilia A and inhibitors were analyzed, the first case with a time horizon of two years, the first year the patient received FVIII in immunotolerance and the second year emicizumab. The second case with a time horizon of 1 year, during the first 6 months the patient received FVIII and aPCC and subsequently 6 months with emicizumab. In both cases it included costs of medications for prophylaxis, immunotolerance or acute bleeding event, the costs of hospitalizations associated with bleeding events were considered. The costs were obtained from public sources in local currency and converted to american dollars according to the national reference rate, the perspective of the analysis is Hospital institution, no indirect costs were included in the analysis.

Results

In the first case, during the period of immunotolerance use, the total annual cost was \$159,765, of which \$24,801 corresponded to 21 days of hospitalization, the annual cost of emicizumab was \$69,970 and did not require hospitalizations.







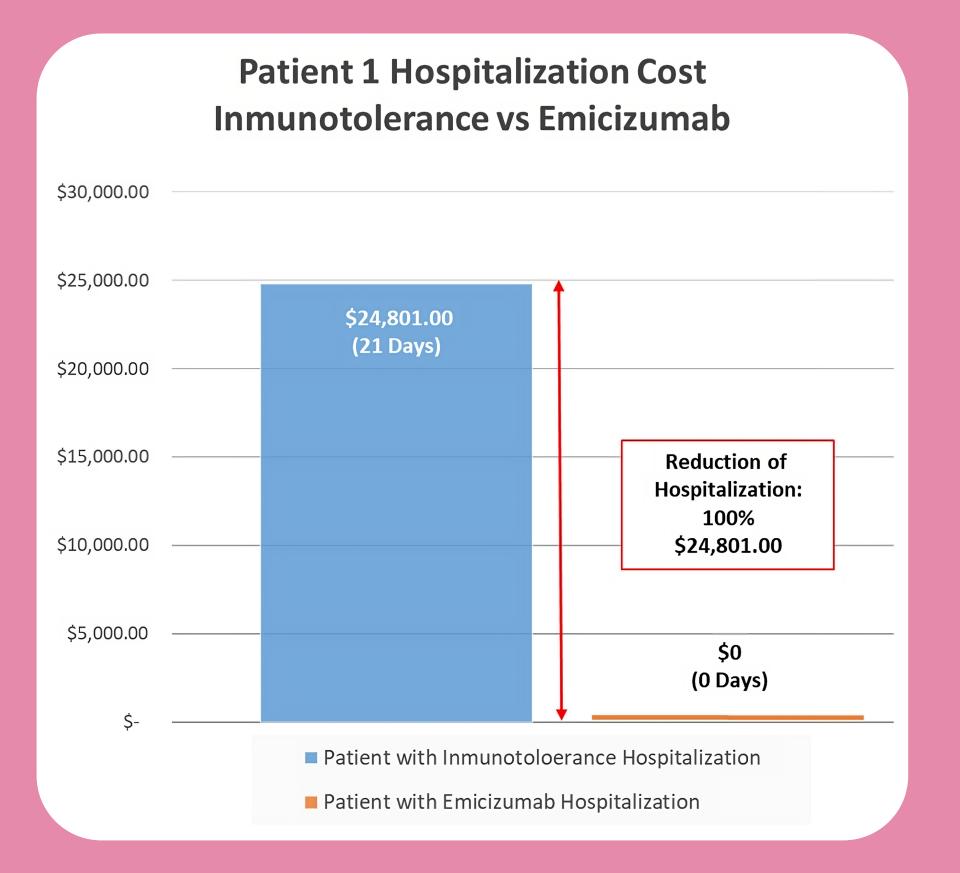
In the second case, the six-month cost using FVIII and

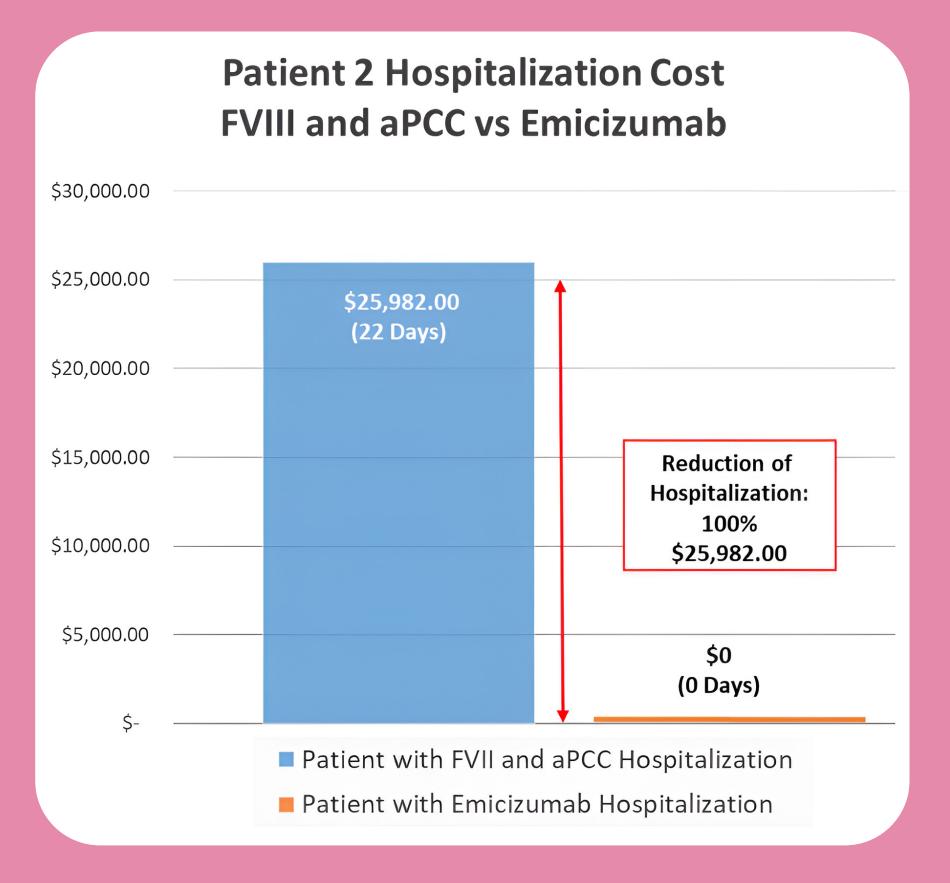
aPCC was \$57,540 of which \$25,982 corresponds to 22

days of hospitalization including Intensive Care, the

six-month cost of using emicizumab was \$29,260 and

did not require hospitalizations.





Conclusions

The use of emicizumab in prophylaxis for pediatric patients with Hemophilia A in El Salvador reduces direct costs of care. In the present analysis, in the first patient the annual care costs were reduced by 56.21% and in the second patient the six-monthly care costs were reduced by 49.2%.

In both cases, during the use of emicizumab, the patients did not present acute bleeding events that required hospitalizations.