

# Population-Level Impact Of Emicizumab In Moderate-Severe Hemophilia A: A Treatment Impact Model in Ecuador

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## Background

Moderate-to-severe hemophilia A is associated with recurrent bleeding episodes, joint damage, disability, and substantial healthcare and societal burden, including productivity losses<sup>1,2</sup>. Prophylactic treatment with emicizumab has been shown to reduce this burden by preventing bleeding events, with associated reductions in healthcare costs, disease-related complications, and work impairment<sup>3,4</sup>. Previous studies have suggested a favorable population-level impact across Latin America; however, country-specific evidence is needed in Ecuador to better inform healthcare planning and access decisions.

## Objectives

To assess the **short- and long-term clinical, economic, and societal impact** of emicizumab adoption in patients with moderate-to-severe **hemophilia A** in Ecuador.

## Methods

A population-level impact model was used to estimate the clinical, economic, and societal impact of emicizumab adoption in Ecuador among patients with moderate-to-severe hemophilia A, with and without inhibitors. Two scenarios were compared: without emicizumab and with emicizumab. For each scenario, the model estimated disease-related outcomes and the associated direct and indirect costs over a 25-year period from local market approval (2020–2045), with assessments at 2025 under current adoption and at 2045 under increased adoption. Inputs included epidemiologic and cost data from public Ecuadorian sources, while effectiveness estimates were informed by the HAVEN trials. Outcomes included bleeding events, hospitalization days, and joint replacements avoided, as well as direct and indirect costs avoided and quality-adjusted life-years (QALYs) gained.

## Results

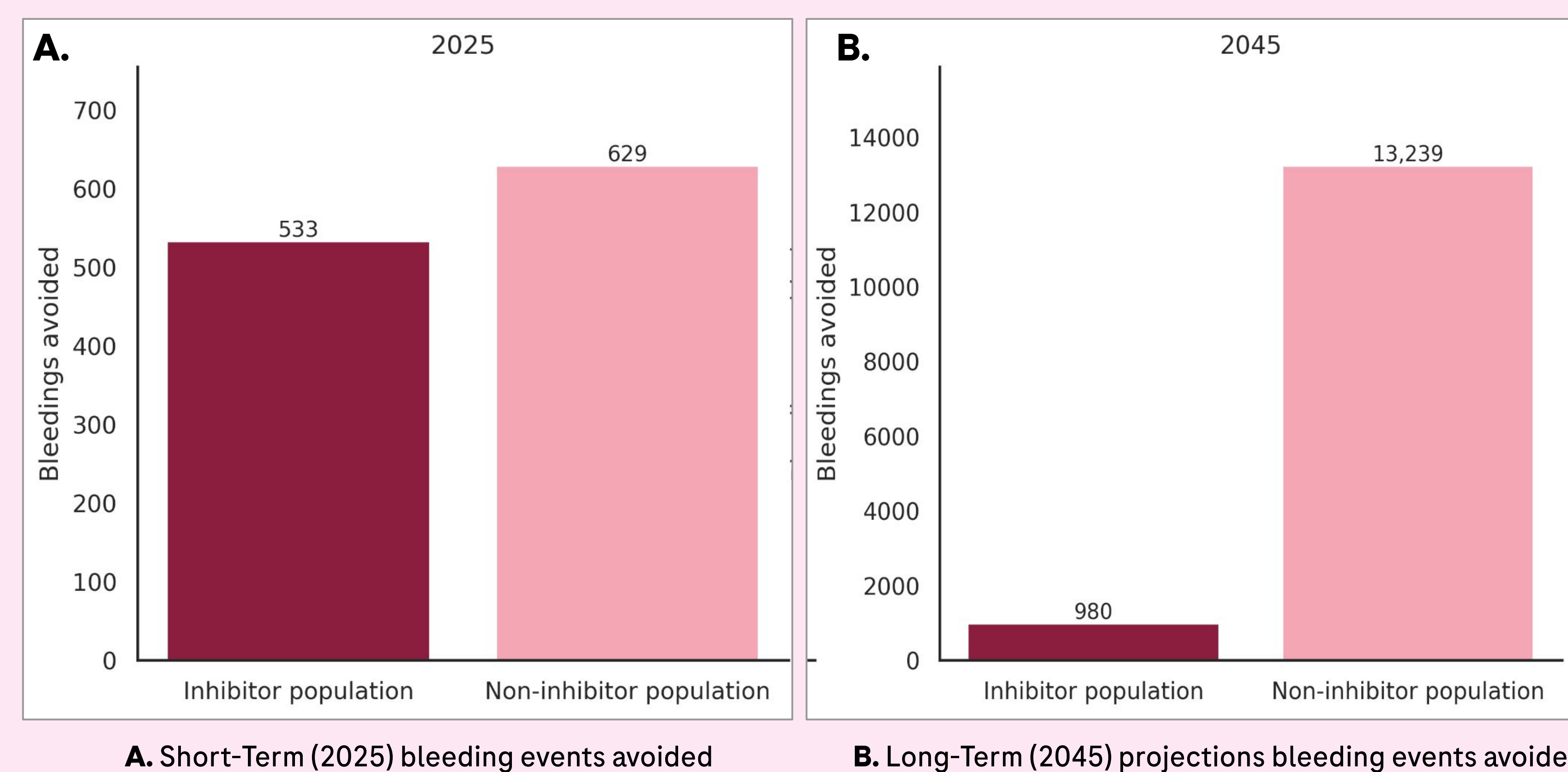
The study analyzed a total of **764 patients in Ecuador, 94.4% without inhibitors**. The introduction of emicizumab projects a **21.7% reduction** in the prevalence of patients **with inhibitors (INH)** by 2045.

The **clinical benefit** of prophylaxis with emicizumab **grows exponentially as its adoption expands across the population (Figure 1)**:

- **Short-term (2025):** The model estimates **1,162 prevented bleeds**, primarily driven by early impact in patients with inhibitors due to high emicizumab adoption (a 54.2% reduction in this cohort).
- **Long-term (2045):** The cumulative impact reaches **14,219 avoided bleedings, 72.8% in the INH cohort and 64.7% in the non-INH cohort**.

Importantly, **73% of all bleeding events prevented by 2045 were joint bleeds (hemarthrosis)**. Reducing these events may help prevent irreversible joint damage and was associated with projected reductions in joint replacement surgeries of **79.0% (INH) and 68.0% (non-INH) patients**.

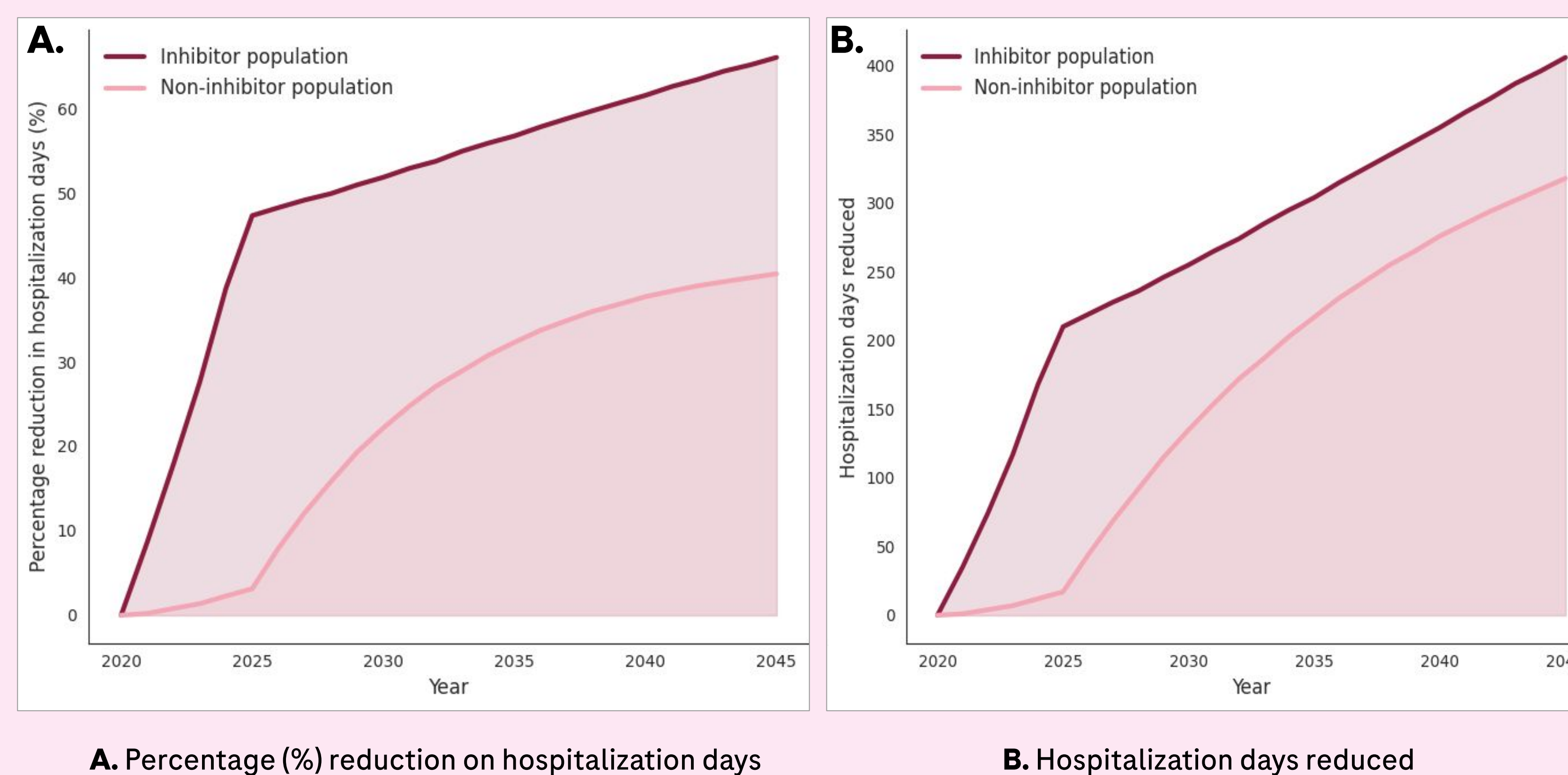
Figure 1. Avoided Bleeding Events in Ecuador by presence of inhibitors



## Economic & social impact

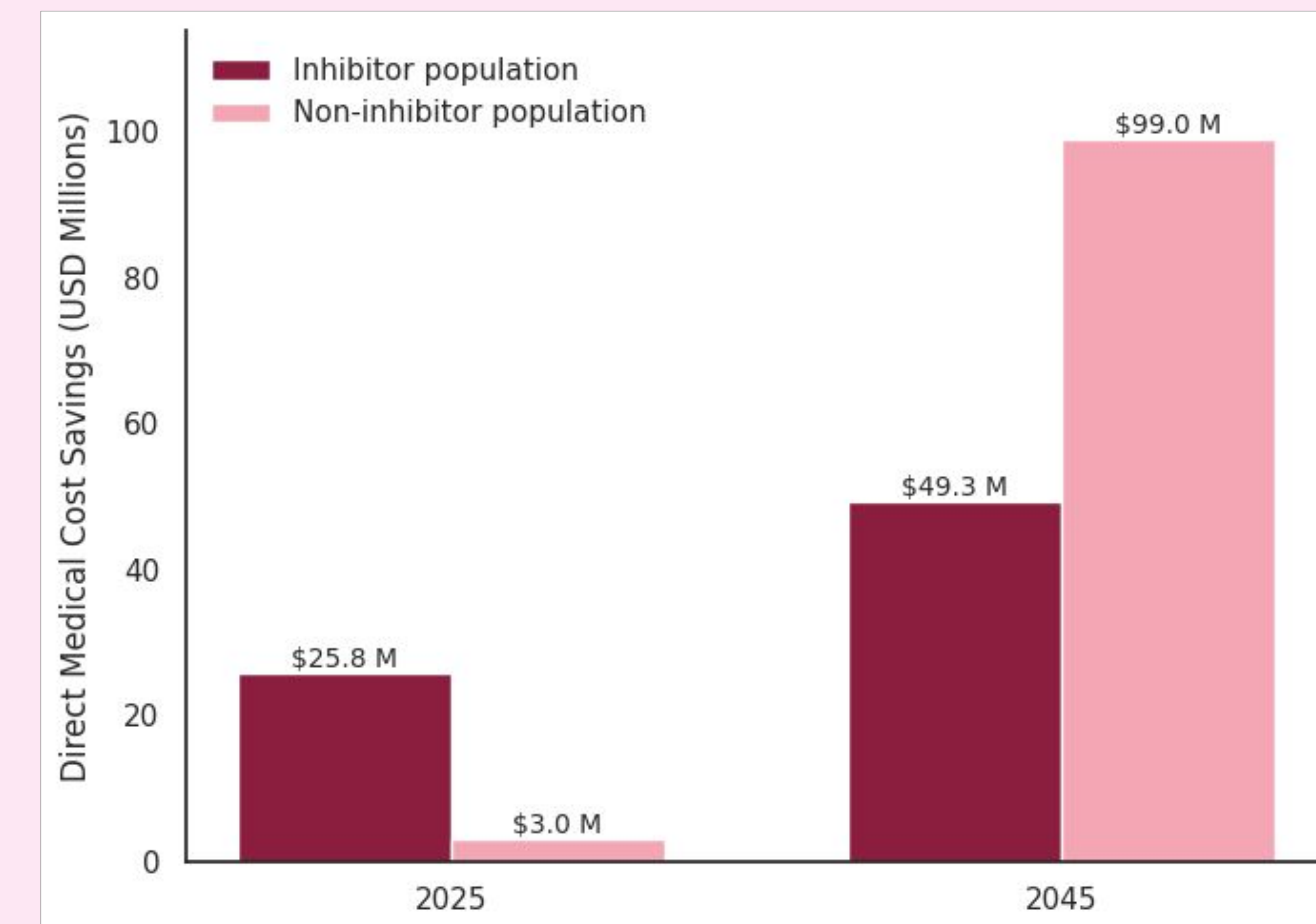
The introduction of emicizumab reduces hospital resource utilization (Figure 2). By 2025, the INH population shows a **47.3% reduction in hospitalization days (234 vs. 444 days)**, a benefit that increases to a **66% reduction (209 vs. 615 days)** by 2045. In contrast, in the non-INH patients the benefit increased from 17 hospitalization days avoided in 2025 to a 40.3% reduction by 2045, corresponding to 317 fewer hospitalization days annually (469 vs. 787).

Figure 2. Projected Reduction in Hospitalization Days (2020–2045) Following Emicizumab Introduction



By 2045, cumulative direct medical cost savings associated with acute bleeding episodes, hospitalizations, and complications reached **USD 148.3 million (Figure 3)**, corresponding to reductions of 72.9% in the INH cohort and 64.0% in the non-INH cohort. Of these savings, bleeding management alone accounted for **USD 49.19 million** in averted costs in patients with INH and **USD 98.96 million** in patients in the non-INH cohort.

Figure 3. Comparative Economic Impact of Emicizumab: Annual Cost Savings for Inhibitor and Non-Inhibitor Populations



These clinical and economic gains were accompanied by an estimated **118 QALYs gained across both cohorts by 2045**. In addition, by 2025, the model calculated the prevention of 5,385 days of work inability in the INH cohort and 4,111 days in the non-INH cohort, generating indirect cost savings of USD 86,743 and USD 66,221, respectively. By 2045, these gains increased to **10,030 days of work inability avoided (-70.4%) in the INH cohort and 87,240 days (-63.6%) in the non-INH cohort**, translating into cumulative indirect cost savings of USD 161,578 and USD 1.40 million, respectively.

## Conclusions

Emicizumab was associated with meaningful reductions in the clinical and socio-economic burden of hemophilia A in Ecuador. Short-term impact reflected higher initial uptake in INH patients, whereas long-term benefits grew with broader adoption in the non-INH population. The projected reductions in joint bleeds, healthcare utilization, and productivity loss provide evidence supporting expanded adoption of emicizumab. Projected reductions in joint bleeds, healthcare utilization, and productivity losses may translate into improved quality of life, preserved functional outcomes, and greater ability to participate in daily and work activities. By preventing costly complications and high resource consuming events in both populations with and without inhibitors, these benefits support the long-term financial sustainability of the healthcare system.

## References

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