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Long-Term Impact of Emicizumab in Hemophilia A: Analysis by Inhibitor Status in three Latin American (LATAM) countries

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Introduction

Hemophilia A (HA) is a severe hereditary bleeding disorder that has been managed with the exogenous administration of plasma-derived or recombinant FVIII concentrates[1,2.] Despite advancements, treatment has led to a rise in the development of inhibitors [2,3]. Presence of inhibitors increases morbidity, bleeding, disability, and decreases quality of life. Faced with these challenges, prophylactic treatment with **emicizumab** has shown significant reductions in annualized bleeding rates with an adequate safety profile in patients with HA, both with and without inhibitors[4-8.]

Objective

To evaluate the clinical, economic, and social impact of emicizumab in patients with moderate-to-severe HA, with and without inhibitors, within public and private healthcare systems in Peru (PE), Mexico (MX), and Chile (CL).

The study assesses short-term outcomes under current adoption scenarios and models long-term effects over a 25-year horizon, assuming broad emicizumab uptake.

Methodology

This treatment impact model (TIM) estimates the quality-adjusted life years (QALYs) gained and the number of bleeding events, hospitalization days, arthroplasties, direct medical costs, and productivity losses (indirect costs) avoided in patients treated with emicizumab versus no emicizumab, providing key inputs for healthcare decision-making in the region.

The TIM quantifies the impact of introducing emicizumab as a prophylactic therapy for HA in patients with and without inhibitors. The model was adapted to Latin American countries such as PE, MEX, CL private system (CL-Priv), and CL public system (CL-Pub). Local epidemiological and cost data related to the consequences of HA were sourced from public databases and published literature, and subsequently validated by local clinical experts to ensure consistency with real-world clinical practice. Clinical efficacy parameters were derived from the HAVEN trials.

Results

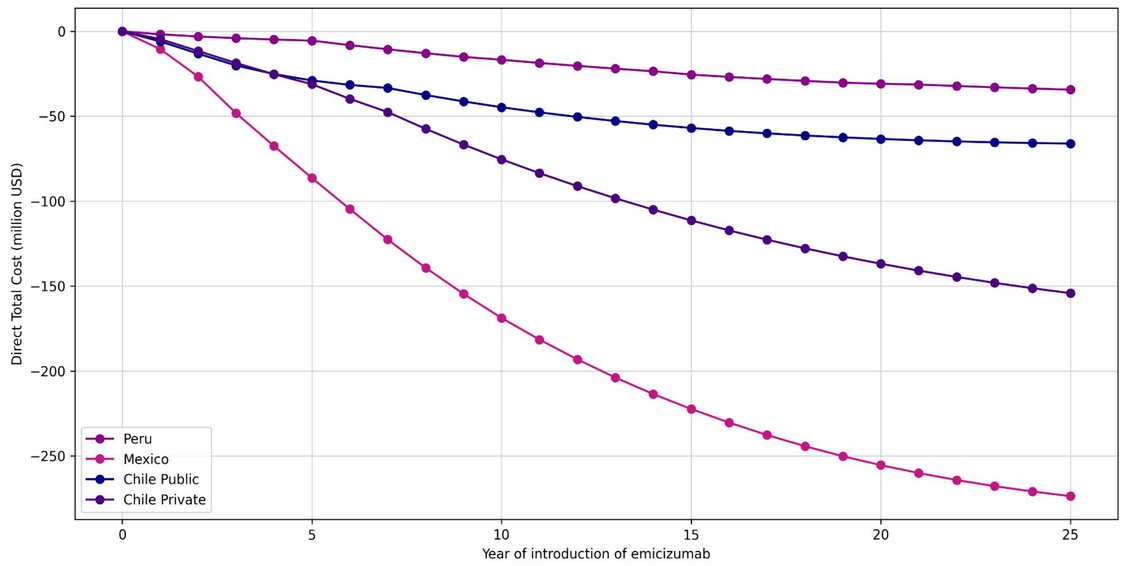
The total number of hemophilia A (HA) patients at the time of emicizumab market entry was 748 in CL-Pub and 230 in CL-Priv (both in 2018), 1,081 in MEX (2019), and 966 in PE (2020).

By 2024, notable reductions in bleeding events were observed across the analyzed countries: CL-Pub (-1017), CL-Priv (-607), PE (-271), and MEX (-2,105). These reductions represent relative decreases ranging from 1.9% (PE) to 13.6% (MEX) associated with emicizumab use. By the 25-year projection, the number of avoided bleeding episodes reaches -3,809 in CL-Pub, -2,953 in CL-Priv, -8,376 in PE, and -8,476 in MEX. Stratified analyses by inhibitor status show a more pronounced impact among patients with inhibitors, with reductions of up to −75.7% in PE. In patients without inhibitors, reductions remain substantial but slightly lower, between -2,605 (CL-Priv) to -8,179 (PE), bleeding events avoided and relative decreases ranging from 58.8% (CL-Pub) to 63.3% (MEX).

As of 2024, healthcare systems in CL, PE, and MEX have already experienced significant cost savings following emicizumab market entry, with direct medical cost reductions estimated at USD 31.6 million for CL-Pub, USD 39.8 million for CL-Priv, USD 4.8 million for PE, and USD 86.3 million for MEX. The total direct costs avoided by 2024 amounts to USD 162 million. Of this total, nearly 70% (USD 112 million) corresponds to patients with inhibitors.

By the 25-year projection, these savings are projected to grow substantially, reaching USD 66.1 million in CL-Pub, USD 154.2 million in Cl-Priv, USD 34.4 million in PE, and USD 273.6 million in MEX (**Figure 1).**

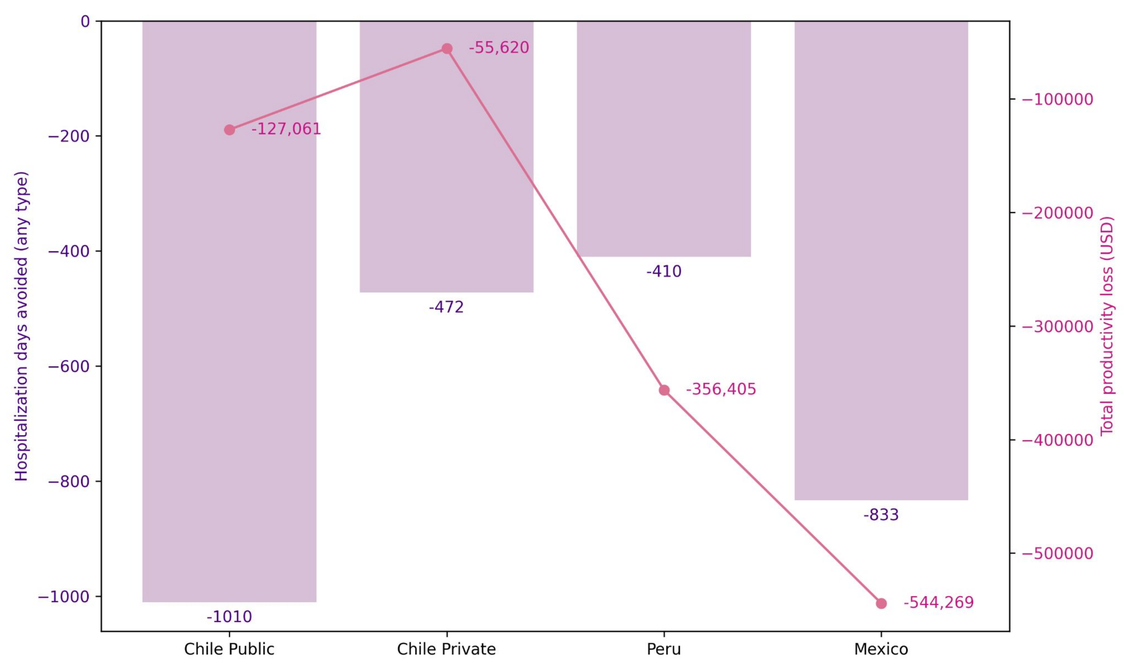
Figure 1. Projected 25-year changes in direct costs with emicizumab uptake in patients with and without inhibitors



Among patients with inhibitors, the highest number of additional QALYs was observed between 2024 and 2029, reaching 27 in CL-Pub (2025), 11 in CL-Priv (2025), 3 in PE (2023), and 18 in MEX (2034). These peaks reflect the point of maximum clinical benefit prior to full emicizumab uptake. For patients without inhibitors, the projected QALY gains by the year 25 of the model were estimated at 35 for CL-Pub, 28 for CL-Priv, 66 for PE, and 72 for MEX.

The model projected reductions in hospitalization days of 1,010 in CL-Pub, 472 in CL-Priv, 410 in PE, and 833 in MEX. Estimated productivity losses associated with hemophilia-related events were estimated at USD 127,062 in CL-Pub, USD 55,621 in CL-Priv, USD 356,405 in PE, and USD 544,269 in MEX. These results highlight both the health and economic value of reducing hemophilia-related events, as shown in **Figure 2.**

Figure 2. Difference in hospitalization days and indirect costs due to work inability with emicizumab in patients with and without inhibitors



Conclusions

While high adoption of emicizumab has already resulted in substantial benefits for patients with inhibitors, the findings strongly support national strategies to expand access to patients without inhibitors. Broader adoption can further reduce bleeding events, hospitalizations, and productivity losses, leading to meaningful cost savings over time. Expanding access would also help close outcome gaps between patient groups and support more equitable, sustainable, and value-based hemophilia care across Latin America.

References

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