

# Improving Precision of Societal Budget Impact of emicizumab Delivery Options in France: Insights from the HemiValue Study Using Real-World Data



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### Objectives

emicizumab is the first prophylactic treatment for hemophilia that can be administered subcutaneously. It has been approved in 2018 for severe hemophilia A (HA), a hereditary bleeding disorder where blood clotting is impaired, leading to prolonged and spontaneous bleeding that can result in significant complications such as long-term joint damage or intracerebral hemorrhage. Prior to June 2021, emicizumab was exclusively dispensed through hospital pharmacies (HP) in France, requiring frequent hospital visits, which added strain on both patients and carers. Since June 2021, emicizumab has also been available in community pharmacies (CP), providing a more accessible alternative and reducing the need for routine hospital trips.

A previous budget impact (BI) analysis, primarily based on published literature not specific to hemophilia patients, suggested a reduced burden for patients and carers. **This study aims to build on that work by incorporating real-world data (RWD) to provide a more precise assessment of the budget impact of this dual dispensing approach.**

### Conclusions

The availability of emicizumab in CP has significantly reduced the financial and logistical burdens for hemophilia A (HA) patients and their carers. While the previous budget impact analysis, based on literature not specific to hemophilia patients, already indicated a reduction in patient and caregiver burden, these findings were further validated and refined with the integration of RWD. The RWD not only enhanced the precision and contextual relevance of the analysis but also underscored the clear benefits of CP delivery. By reducing the time and costs associated with HP visits, especially for low-income families and families far from hospital pharmacies, CP availability has the potential to alleviate income and geographical inequalities.

While dual dispensing introduces challenges—such as ensuring the safety and quality of treatment administration, facilitating effective communication between healthcare professionals, and adequately training community pharmacists on therapeutic education—it has the potential to reduce the overall burden of treatment for patients already managing a chronic disease. When properly implemented, dual dispensing can improve patient and caregiver satisfaction, offering a more flexible, patient-centered approach to hemophilia care.

### Methods

An existing BI model was updated with results from the PASO DOBLE DEMI<sup>1-4</sup> study to estimate the time and costs associated with one year of emicizumab delivery from the patient or caregiver perspective. PASO DOBLE DEMI<sup>1</sup> is a national cross-sectional study, which generated RWD to evaluate the systemic impact of CP dispensing on patient outcomes. Costs included transportation to and from HP or CP, accounting for average distances, modes of transportation, and wage losses for self-employed individuals due to time spent in transport and waiting for drug delivery. The BI was assessed separately for HP and CP delivery, as well as for the overall population of emicizumab users in France. Model parameters were informed by a systematic literature review and data from the PASO DOBLE DEMI<sup>1</sup> study.

Two scenarios were compared, assuming 1,450 patients with hemophilia A (HA) are currently receiving emicizumab in France: the first scenario reflecting HP-only availability prior to CP access, and the second scenario representing dual availability of HP and CP, with an assumption that 60% of patients opted for CP in 2022. The updated model outcomes were compared to those derived from previous assumptions, allowing for a more accurate assessment of the real-world impact of CP availability on time and costs for patients.

### Results

The inclusion of RWD:

- Increased the estimated burden of accessing emicizumab, amplifying the differences between HP and CP delivery. Despite these adjustments, the overall conclusions of the analysis remain consistent.
- Confirmed previous estimates, showing that accessing HP significantly reduces the economic burden of obtaining emicizumab for low-income individuals (1st decile) compared to high-income individuals (last decile). Additionally, the RWD enabled an estimation of the impact of travel distance impact, revealing that the CP option provides substantial cost relief for patients or carers living more than 30 minutes away from the hospital.
- Reduced uncertainty, as indicated by the smaller confidence intervals, while the overall conclusions remain consistent.

Table 1. Impact Model Parameters & Sources

	Original Parameters		Updated Parameters with RWD		
	Hospital Pharmacy	Community Pharmacy	Hospital Pharmacy	Community Pharmacy	
Number of patients treated in 2022	500	500	580	870	Updated based on sales data and proportion of hospital / community in the PASO DOBLE DEMI study <sup>2</sup>
Average travel times (minutes)	20 [10-30]	5 [5-10]	39 [1-147]	6 [5-7]	PASO DOBLE DEMI study <sup>1-4</sup>
Average time spent waiting (min)	20 [15-25]	6 [5-7]	15 [2-44]	8 [1-23]	PASO DOBLE DEMI study <sup>1-4</sup>
Average distance traveled (km)	22.5 [18.0-27.0]	3.8 [3.0-4.6]	17.3 [13.8-20.7]	4.3 [3.5-5.2]	Extrapolated based on expected distribution of travel times, and average speeds in France
Mode of transportation					
Car	88.5%	66.7%			PASO DOBLE DEMI study <sup>1-4</sup>
Foot	1.9%	26.8%			PASO DOBLE DEMI study <sup>1-4</sup>
Public transport	9.6%	1.6%			PASO DOBLE DEMI study <sup>1-4</sup>
Proportion of patients or carers taking half a day to go the pharmacy	75%	0%	29%	0%	Assumption based on proportion of patients/carers living more than 30 min from the pharmacy <sup>2</sup>
Transportation costs (€)					
Car (per km)		0.339			French Tax rate
Public transport (on way)		1.90			Average cost from major cities in France
Average income in France (€)		2448.1			INSEE, 2020
Proportion of adults employed		82%			INSEE, 2020
Proportion of independently employed		11.4%			INSEE, 2020
Frequency of pharmacy visits		Once per month			Assumption

Table 2. Time and Costs associated with a year of emicizumab when going to hospital (HP) compared to community (CP) pharmacies.

	HP	CP	Δ
Time Spent (hours)			
Previous analyses	37.4	1.2	36.2 (97%)
Updated with RWD	26.0	4.3	21.7 (83%)
Direct Costs (€)			
Previous analyses	298.0	10.0	288.0 (97%)
Updated with RWD	341.5	19.7	321.8 (94%)
Proportion of available income			
Previous analyses	1.9%	0.1%	1.8%
Updated with RWD	2.6%	0.2%	2.5%

Table 3. Costs saved depending on revenue bracket and distance from the hospital pharmacy (updated with RWD)

	Revenue bracket		Δ
	1st decile	Last decile	
Average Cost for HP (€)	241	467	
Average Cost for CP (€)	20	20	
Δ HP vs CP (€)	222	447	
Proportion of available income			
HP	5.3%	1.8%	-3.5%
CP	0.4%	0.1%	-0.4%
	Distance from the hospital pharmacy		Δ
	< 10 min	> 30 min	
Average Cost for HP (€)	126	540	
Average Cost for CP (€)	20	20	
Δ HP vs CP (€)	106	520	414
Proportion of available income			
HP	1.0%	4.1%	3.1%
CP	0.2%	0.2%	0.0%

### References

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Figure 1. Comparison of probabilistic sensitivity analysis results for total time saved and annual cost savings for patients and carers, comparing the current dual dispensing system to a scenario without it. The figure contrasts the previous findings with the updated results incorporating real-world data (RWD).

