# Caplacizumab: hospital budget impact and clinically appropriate use at the University Public Hospitals of Paris, France (AP-HP)

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Improving healthcare decisions

## OBJECTIVES

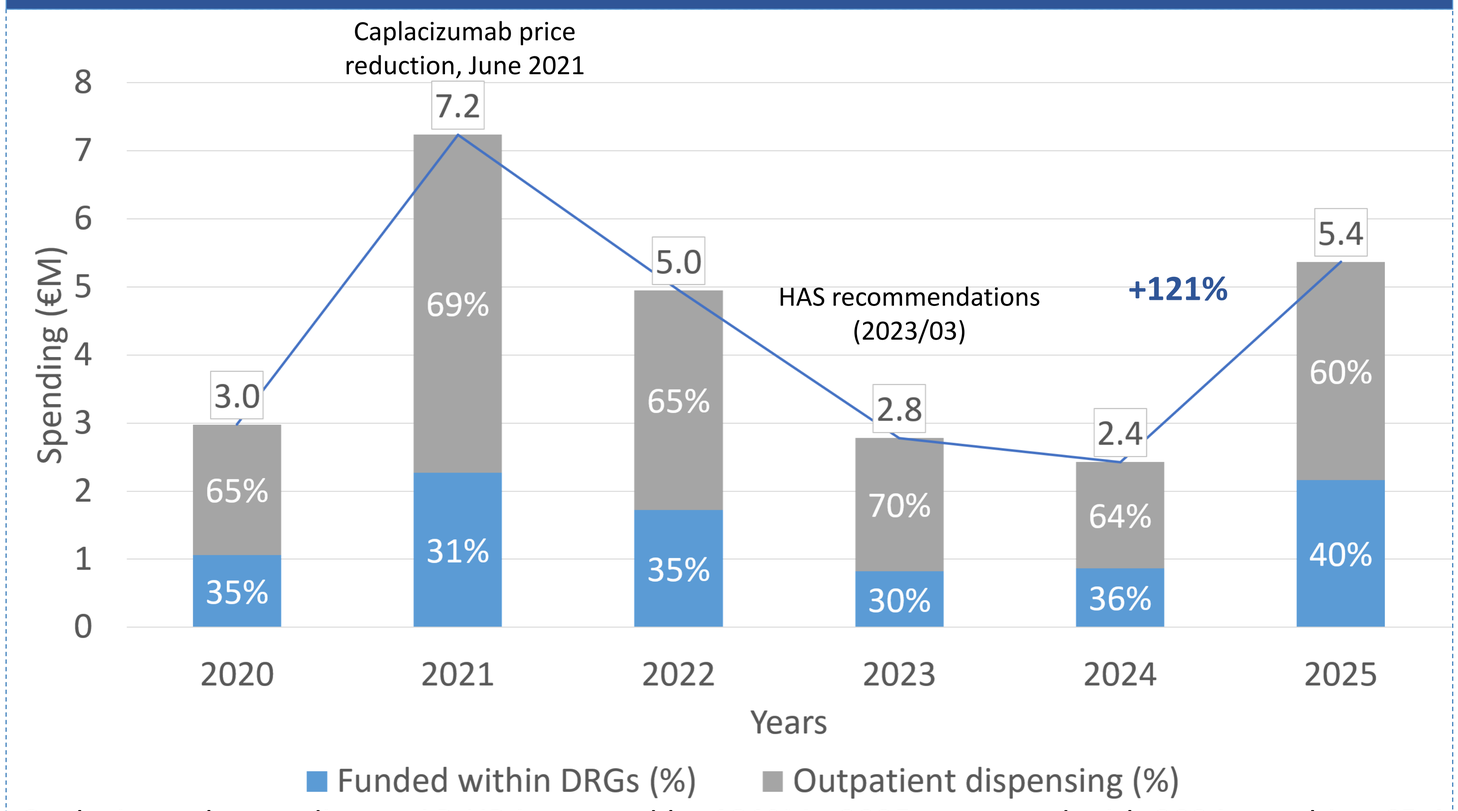
In France, hospital drugs are reimbursed through the Diagnosis-Related Group (DRG)-based payment system. Innovative, expensive drugs are funded in addition to the DRGs only if they meet specific criteria. Acquired thrombotic thrombocytopenic purpura (aTTP) is a rare, life-threatening thrombotic microangiopathy; since 31/08/2018, caplacizumab has been indicated, in combination with plasma exchange, for patients ≥12 years and ≥40 kg experiencing an aTTP episode. As caplacizumab is not funded in addition to DRGs, its cost is borne by hospitals during hospitalization and, after discharge, is reimbursed through hospital outpatient dispensing (retrocession). This study aimed to quantify caplacizumab's budget impact for AP-HP hospitals and to assess how treatment duration recommendations influence the cost per treated episode, comparing Marketing Authorisation and HAS recommendation-based scenarios.

## METHODS

Caplacizumab consumptions and expenditure data were extracted from the AP-HP hospitals discharge database between 2020 and 2025. Annual expenditure, the estimated number of treated episodes and the mean cost per treated aTTP episode were assessed. Two hypothetical cost scenarios were compared: a Marketing Authorisation scenario and a scenario based on the Haute Autorité de Santé (HAS) recommendations and real-world clinical practice.

## RESULTS

**Figure 1 : Caplacizumab Spending at AP-HP (2020-2025)**




Caplacizumab spending at AP-HP increased by **121%** in 2025 compared with 2024, reaching €5.4 million. Expenditure comprised €2.2 million funded within DRGs and €3.2 million reimbursed through hospital outpatient dispensing.

**Figure 2 : Estimated number of aTTP episodes treated**

This increase was associated with **a rise** in the number of treated episodes, from around **20** in 2024 to approximately **45** in 2025.

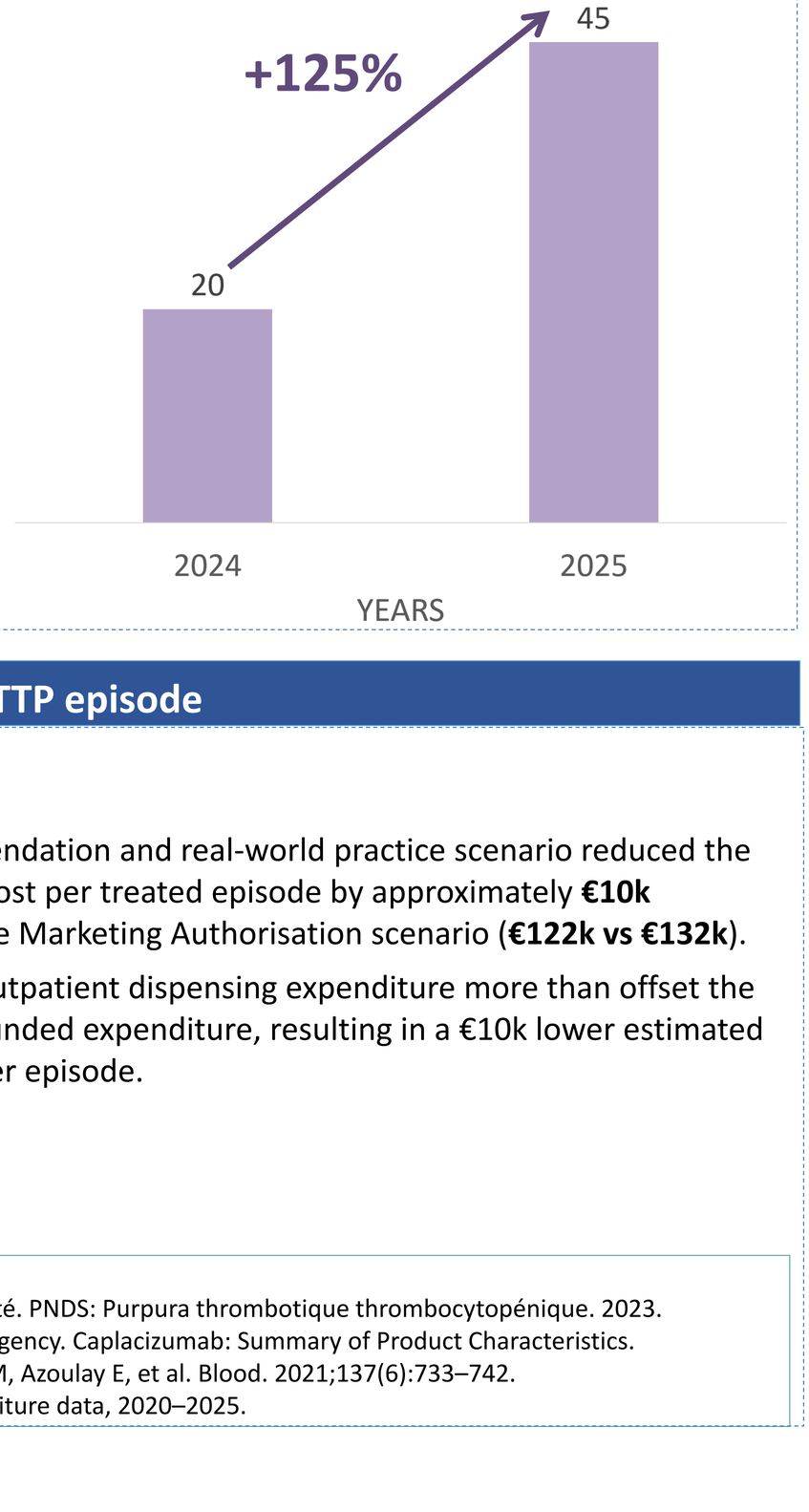



**Figure 3 : Mean caplacizumab cost per treated aTTP episode**




The HAS recommendation and real-world practice scenario reduced the estimated mean cost per treated episode by approximately **€10k** compared with the Marketing Authorisation scenario (**€122k vs €132k**).

The decrease in outpatient dispensing expenditure more than offset the increase in DRG-funded expenditure, resulting in a €10k lower estimated mean total cost per episode.

References:
1. Haute Autorité de Santé. PNDS: Purpura thrombotique thrombocytopénique. 2023.
2. European Medicines Agency. Caplacizumab: Summary of Product Characteristics.
3. Coppo P, Bubenheim M, Azoulay E, et al. Blood. 2021;137(6):733–742.
4. AP-HP internal expenditure data, 2020–2025.

## CONCLUSION

Caplacizumab expenditure at AP-HP increased substantially between 2024 and 2025, mainly driven by a higher number of treated aTTP episodes. The HAS recommendation-based and real-world practice scenario was associated with a lower mean total cost per episode than the Marketing Authorisation scenario, due to lower outpatient dispensing expenditure. However, the share borne by hospitals within DRGs increased. These findings highlight the gap between overall savings and hospital-borne costs, and the importance of monitoring real-world drug spending and using economic assessment tools to identify medicines requiring targeted institutional oversight, to promote clinically appropriate and financially sustainable use.

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