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Caplacizumab for the treatment of acquired thrombotic thrombocytopenic purpura (aTTP): Technology Qualification in Colombia

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INTRODUCTION

Internationally, caplacizumab has been included as part of the initial treatment of acquired thrombotic thrombocytopenic purpura (aTTP) along with plasma exchange and immunosuppression[1-6]. In Colombia, caplacizumab was approved in January 2022.

OBJECTIVE

To determine the therapeutic qualification of caplacizumab in conjunction with plasma exchange and immunosuppression, compared with the pre-caplacizumab standard regimen (plasma exchange and immunosuppression), using the methodology of the local HTA agency (Colombian Institute of Technological Evaluation in Health)[7,8].

METHODS

- Qualification was performed following the modified Delphi technique with a panel of experts composed of four haemato-oncologists, a pharmaceutical chemist, and one patient.
- For the qualification, the results of effectiveness and safety obtained through a systematic review of the literature (statistical significance), therapeutic threshold values (clinical significance), and degree of acceptability (willingness to use the technology) were considered.
- The threshold and acceptability were previously established in a deliberative process with the same panel of experts.

POSTER HIGHLIGHT: This is the first Sanofi experience applying the new effectiveness and safety Colombian guideline for HTA. Treatment using caplacizumab together with plasma exchange and immunosuppression was considered superior to plasma exchange and immunosuppression for the treatment of patients with aTTP.

Figure 1: PRISMA flowchart for effectiveness and safety evaluation

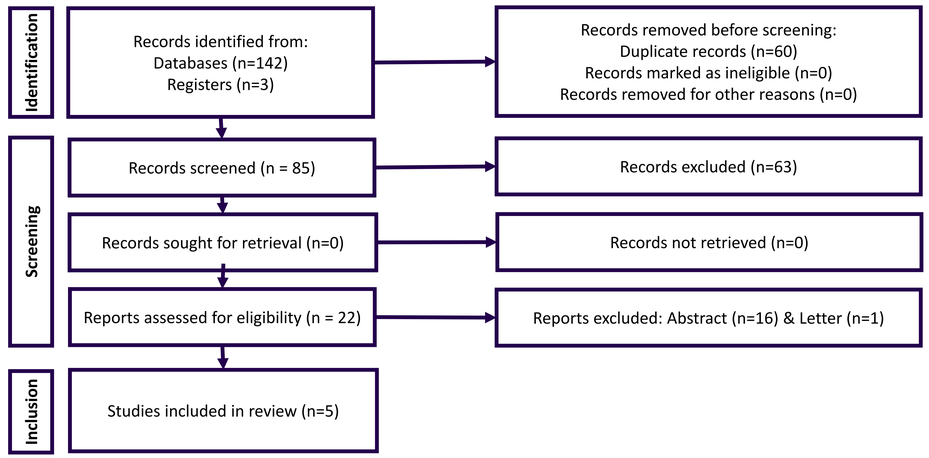

Table 1: Outcomes included in the Qualification

Effectiveness outcomes	Safety outcomes
Response to treatment	Serious adverse events
Response time to treatment	Bleeding adverse events
Recurrences (exacerbation and relapse)	Serious bleeding adverse events
Composite outcome (aTTP-related death, aTTP recurrence, or at least one major thromboembolic event during the treatment period of the clinical trial)	Gingival bleeding
Composite outcome (Death or refractoriness within 30 days of diagnosis)	Epistaxis
Refractoriness	
Exacerbation	
Number of days of plasma exchange	
Number of days of hospitalization	

CONCLUSIONS

Treatment using caplacizumab together with plasma exchange and immunosuppression was considered superior for the standard treatment of aTTP, since it showed clinically significant benefits in critical outcomes and has a safety profile which is not different from its comparator.

RESULTS

- Fourteen effectiveness and safety outcomes were submitted for the qualification process (Table 1).
- Caplacizumab displayed clinical significance for some effectiveness outcomes, was not considered inferior in terms of safety, and showed acceptability for its use.
- By consensus, the panel determined that the technology of interest is superior to the pre-caplacizumab standard treatment, in terms of treatment response and composite outcome (aTTP-related death, aTTP recurrence, or at least one major thromboembolic event), and is not different for other effectiveness and safety outcomes.
- In overall terms, the panel established that using caplacizumab is superior to the pre-caplacizumab standard treatment.

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