COST-EFFECTIVENESS OF CAPLACIZUMAB FOR THE TREATMENT OF THROMBOTIC THROMBOCYTOPENIC PURPURA IN BRAZIL

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INTRODUCTION

- Acquired thrombotic thrombocytopenic purpura (aTTP) is a rare autoimmune thrombotic microangiopathy characterized by hemolytic anemia and thrombocytopenia.
- Caplacizumab is an innovative treatment, that leads to faster platelet count normalization compared to placebo.[1]
- In the HERCULES trial, a double-blind randomized clinical study with 145 patients, the composite endpoint of aTTP-related death, aTTP recurrence, or thromboembolic event during the treatment period was 74% lower with caplacizumab than with placebo (12% vs. 49%, p<0.001).[1]

OBJECTIVE

- To evaluate the cost-effectiveness of caplacizumab for the treatment of patients with aTTP compared to standard of care from the perspective of the Brazilian Unified Health System (SUS).
- To investigate if investing in future research would be worthwhile from the SUS perspective.

METHOD

A decision tree followed by a Markov model with a lifetime horizon was developed.

- Patients entered the model with an acute aTTP event. All patients were assumed to be admitted to hospital where they either respond to treatment or die.
- The model offered three health states: remission, relapse or death. Input data were obtained from literature searches with the data on efficacy of caplacizumab based on the HERCULES trial.
- The incremental cost-effectiveness ratio (ICER) was compared to the willingness-to-pay threshold for rare diseases of R$120,000/QALY defined in the Brazilian Guideline for economic evaluations.[2]
- A value of information (VOI) analysis was conducted using the Sheffield Accelerated Value of Information (SAVI) web-tool. [3]

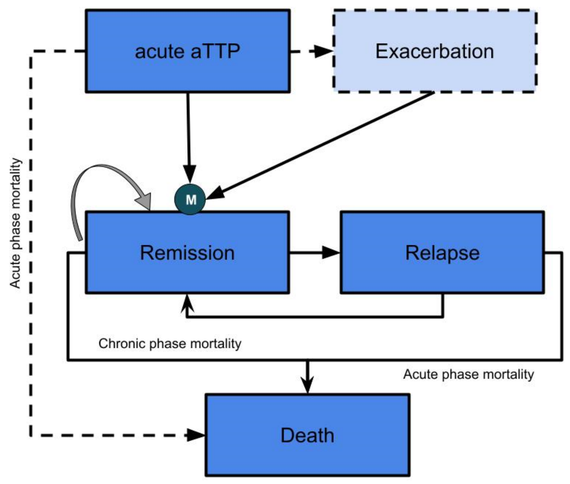

Figure 1. Model structure

RESULTS

- In the base case, the caplacizumab strategy costed R$ 1,372,780 and standard of care R$ 566,243, resulting in an additional cost of R$ 806,537.
- Treatment with caplacizumab was more effective (9.74 vs. 8.96 QALYs), resulting in 0.78 additional QALYs.
- The incremental cost-effectiveness ratio was R$ 1,030,496 per QALY gained.
- The cost of the caplacizumab vial was the most influential parameter as shown in deterministic sensitivity analysis.
- Probabilistic analysis showed that caplacizumab was cost-effective in less than 10% of iterations.

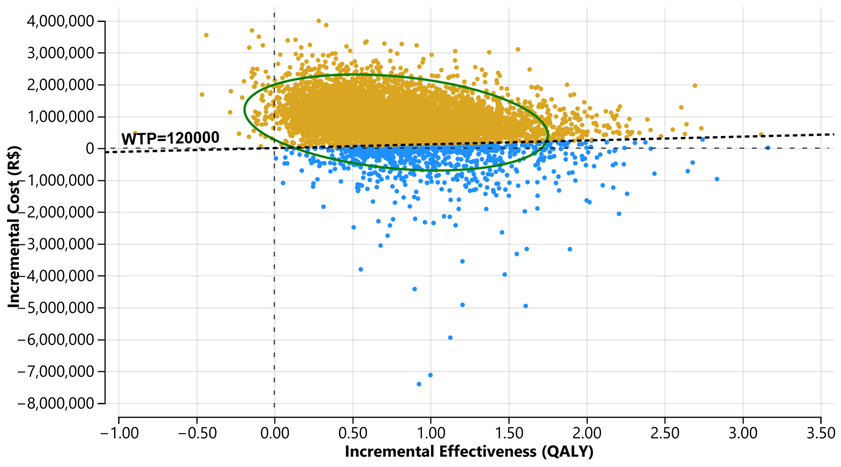

Figure 2. Incremental cost-effectiveness plane.
90% of iterations fall on the upper right quadrant and above the WTP threshold.

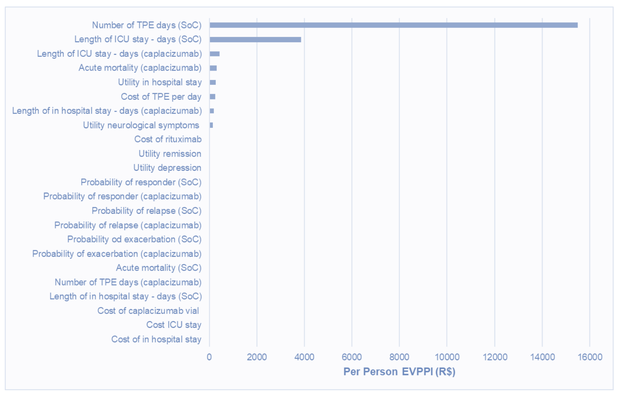

Figure 3. Per person partial expected value of perfect information (EVPPI) for Brazil

- The expected value of perfect information per year is R$ 238 million for 10 years.
- The most relevant parameters worthy of collecting further information were the number of TPE days and the length of ICU stay in the standard of care-group.

CONCLUSIONS

- Caplacizumab is the first targeted therapy for patients with aTTP, and results in faster normalization of platelet count, thereby reducing in-hospital length-of-stay.
- Based on the proposed cost, caplacizumab is not cost-effective from the SUS perspective.
- The high EVPI (R$ 237 million) indicates the return from investment in research, that is, the health gains (valued in monetary terms) and cost savings expected of enabling a reassessment of the cost-effectiveness decision of caplacizumab vs. standard of care in light of less uncertainty.
- As a result, the decision to add caplacizumab to the benefit list may depend on efforts for purchasing additional data, i.e., by developing a national registry of patients with aTTP.

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