EE265

COST-EFFECTIVENESS OF PEGCETACOPLAN FOR THE TREATMENT OF PAROXYSMAL NOCTURNAL HEMOGLOBINURIA IN PATIENTS WITH TREATMENT FAILURE AFTER C5 INHIBITOR THERAPY: AN ANALYSIS FROM THE BRAZILIAN PUBLIC HEALTHCARE SYSTEM PERSPECTIVE

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Context

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare and life-threatening clonal hematopoietic stem cell disorder characterized by complement-mediated hemolysis, cytopenias, and thrombosis. [1,2]

Despite the introduction of C5 inhibitors (eculizumab and ravulizumab), a substantial proportion of patients remain anemic and transfusion-dependent due to ongoing extravascular hemolysis. [3-6]

In Brazil, both eculizumab and ravulizumab are available in the public healthcare system (SUS), but no therapy directly targeting C3 is currently accessible. [7, 8] Pegcetacoplan, a C3 inhibitor, blocks both intra- and extravascular hemolysis, potentially offering improved hematologic outcomes and quality of life for patients who remain anemic after C5 inhibitor therapy. [9,10]

The present analysis was conducted in the Brazilian public healthcare system context, assessing whether pegcetacoplan represents a cost-effective and affordable option for patients with prior C5 inhibitor use.

Objectives

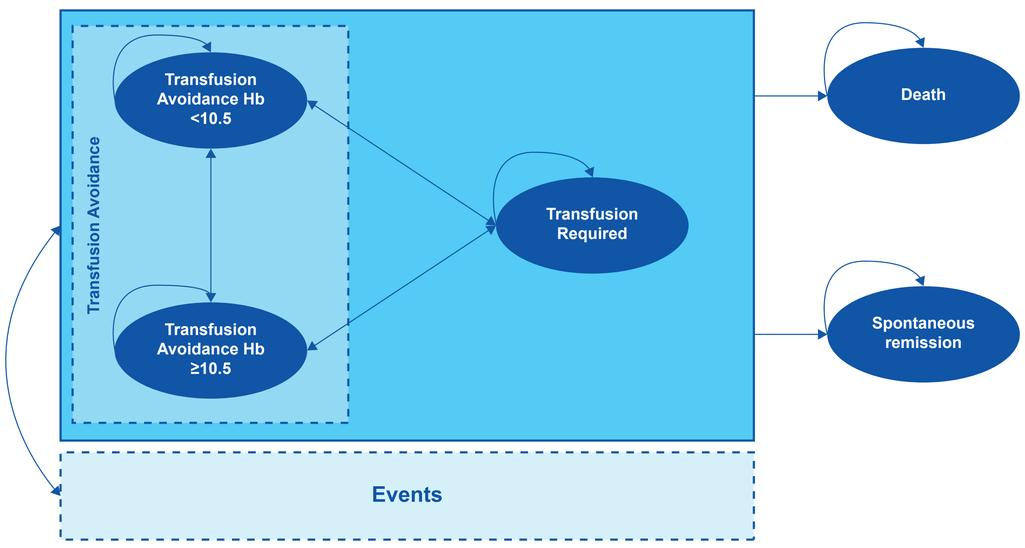
To evaluate the cost-effectiveness of pegcetacoplan compared with eculizumab and ravulizumab for the treatment of adult patients with PNH who remain anemic despite prior use of C5 inhibitors from the perspective of the Brazilian public healthcare system.

Methods

A cost-utility analysis was conducted using a Markov model with monthly cycles and a lifetime horizon to simulate the clinical and economic outcomes of pegcetacoplan compared with eculizumab and ravulizumab, the two C5 inhibitors currently available in Brazilian public healthcare system. (Figure 1) The model included five mutually exclusive health states based on hemoglobin levels and transfusion requirements: Hb ≥ 12 g/dL, Hb 10-12 g/dL, Hb <10 g/dL without transfusion, Hb 10 g/dL with transfusion, and death. Transition probabilities were derived from the PEGASUS randomized controlled trial (pegcetacoplan vs. eculizumab) [11] and a matching -adjusted indirect comparison for pegcetacoplan vs. ravulizumab. [12] Utility weights were obtained from published studies using EQ-5D and FACIT-F mapping. [13,14] Costs (in 2024 BRL) included drug acquisition, administration, monitoring, transfusions, and complication management. The analysis considered a 5% annual discount rate for both costs and outcomes.

Uncertainty was explored through probabilistic (Monte Carlo, 1,000 iterations) sensitivity analyses.

Figure 1. Markov model structure.



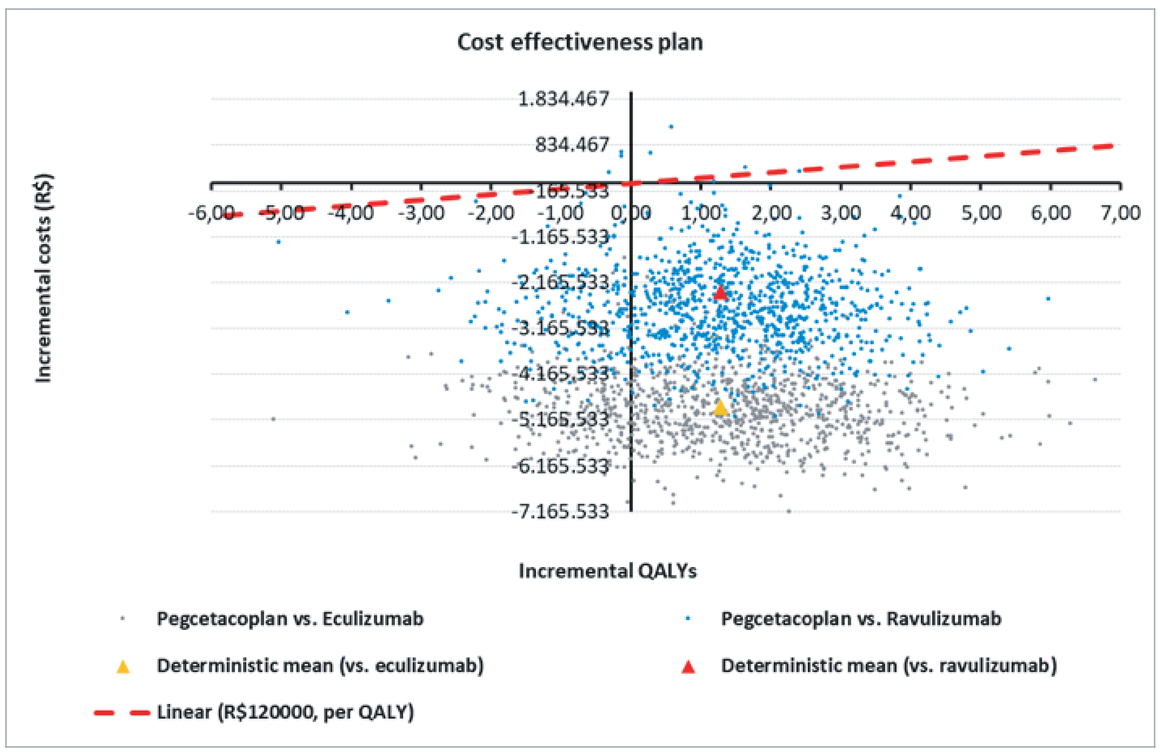
Hb = hemoglobin.

Results

Pegcetacoplan was associated with an increase of 1.04 QALYs and 0.89 life years compared with eculizumab and an increase of .01 QALYs and 0.87 life years compared with ravulizumab. Total costs for pegcetacoplan were 27.63% and 13.46% lower when compared to eculizumab and ravulizumab, respectively, over the lifetime horizon, driven primarily by reduced need for transfusions and lower overall treatment and management costs. Pegcetacoplan was therefore dominant (more effective and less costly) in both comparisons.

Probabilistic analyses confirmed these results, with >95% of simulations falling below the WTP threshold of BRL 120,000 per QALY considered in the Brazilian public healthcare system. (Figure 2)

Figure 2. Cost-effectiveness plane (probabilistic sensitivity analysis). Pegcetacoplan dominates eculizumab and ravulizumab in >95% of simulations.



Conclusion

Pegcetacoplan demonstrated superior clinical outcomes and cost savings compared with eculizumab and ravulizumab in the treatment of PNH patients with persistent anemia after C5 inhibitor therapy.

These findings indicate that pegcetacoplan is a cost-effective and potentially cost-saving therapy within the Brazilian public healthcare system, addressing an unmet medical need and supporting its incorporation as a novel complement inhibitor targeting C3.

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