

Assessment of the Budgetary Impact of Introducing Pegcetacoplan and Ravulizumab for Treating Paroxysmal Nocturnal Hemoglobinuria in the Kingdom of Saudi Arabia

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BACKGROUND

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired clonal hematopoietic cell disease characterized by the destruction of hematopoietic cells.
- It occurs by activation of the complement system and may lead to ongoing hemolysis, thrombosis, and marrow failure¹.
- Treatments for PNH patients include allogeneic hematopoietic stem cell transplant (HCT) and drugs that inhibit complement activation².
- HCT is risky and is not a therapeutic option for most patients^{3,4}.
- Thus, current therapeutic strategy includes treatment with complement-inhibitory drugs for managing disease symptoms.
- Complement-inhibitory drugs used for PNH patients include eculizumab, ravulizumab, and pegcetacoplan^{5,6,7,8}.

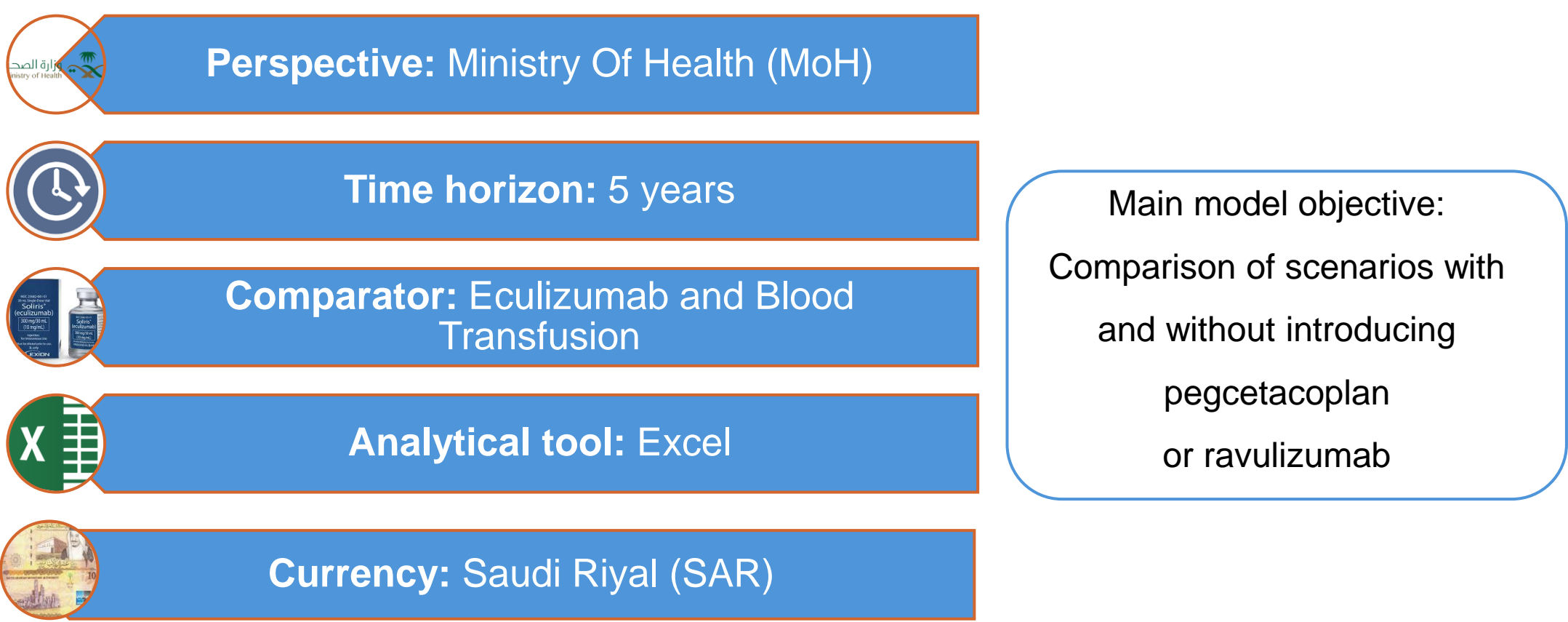
OBJECTIVES

- To assess the budget impact of introducing either ravulizumab for pediatric and adult patients with PNH or pegcetacoplan for adult patients with PNH from the perspective of Ministry of Health (MoH), Kingdom of Saudi Arabia (KSA).
- To understand the impact of introducing these drugs on direct medical costs and other costs with and without Managed Entry Agreements (MEAs).

METHODS

- A budget impact model (BIA) was developed to estimate the total cost of introducing pegcetacoplan for treating adult patients or ravulizumab for treating pediatric and adult patients with PNH (with and without managed entry agreement (MEA)) in comparison to eculizumab and blood transfusion over a 5-year period.

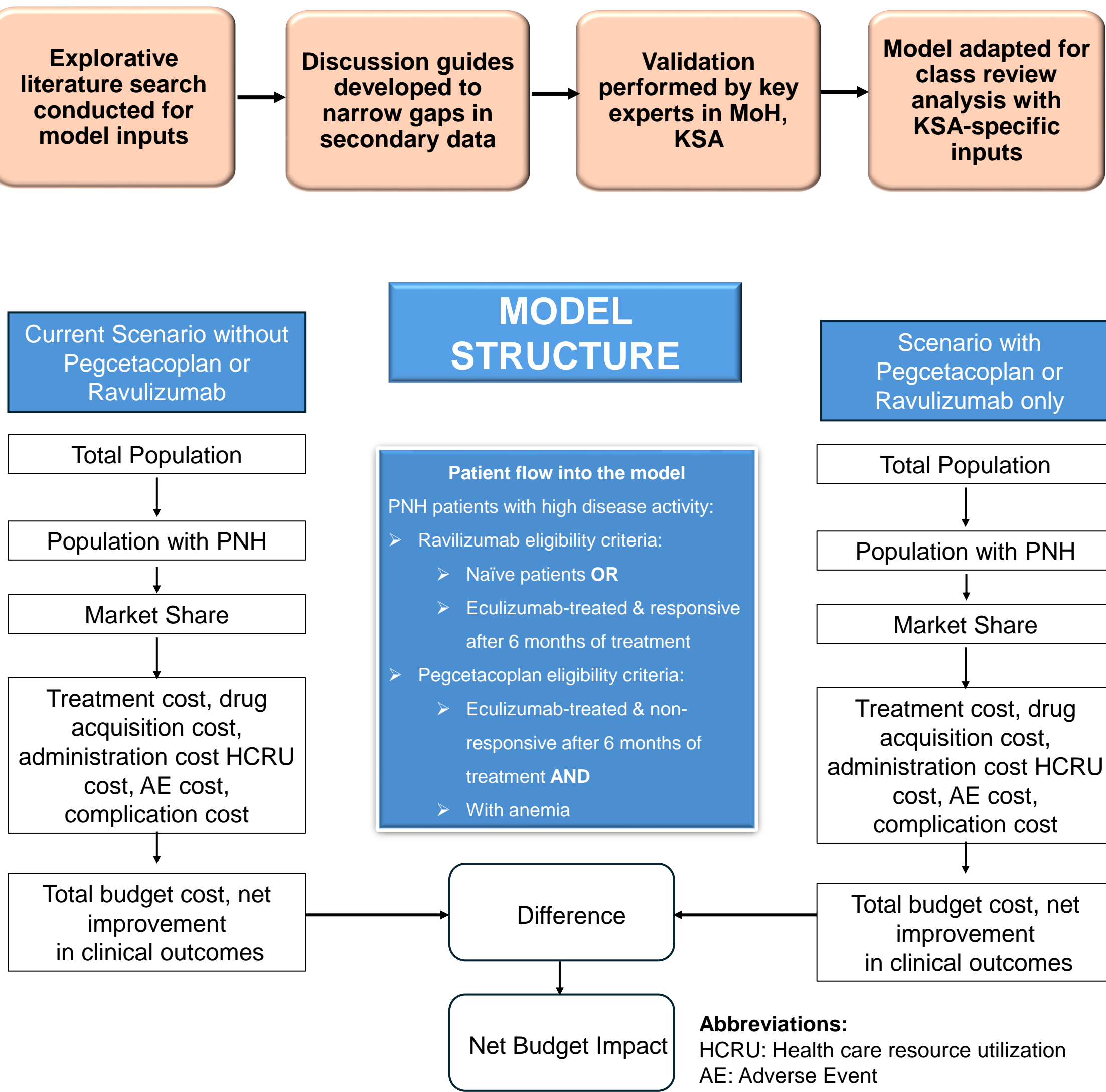
MODEL DETAILS



MODEL INPUTS

The model inputs included the following: total population, market share distribution, drug acquisition cost, drug dosing, administration cost, healthcare services and monitoring costs, adverse event costs and complication costs.

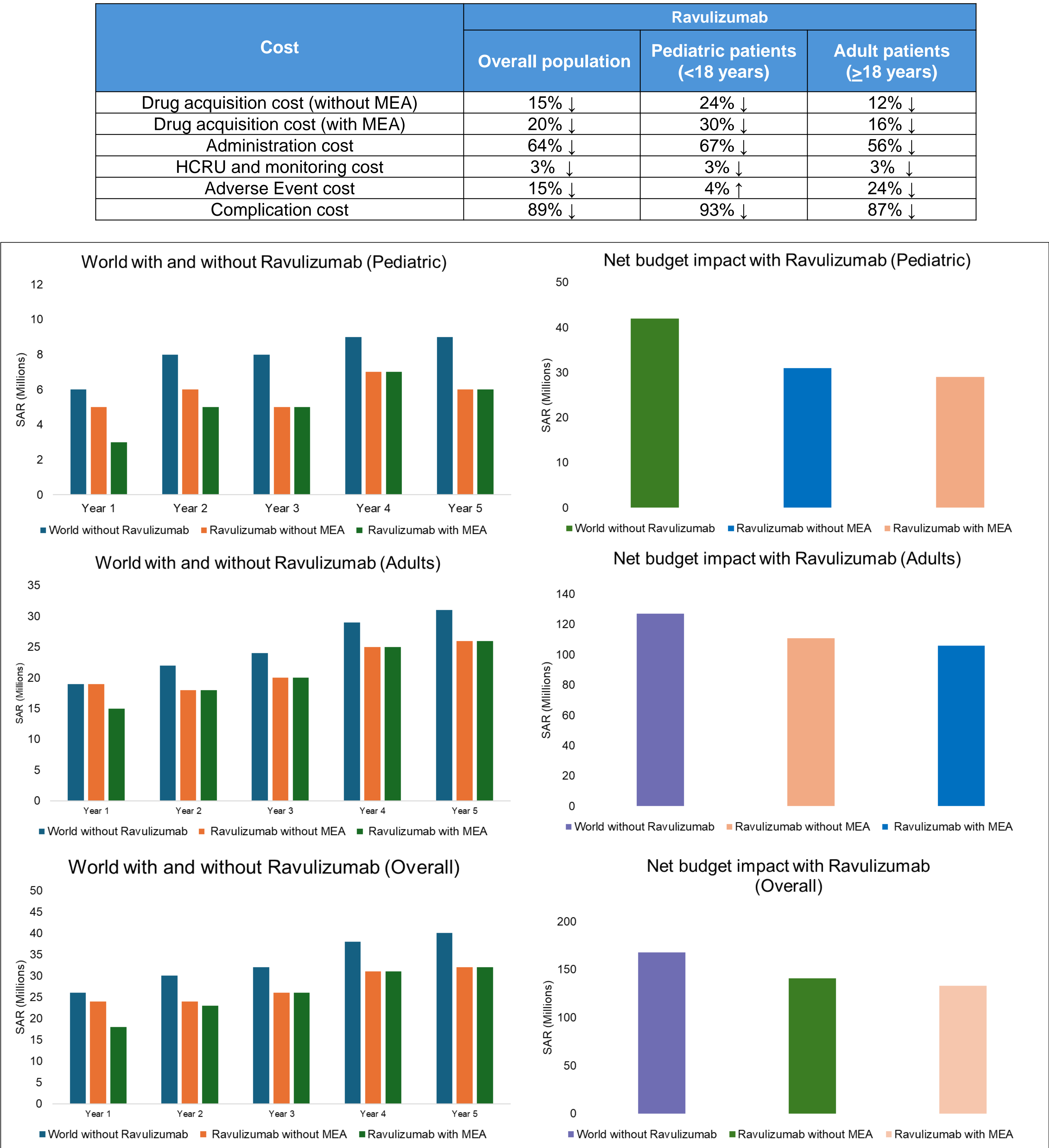
Model inputs were obtained and applied as detailed in the schematic below:



RESULTS

Ravulizumab for Pediatric and Adult PNH Patients

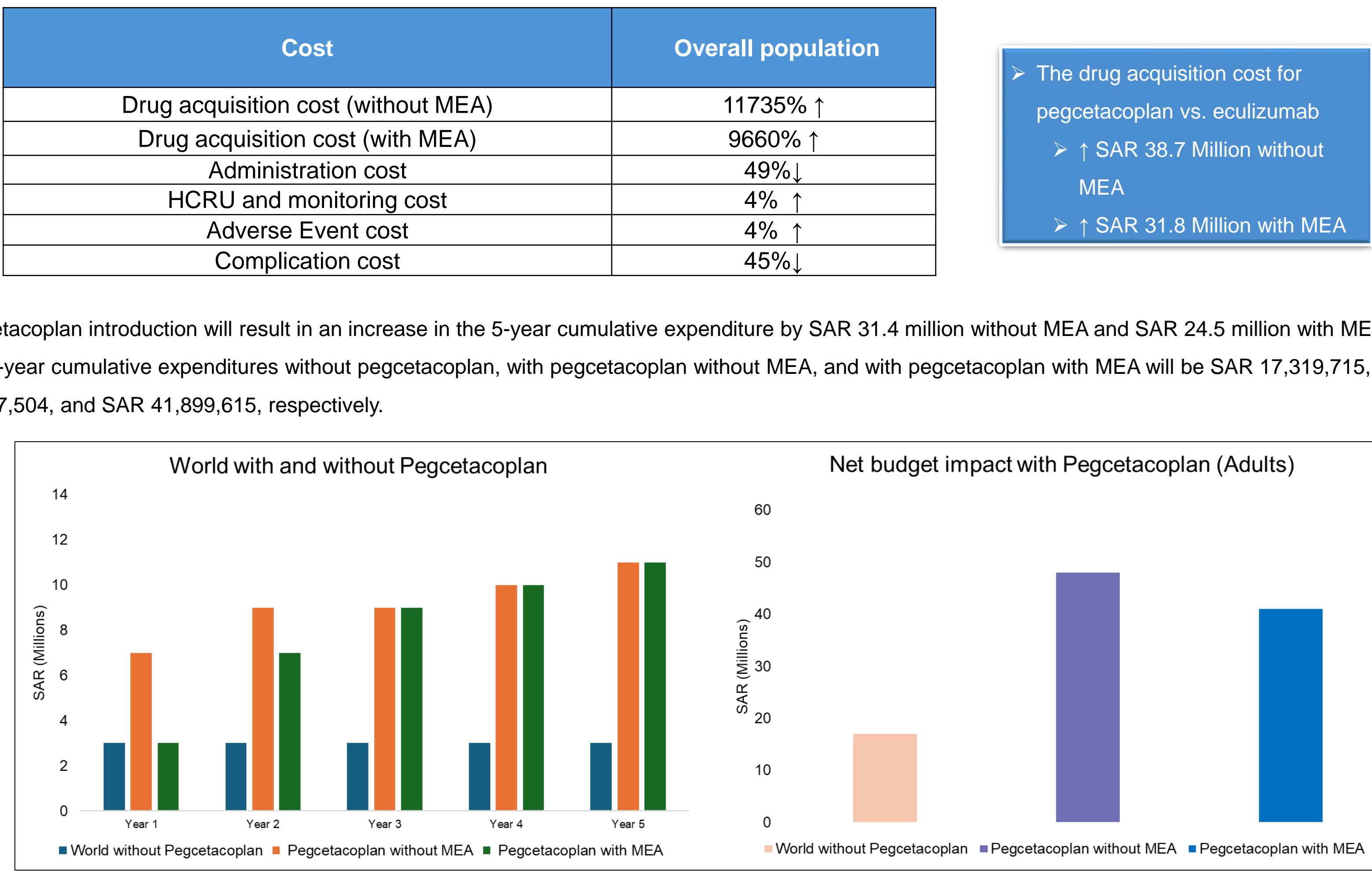
- Effect of introduction of ravulizumab on costs over 5 years compared to without ravulizumab will be as follows :



- BIA showed that introducing ravulizumab will result in a decrease in expenditure for pediatric PNH patients by 25% (SAR 10.4 million) without MEA and 30% (SAR 12.7 million) with MEA.
- In adult PNH patients, introducing ravulizumab will result in a decrease in expenditure by 13% (SAR 16.2 million) without MEA and 17% (SAR 21.3 million) with MEA.
- The 5-year cumulative expenditure will be SAR 168,398,879 without ravulizumab, SAR 141,058,184 with ravulizumab without MEA, and SAR 133,590,053 with ravulizumab with MEA.

Pegcetacoplan for Adult Patients

- Effect of introducing pegcetacoplan on costs over 5 years compared to without pegcetacoplan will be as follows :



- Pegcetacoplan introduction will result in an increase in the 5-year cumulative expenditure by SAR 31.4 million without MEA and SAR 24.5 million with MEA.
- The 5-year cumulative expenditures without pegcetacoplan, with pegcetacoplan without MEA, and with pegcetacoplan with MEA will be SAR 17,319,715, SAR 48,747,504, and SAR 41,899,615, respectively.

REFERENCES

- Cooper, J. P., et al. (2019). *Biology of Blood and Marrow Transplantation*, 25(7), 1331–1339.
- Mitchell, R., et al. (2017). *Journal of Clinical Oncology*, 1(1), 1001.
- Sahin, G., et al. (2021). *Annals of Hematology*, 100(7), 1667-1675.
- Young, N.S., et al. (2009). *Seminars in Hematology* 46(1) (Suppl 1), S1.
- Gediz, F., et al. (2017). *Hematology Reports*, 9(1), 6862.
- Panse, J. (2023). *American Journal of Hematology*, 98, S20-S32.
- Lee, J.W., et al. (2020). *Expert Opinion on Biological Therapy*, 20(3), 227-237.
- Wong, R. S. (2022) *Therapeutic Advances in Hematology*, 13, 20406207221114673.

CONCLUSION

- Introducing ravulizumab for treating PNH will result in an overall decrease in budget across all age groups, with a budgetary saving of SAR 27.3 million without MEA and SAR 34.8 million with MEA.
- Introduction of pegcetacoplan treatment will result in overall budgetary increase of SAR 31.4 million without MEA and SAR 24.5 million with MEA.
- Drug acquisition cost will be the primary determinant for the cumulative net budget impact of ravulizumab and pegcetacoplan in the KSA.