Background
Paroxysmal nocturnal hemoglobinuria (PNH) is a rare blood disorder characterized by red blood cell hemolysis, severe anemia, bone marrow failure, thrombosis, and increased risks of bacterial and fungal infections. PNH is diagnosed in approximately 0.7 people per million each year.1

Methods
Model Structure
A budget impact model was developed using a 1-year time horizon and US health plan perspective (Figure 1). This model compared treatment of patients using 20% of the market share for ravulizumab with 80% for eculizumab while preserving the 80/20 market share split for ravulizumab and eculizumab. The following outcomes were estimated annually and cumulatively over 3 years for the treatment-naive patients (Table 3).

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Objectives
To evaluate the impact of introducing pegcetacoplan to a health plan’s formulary for the treatment of PNH-ineligible adults (Figure 2).

Model Outputs
Annualized transfusion and BTH rates were derived from the phase 1b, open-label PADDOCK trial (NCT02588833) for pegcetacoplan for patients without previous C5i treatment (meaning patients who were naïve to C5i treatment). The PADDOCK trial evaluated pegcetacoplan in reverse hemolytic uremic syndrome (R-HUS) treatment-naive patients.10

Results
For a health plan of 10 million members, the total annual costs ranged from $24.5-$27.1 million each year with pegcetacoplan and $24.5-$31.4 million each year without pegcetacoplan (Figure 3).

Discussion
This analysis, which used head-to-head clinical trial data, showed that introducing pegcetacoplan has the potential to reduce US health plan costs for the treatment of adults with PNH by reducing both drug costs and associated healthcare costs (including transfusion costs) due to the lower prior and substantive administration.

In addition, pegcetacoplan is estimated to lead to a reduction in medical costs by avoiding transfusions and BTH events, which are both costly and represent a substantial burden on patients with PNH currently treated with C5i.

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