

Terms of Reference for the Review of Life Saving Drugs Program eculizumab for the Treatment of Paroxysmal Nocturnal Haemoglobinuria (PNH)

Medicine under review:

- Eculizumab (Soliris®)
1. Review the prevalence of Paroxysmal Nocturnal Haemoglobinuria (PNH) in Australia.
 2. Review evidence for the management of PNH and compare to the LSDP treatment guidelines, patient eligibility and testing requirements for the use of these medicines on the program (including the validity of the tests).
 3. Review clinical effectiveness and safety of eculizumab for the treatment of PNH, including analysis of LSDP patient data and international literature to provide evidence of life extension.
 4. Review relevant patient based outcomes that are most important or clinically relevant to patients with PNH.
 5. Assess the value for money of eculizumab under the current funding arrangements by evaluating the benefit of the drug's treatment outcomes and cost.
 6. Review the utilisation of eculizumab including storage, dispensing and evidence of patient compliance to treatment.
 7. Investigate developing technologies that may impact future funded access.