HSCB Service Notification for the managed entry of new medicines and technologies

1.	**Treatment & Condition** Ravulizumab for treating paroxysmal nocturnal haemoglobinuria (PNH)
2.	**Associated appraisal body *&* Summary of ruling** Ravulizumab (Ultomiris®) is recommended, within its marketing authorisation, as an option for treating paroxysmal nocturnal haemoglobinuria in adults: • with haemolysis with clinical symptoms suggesting high disease activity, or • whose disease is clinically stable after having eculizumab for at least 6 months, and • the company provides it according to the commercial arrangement.
3.	**Number of people in Northern Ireland expected to take up service/therapy** According to the NICE Resource Impact Template that accompanies NICE TA698, it is expected that by year 5 patient numbers will be as follows: • 10 people in the prevalent population will be on treatment each year • 1 person from the incident population will start treatment each year Hence, at steady state, 11 people will be treated with ravulizumab for PNH each year.
4.	**Patient Access Scheme Availability** (**Yes**/No) The list price of ravulizumab is £4,533.00 per 300mg/3ml concentrate for solution for infusion vial; £16,621.00 per 1,100mg/11ml concentrate for solution for infusion vial (excluding VAT). The company (Alexion Pharmaceuticals) has a commercial arrangement. This makes ravulizumab available to the NHS with a discount. The size of the discount is commercial in confidence.
5.	**Infrastructure Requirements** Any additional infrastructure costs associated with the introduction of this medicine for this indication will be dealt with as part of the routine commissioning process.
6.	**Expected implementation period** There is no impediment to implementation of this guidance.
7.	**Commissioning arrangements** This regimen will be formally commissioned by the HSCB/PHA via the Specialist Services Commissioning Team initially on a cost per case basis initially. Thereafter, numbers of patients who received or are receiving treatment will be reviewed and consideration will be given to moving to recurrent funding to support this regimen.

8.	**Monitoring arrangements** The HSC Board has robust arrangements in place for the monthly monitoring of all biologic therapies (patient numbers and waiting times), and this regime will be included within the routinely provided return. All monitoring returns for biologics are reviewed by the Specialist Services Commissioning Team monthly.
9.	**DoH (NI) Legislative/Policy Caveats** This advice does not override or replace the individual responsibility of health professionals to make appropriate decisions in the circumstances of their individual patients, in consultation with the patient and/or guardian or carer. This would, for example, include situations where individual patients have other conditions or complications that need to be taken into account in determining whether the NICE guidance is fully appropriate in their case. The Rural Needs Act NI 2016 has been considered and this guidance, which is purely of a technical nature, is not regarded as falling within the scope of the act.