Hematology, Nephrology and Transplant Research Areas of Interest

Atypical Hemolytic Uremic Syndrome (aHUS)

- Improve characterization of aHUS (including patients with different triggering conditions and extra-renal manifestations) and role of C5 inhibition
- Innovative approaches and technologies for increasing confidence in diagnosis of aHUS
- Effectiveness of ravulizumab as first-line therapy for those with aHUS with and without triggers

Paroxysmal Nocturnal Hemoglobinuria (PNH)

- Ravulizumab in PNH subpopulations not studied in the Alexion sponsored clinical programme
- Role of dual inhibition with danicopan as add-on treatment with ravulizumab/eculizumab in PNH patients in the real-world setting
- Characterization of different PNH patient populations and their therapeutic considerations (e.g. women of child-bearing potential & pregnancy, pediatric, patients with thrombotic complications without significant hemolysis, patient with concomitant BMF)
- Clinical and other disease-related outcomes for patients switching from proximal inhibitor monotherapy, biosimilars, or other C5-inhibitors to ravulizumab, with or without danicopan add-on
- Burden of disease and treatment, and associated outcomes

Out of Scope

- Clinical comparative studies with other complement inhibitors

Hematopoietic Stem Cell Transplant-associated Thrombotic Microangiopathy (HSCT-TMA)

- Role of terminal complement in the pathophysiology of HSCT-TMA
- Novel identification and diagnosis of HSCT-TMA
- Ravulizumab in HSCT-TMA in subpopulations not studied in the Alexion sponsored clinical programme

Transplant

- Patient and caregiver burden associated with delayed graft function and antibody mediated rejection
- Association of long-term clinical outcomes with acute measures of kidney function or rejection
- Preclinical or clinical research on complement therapy and the role of complement in transplant associated conditions