RATIONALE FOR INCLUSION IN PA PROGRAM

Background
Soliris is a complement inhibitor indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis, atypical hemolytic uremic syndrome (aHUS), or generalized Myasthenia Gravis (gMG). Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired genetic blood disorder characterized by hemolytic anemia, thrombosis, impaired bone marrow function and a 3% to 5% risk of developing leukemia. Atypical hemolytic uremic syndrome (aHUS) is a rare and chronic blood disease that can lead to kidney failure and is associated with increased risk of death and stroke. Soliris is a targeted therapy that works by inhibiting proteins that play a role in aHUS (1).

Regulatory Status
FDA- approved indication: Soliris is a complement inhibitor indicated for the treatment of patients with: (1)
1. Paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis
2. Atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy
3. Generalized Myasthenia Gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody positive

Limitation of Use:
Soliris is not indicated for the treatment of patients with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS) (1).

Soliris includes a boxed warning of life-threatening and fatal meningococcal infections. Additionally, all patients must be vaccinated with a meningococcal vaccine at least 2 weeks prior to receiving their first dose (1).

Soliris is not indicated for the treatment of patients with Shiga toxin E. coli- related hemolytic uremic syndrome (STEC-HUS). Alexion Pharmaceutical has developed the Soliris OneSource program to assist patients and healthcare providers with education on PNH and aHUS, and to facilitate access to Soliris (1).
The safety and effectiveness of Soliris for the treatment of PNH and gMG in pediatric patients below the age of 18 years have not been established. Four clinical studies assessing the safety and effectiveness of Soliris for the treatment of aHUS included a total of 25 pediatric patients (ages 2 months to 17 years). The safety and effectiveness of Soliris for the treatment of aHUS appear similar in pediatric and adult patients (1).

Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS prescribers must enroll in the Program (1).

Summary
Soliris is a complement inhibitor indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis and for the treatment of patients age 18 and older with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy. Soliris includes a boxed warning of life-threatening and fatal meningococcal infections. Soliris is not indicated for the treatment of patients with Shiga toxin E. coli- related hemolytic uremic syndrome (STEC-HUS). Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) (1).

Prior authorization is required to ensure the safe, clinically appropriate and cost effective use of Soliris while maintaining optimal therapeutic outcomes.

References