SOLIRIS
(eculizumab)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background
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 (1). PNH typically affects people in young adulthood with a median age of 30-40 years. Soliris is a monoclonal antibody that specifically binds to the complement protein, thereby inhibiting generation of the terminal complement complex C5b-9. This mechanism of action allows Soliris to inhibit terminal complement mediated intravascular hemolysis in PNH patients. Previous treatment for PNH was dependent on the severity of patient symptoms, and the only curative therapy is allogenic bone marrow transplantation (2-3).

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. Safety and efficacy in pediatric patients was found to be similar to adult patients for the treatment of aHUS. Soliris is a targeted therapy that works by inhibiting proteins that play a role in aHUS (4).

Regulatory Status
FDA-approved indication: 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 (4).

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 (4).

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 (4).

The safety and effectiveness of Soliris for the treatment of PNH 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 (4).

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 (4).

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) (4).
Prior authorization is required to ensure the safe, clinically appropriate and cost effective use of
Soliris while maintaining optimal therapeutic outcomes.

References
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