Soliris

Description
Soliris (eculizumab)

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).

### Related policies

**Policy**

*This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.*

Soliris may be considered medically necessary in patients 18 years of age or older for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) and in pediatric and adult patients for the treatment of atypical hemolytic uremic syndrome (aHUS); who do not have Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS). Vaccination against Neisseria meningitidis at least 2 weeks prior to initiation of first dose is required for both diagnoses. Prescribers must be enrolled in the Soliris REMS program.

Soliris is considered investigational for all indications other than PNH and aHUS for patients 18 years of age or older, and for all indications other than aHUS for patients less than 18 years of age.

### Prior-Approval Requirements

**Diagnoses**
The patient must have ONE of the following:

1. Paroxysmal nocturnal hemoglobinuria (PHN)
   a. Age of 18 years of age or older

2. Atypical hemolytic uremic syndrome (aHUS)

AND ALL of the following:

a. Vaccination against Neisseria meningitides at least 2 weeks prior to initiation
b. Does NOT have Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS)
c. Prescribing physician is enrolled in Soliris REMS program

Prior – Approval Renewal Requirements
Same as above

Policy Guidelines
Pre – PA Allowance
None

Prior - Approval Limits
Duration 12 months

Prior – Approval Renewal Limits
Duration 12 months

Rationale
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