I. Medication Description

Soliris (eculizumab) is an intravenous monoclonal antibody indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis by complement inhibition. Patients with PNH have a genetic mutation that leads to development of abnormal red blood cells deficient in terminal complement inhibitors. Eculizumab binds specifically with complement proteins and prevents the generation of the terminal complement complex, consequently inhibiting complement mediated intravascular hemolysis and a reduction in the need for red blood cell transfusions.

II. Position Statement

Coverage is determined through a prior authorization process with supporting clinical documentation for every request.

III. Policy

Coverage of Soliris is provided when the following criteria are met:

- Patient is under the care of a hematologist, nephrologist and/or other appropriate specialists AND
- Patient has a confirmed diagnosis of one of the following:
  - Paroxysmal nocturnal hemoglobinuria (PNH) where diagnosis is accompanied by detection of PNH clones by flow cytometry diagnostic test OR
  - Atypical hemolytic uremic syndrome (aHUS)

IV. Quantity Limitations

Available as 300 mg single-use vials each containing 30 mL of 10 mg/mL sterile, preservative-free solution

- PNH:
  - Maximum per infusion: 900mg (90 billable units or 3 vials)
  - Maximum covered units:
    - Initial/Loading Doses: 3300mg (330 billable units or 11 vials) over 5 weeks
    - Maintenance Dose: 1800 mg (180 billable units or 6 vials) per each 28 days
- aHUS:
  - Maximum per infusion: 1200mg (120 billable units or 4 vials)
  - Maximum covered units:
    - Initial/Loading Doses: 4800mg (480 billable units or 16 vials) over 5 weeks
    - Maintenance Dose: 2400mg (240 billable units or 8 vials) per each 28 days
V. Coverage Duration

Initial coverage is provided for 6 months and may be renewed.

VI. Coverage Renewal Criteria

Coverage can be renewed in 12 month intervals based upon the following criteria:

- Absence of unacceptable toxicity from the drug AND
- Patient displays improvement in disease symptoms or stabilization of disease state, such as:
  - A higher rate of stabilization of hemoglobin levels in the absence of transfusion, fewer packed red blood cell transfusions, less intravascular hemolysis, and improvement in quality of life for PNH OR
  - Effects on thrombotic microangiopathy and renal function for aHUS

VII. Billing/Coding Information

- J1300: 1 unit = 10mg
- Available in 300mg single-use vials containing 30ml of 10mg/ml Soliris solution
- Pertinent diagnoses: D59.3, D59.5

VIII. Summary of Policy Changes

- 6/15/12: addition of criteria for aHUS
- 3/15/13: removal of documentation of meningococcal vaccination requirement for approval for aHUS and PNH
- 3/15/14: maximum dosing clarified per diagnosis; REMS registration requirement removed from section III
- 3/15/15: no policy changes
- 6/15/15: no policy changes
- 7/1/15: formulary distinctions made
- 6/15/16: no policy changes
- 4/5/17: no policy changes

IX. References


6. Effect of eculizumab on hemolysis and transfusion requirements in patients with paroxysmal nocturnal hemoglobinuria. Hillmen P; Hall C; Marsh JC; Elebute M; Bombara MP; Petro BE; Cullen MJ; Richards SJ; Rollins SA; Mojcik CF; Rother RP. N Engl J Med 2004 Feb 5;350(6):552-9.

7. The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria. Hillmen P; Young NS; Schubert J; Brodsky RA; Socie G; Muus P; Roth A; Szer J; Elebute MO; Nakamura R; Browne P; Risitano AM; Hill A; Schrezenmeier H; Fu CL; Maciejewski J; Rollins SA; Mojcik CF; Rother RP; Luzzatto L. N Engl J Med. 2006 Sep 21;355(12):1233-43.

8. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria. Brodsky RA; Young NS; Antonioli E; Risitano AM; Schrezenmeier H; Schubert J; Gaya A; Coyle L; de Castro C; Fu CL; Maciejewski JP; Bessler M; Kroon HA; Rother RP; Hillmen P. Blood. 2008 Feb 15;111(4):1840-7. Epub 2007 Nov 30.

The Plan fully expects that only appropriate and medically necessary services will be rendered. The Plan reserves the right to conduct pre-payment and post-payment reviews to assess the medical appropriateness of the above-referenced therapies.

Drug therapy initiated with samples will not be considered as meeting medical necessity for coverage for non-preferred or prior authorized medications.

The preceding policy is a guideline to allow for coverage of the pertinent medication/product, and is not meant to serve as a clinical practice guideline.