

SPECIALTY GUIDELINE MANAGEMENT

SOLIRIS (eculizumab) SGM

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

- A. Paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis
- B. Atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy
- C. Generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody positive
- D. Neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive

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

All other indications are considered experimental/investigational and are not covered benefits.

II. REQUIRED DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review for new requests for treatment of:

- A. Atypical hemolytic uremic syndrome: ADAMTS 13 level
- B. Paroxysmal nocturnal hemoglobinuria: flow cytometry used to show results of GPI-APs deficiency
- C. Generalized myasthenia gravis: anti-acetylcholine receptor (AChR) antibody positive, clinical classification of myasthenia gravis score, MG activities of daily living score, use of IVIG and rituximab, use of two immunosuppressive therapies
- D. Neuromyelitis optica spectrum disorder: immunoassay used to confirm anti-aquaporin-4 (AQP4) antibody is present

III. CRITERIA FOR INITIAL APPROVAL

A. Atypical hemolytic uremic syndrome

Authorization of 6 months may be granted for treatment of atypical hemolytic uremic syndrome not caused by Shiga toxin when all of the following criteria are met:

1. ADAMTS 13 activity level above 5%
2. Absence of Shiga toxin

B. Paroxysmal nocturnal hemoglobinuria

Authorization of 6 months may be granted for treatment of paroxysmal nocturnal hemoglobinuria (PNH) when all of the following criteria are met:

Reference number
2373-A

1. The diagnosis of PNH was confirmed by detecting a deficiency of glycosylphosphatidylinositol-anchored proteins (GPI-APs) as demonstrated by either of the following:
 - a. At least 5% PNH cells
 - b. At least 51% of GPI-anchored protein deficient poly-morphonuclear cells
2. Flow cytometry is used to demonstrate GPI-anchored proteins deficiency

C. Generalized myasthenia gravis (gMG)

Authorization of 6 months may be granted for treatment of generalized myasthenia gravis (gMG) when all of the following criteria are met:

1. Anti-acetylcholine receptor (AChR) antibody positive
2. Myasthenia Gravis Foundation of America (MGFA) clinical classification II to IV
3. MG activities of daily living (MG-ADL) total score ≥ 6
4. Meets both of the following:
 - a. Patient has had an inadequate response to at least two immunosuppressive therapies listed below:
 - i. azathioprine
 - ii. cyclosporine
 - iii. mycophenolate mofetil
 - iv. tacrolimus
 - v. methotrexate
 - vi. cyclophosphamide
 - b. Patient has inadequate response to chronic IVIG AND rituximab

D. Neuromyelitis Optica Spectrum Disorder (NMOSD)

Authorization of 6 months may be granted for treatment of neuromyelitis optica spectrum disorder (NMOSD) when all of the following criteria are met:

1. Anti-aquaporin-4 (AQP4) antibody positive
2. Member exhibits one of the following core clinical characteristics of NMOSD:
 - a. Optic neuritis
 - b. Acute myelitis
 - c. Area postrema syndrome (episode of otherwise unexplained hiccups or nausea and vomiting)
 - d. Acute brainstem syndrome
 - e. Symptomatic narcolepsy or acute diencephalic clinical syndrome with NMOSD-typical diencephalic MRI lesions
 - f. Symptomatic cerebral syndrome with NMOSD-typical brain lesions
3. The member will not be treated with rituximab and eculizumab concomitantly

IV. CONTINUATION OF THERAPY

A. Atypical hemolytic uremic syndrome

Authorization of 12 months may be granted to all members requesting continuation of therapy provided they meet all initial authorization criteria and demonstrate a positive response to therapy (e.g., normalization of lactate dehydrogenase (LDH) levels, platelet counts).

B. Paroxysmal nocturnal hemoglobinuria

Authorization of 12 months may be granted to all members requesting continuation of therapy provided they meet all initial authorization criteria and demonstrate a positive response to therapy (e.g., improvement in hemoglobin levels, normalization of LDH levels).

C. Generalized myasthenia gravis (gMG)

Authorization of 12 months may be granted to all members requesting continuation of therapy provided they meet all initial authorization criteria and demonstrate a positive response to therapy

(e.g., improvement in MG-ADL score, changes compared to baseline in Quantitative Myasthenia Gravis (QMG) total score).

D. Neuromyelitis optica spectrum disorder (NMOSD)

Authorization of 12 months may be granted to all members requesting continuation of therapy provided they meet all initial authorization criteria and demonstrate a positive response to therapy (e.g., reduction in number of relapses).

IV. REFERENCES

1. Soliris [package insert]. New Haven, CT: Alexion Pharmaceuticals, Inc.; June 2019.
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9. Borowitz MJ, Craig F, DiGiuseppe JA, et al. Guidelines for the Diagnosis and Monitoring of Paroxysmal Nocturnal Hemoglobinuria and Related Disorders by Flow Cytometry. *Cytometry B Clin Cytom*. 2010; 78: 211-230.
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12. Pittock SJ, Berthele A, Kim HJ, et al. Eculizumab in Aquaporin-4-Positive Neuromyelitis Optica Spectrum Disorder. *N Engl J Med*. 2019 May 3. doi: 10.1056/NEJMoA1900866.
13. Wingerchuk DM, Banwell B, Bennett JL, et al. International consensus diagnostic criteria for neuromyelitis optica spectrum disorders. *Neurology*. 2015; 85:177-189.