| Reference number |
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| 3537-A |

# 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 not medically necessary.

### II. DOCUMENTATION

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

A. For initial requests:
    1. Atypical hemolytic uremic syndrome: ADAMTS 13 level
    2. Paroxysmal nocturnal hemoglobinuria: flow cytometry used to show results of glycosylphosphatidylinositol-anchored proteins (GPI-APs) deficiency
    3. Generalized myasthenia gravis: anti-acetylcholine receptor (AchR) antibody positive, clinical classification of myasthenia gravis score, MG activities of daily living score, use of IVIG, use of two immunosuppressive therapies
    4. Neuromyelitis optica spectrum disorder: immunoassay used to confirm anti-aquaporin-4 (AQP4) antibody is present
B. For continuation requests: Chart notes or medical record documentation supporting positive clinical response.

### 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:

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-AP deficient poly-morphonuclear cells
2. Flow cytometry is used to demonstrate GPI-APs 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. Member 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
        vii. rituximab
    b. Member has inadequate response to chronic IVIG

**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 receive the requested drug concomitantly with other biologics for the treatment of NMOSD.

## IV. CONTINUATION OF THERAPY

**A. Atypical hemolytic uremic syndrome**

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization when there is no evidence of unacceptable toxicity or disease progression while on the current regimen 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 for continued treatment in members requesting reauthorization when there is no evidence of unacceptable toxicity or disease progression while on the current regimen and demonstrate a positive response to therapy (e.g., improvement in hemoglobin levels, normalization of lactate dehydrogenase [LDH] levels).

**C. Generalized myasthenia gravis (gMG)**
Authorization of 12 months may be granted for continued treatment in members requesting reauthorization when there is no evidence of unacceptable toxicity or disease progression while on the current regimen 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 for continued treatment in members requesting reauthorization when all of the following criteria are met:
1. There is no evidence of unacceptable toxicity or disease progression while on the current regimen.
2. The member demonstrates a positive response to therapy (e.g., reduction in number of relapses).
3. The member will not receive the requested drug concomitantly with other biologics for the treatment of NMOSD.

## V. DOSAGE AND ADMINISTRATION

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

## VI. REFERENCES

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13. Wingerchuk DM, Banwell B, Bennett JL, et al. International consensus diagnostic criteria for neuromyelitis optica spectrum disorders. *Neurology.* 2015; 85:177-189.
14. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. Hematology Am Soc Hematol Educ Program. 2016;2016(1):208-216.