# PHARMACY POLICY STATEMENT
## North Carolina Marketplace

| DRUG NAME | Soliris (eculizumab) |
|---|---|
| BENEFIT TYPE | Medical |
| STATUS | Prior Authorization Required |

Soliris is a C5 Complement inhibitor initially approved by the FDA in 2007. It is approved for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis, atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy, generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AchR) antibody positive, and neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive.
PNH is a rare hematopoietic stem cell disorder in which activation of the complement system destroys red blood cells.
aHUS is a type of thrombotic microangiopathy (TMA), a group of syndromes defined by the presence of hemolytic anemia, low platelets and organ damage due to microscopic blood clots in the capillaries. Unlike typical HUS, aHUS is usually genetic. The three main signs of aHUS are hemolytic anemia, thrombocytopenia, and acute kidney failure. Of note, the other type of TMA is called thrombotic thrombocytopenic purpura (TTP); Soliris is not used to treat TTP.

Soliris (eculizumab) will be considered for coverage when the following criteria are met:

## Paroxysmal Nocturnal Hemoglobinuria (PNH)

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication is prescribed by or in consultation with a hematologist; AND
3. Member has a diagnosis of PNH as confirmed by flow cytometry; AND
4. Member has a lactate dehydrogenase (LDH) level >1.5x upper limit of normal (ULN); AND
5. Member has at least one PNH-related sign/symptom e.g., fatigue, hemoglobin <10 g/dL, thrombosis, pRBC transfusion, shortness of breath; AND
6. Member has tried and failed or is unable to try Ultomiris; AND
7. Member has received meningococcal vaccine.
8. **Dosage allowed/Quantity limit:** 600mg IV weekly x 4 weeks, then 900mg 1 week later, then 900mg every 2 weeks thereafter.

***If all the above requirements are met, the medication will be approved for 6 months.***

For **reauthorization**:
1. Clinical evidence of positive response to therapy such as increased hemoglobin level, decreased need for transfusions, normalized LDH levels, improved fatigue.

***If all the above requirements are met, the medication will be approved for an additional 12 months.***

# Atypical Hemolytic Uremic Syndrome (aHUS)

For **initial** authorization:

1. Medication is prescribed by or in consultation with a hematologist or nephrologist; AND
2. Member has a diagnosis of aHUS supported by ALL of the following:
   a) Thrombocytopenia (platelet count < 150 x $10^9$/L),
   b) Evidence of microangiopathic hemolytic anemia (MAHA) e.g., hemoglobin < 10 g/dL, elevated lactate dehydrogenase (LDH), low haptoglobin, presence of fragmented red blood cells or schistocytes on blood smear
   c) Evidence of renal impairment (e.g., raised SCr or low eGFR); AND
3. Shiga toxin-producing E. coli related HUS (STEC-HUS) has been ruled out; AND
4. ADAMTS13 activity level is > 10% (to rule out TTP); AND
5. Member has tried and failed or is unable to try Ultomiris; AND
6. Member has received meningococcal vaccine.
7. **Dosage allowed/Quantity limit:**
   Pediatrics: See weight-based dosing in package insert.
   Adults: 900mg IV weekly x 4 weeks, then 1200mg 1 week later, then 1200mg every 2 weeks thereafter.

***If all the above requirements are met***

## Neuromyelitis Optica Spectrum Disorder (NMOSD)

For **initial** authorization:

1. Member is at least 18 years of age; AND
2.

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| | NMOSD: Added references. Removed requirement for trial of Enspryng.<br>MG: Added reference. Removed 'MGFA class II-IV'. Added MGFA appendix. Added trial of Ultomiris. Shortened and simplified list of conventional therapy trials. |

APPENDIX:

References:


1. 2021 Georgia Code Title 33 – Insurance Chapter 20A - Managed Health Care Plans Article 2 - Patient's Right to Independent Review § 33-20A-31 Definitions. Justia US Law. Accessed April 25, 2023.