Mass General Brigham
Health Plan

Soliris (eculizumab)
Effective 01/01/2024

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☒ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Medical and Specialty Medications**		
	All Plans	Phone: 877-519-1908	Fax: 855-540-3693
	Non-Specialty Medications		
	All Plans	Phone: 800-711-4555	Fax: 844-403-1029
Exceptions	N/A		

Overview

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.

Coverage Guidelines

Authorization may be granted for members new to the plan who are currently receiving treatment with the requested medication, excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

OR

Authorization may be granted when the following diagnosis-specific criteria is met:

A. Atypical hemolytic uremic syndrome

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

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

B. Paroxysmal nocturnal hemoglobinuria

Authorization 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 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 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 confirmed by use of immunoassay
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.

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

Continuation of Therapy

Authorization may be granted for continued treatment when the following diagnosis-specific criteria is met:

A. Atypical hemolytic uremic syndrome

Authorization 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 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 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 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.

Limitations

1. Initial approvals will be granted for 6 months.
2. Reauthorizations will be granted for 12 months.

References

1. Soliris [package insert]. New Haven, CT: Alexion Pharmaceuticals, Inc.; November 2020.
2. Loirat C, Fakhouri F, Ariceta G, et al. An international consensus approach to the management of atypical hemolytic uremic syndrome in children. *Pediatr Nephrol*. Published online: April 11, 2015.
3. Parker CJ. Management of paroxysmal nocturnal hemoglobinuria in the era of complement inhibitory therapy. *Hematology.* 2011; 21-29.

4. Sanders D, Wolfe G, Benatar M et al. International consensus guidance for management of myasthenia gravis. *Neurology*. 2021; 96 (3) 114-122 .
5. Jaretzki A, Barohn RJ, Ernstoff RM et al. Myasthenia Gravis: Recommendations for Clinical Research Standards. *Ann Thorac Surg*. 2000;70: 327-34.
6. Hillmen P, Young NS, Schubert J, et al. The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria. *NEJM.* 2006;335:1233-43.
7. Howard JF, Utsugisawa K, Benatar M. Safety and efficacy of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalized myasthenia gravis (REGAIN); a phase 3, randomized, double-blind, placebo-controlled, multicenter study. *Lancet Neurol*. 2017 Oct 20. http://dx.doi.org/10.1016/S1474-4422(17)30369-1Ingenix HCPCS Level II, Expert 2011.
8. Brodsky RA, Young NS, Antonioli E, et al. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria. *Blood*. 2008;111(4):1840-1847.
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.
10. Preis M, Lowrey CH. Laboratory tests for paroxysmal nocturnal hemoglobinuria (PNH). Am J Hematol. 2014;89(3):339-341.
11. Lee JW, Sicre de Fontbrune F, Wong LL, et al. Ravulizumab (ALXN1210) vs eculizumab in adult patients with PNH naive to complement inhibitors: The 301 study. Blood. 2018 Dec 3; pii: blood-2018-09-876136.
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.
14. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. Hematology Am Soc Hematol Educ Program. 2016;2016(1):208-216.

Review History

12/13/2023: Reviewed at Dec P&T, switched from SGM to Custom. Effective 1/1/2024