

POLICY Document for Ultomiris

The overall objective of this policy is to support the appropriate and cost effective use of the medication, lower cost site of care and overall clinically appropriate use. This document provides specific information to each section of the overall policy.

Section 1: Site of Care

- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

Section 2: Clinical Criteria

- Policy information specific to the clinical appropriateness for the medication

Section 1: Site of Care

CareFirst Site of Care Criteria Administration of Ultomiris

POLICY

I. CRITERIA FOR APPROVAL FOR ADMINISTRATION IN OUTPATIENT HOSPITAL SETTING

This policy provides coverage for administration of Ultomiris in an outpatient hospital setting for up to 45 days when a member is new to therapy or is reinitiating therapy after not being on therapy for at least 6 months.

This policy provides coverage for administration of Ultomiris in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

- A. The member has experienced an adverse reaction to the drug that did not respond to conventional interventions (eg, acetaminophen, steroids, diphenhydramine, fluids, other pre-medications or slowing of infusion rate) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after an infusion.
- B. The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
- C. The member has severe venous access issues that require the use of special interventions only available in the outpatient hospital setting.
- D. The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver.
- E. The member is less than 21 years of age or is 65 years of age or older..

For situations where administration of Ultomiris does not meet the criteria for outpatient hospital infusion, coverage for Ultomiris is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the site of care prior authorization review (where applicable):

- A. Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
- B. Medical records supporting the member is medically unstable
- C. Medical records supporting the member has severe venous access issues that requires specialized interventions only available in the outpatient hospital setting
- D. Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver

Section 2: Clinical Criteria

SPECIALTY GUIDELINE MANAGEMENT

ULTOMIRIS (ravulizumab)

POLICY

I. INDICATIONS

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

FDA-Approved Indication

1. Ultomiris is indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria.
2. Ultomiris is indicated for the treatment of patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy (TMA).

Limitations of Use: Ultomiris 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. REQUIRED DOCUMENTATION

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

- A. Paroxysmal nocturnal hemoglobinuria: flow cytometry used to show results of GPI-APs deficiency
- B. Atypical hemolytic uremic syndrome: ADAMTS 13 level

III. CRITERIA FOR INITIAL APPROVAL

A. 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:
 1. At least 5% PNH cells
 2. At least 51% of GPI deficient poly-morphonuclear cells
2. Flow cytometry is used to demonstrate GPI-APs deficiency

B. Atypical hemolytic uremic syndrome (aHUS)

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

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

IV. CONTINUATION OF THERAPY

A. 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 lactate dehydrogenase [LDH] levels).

B. Atypical hemolytic uremic syndrome (aHUS)

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).

REFERENCES:

SECTION 1

1. Ultomiris [package insert]. Boston, MA: Alexion Pharmaceuticals, Inc.; October 2019.

SECTION 2

2. Ultomiris [package insert]. Boston, MA: Alexion Pharmaceuticals, Inc.; December 2018.
3. Parker CJ. Management of paroxysmal nocturnal hemoglobinuria in the era of complement inhibitory therapy. *Hematology*. 2011; 21-29.
4. 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.
5. 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.
6. Preis M, Lowrey CH. Laboratory tests for paroxysmal nocturnal hemoglobinuria (PNH). *Am J Hematol*. 2014;89(3):339-341.
7. 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.