Mylotarg

Description

Mylotarg (gemtuzumab ozogamicin)

Background
Mylotarg is a CD33-directed antibody-drug conjugate. The conjugate binds to CD33-expressing tumor cells and induces cell cycle arrest and apoptopic cell death. Mylotarg is indicated for the treatment of CD33-positive acute myeloid leukemia (AML) which is a rapidly progressing cancer that forms in the bone marrow and results in an increased number of abnormal white blood cells in the bloodstream and bone marrow (1).

Regulatory Status
FDA-approved indication: Mylotarg is a CD33-directed antibody-drug conjugate indicated for:

1. Treatment of newly-diagnosed CD33-positive acute myeloid leukemia (AML) in adults
2. Treatment of relapsed or refractory CD33-positive AML in adults and in pediatric patients 2 years and older

Mylotarg has a boxed warning for hepatotoxicity, including life-threatening and sometimes fatal hepatic VOD events, which have been reported in patients receiving Mylotarg as a single agent or as part of a combination chemotherapy regimen. It is recommended to assess ALT, AST, total bilirubin, and alkaline phosphatase prior to each dose of Mylotarg. Also physicians should monitor for signs and symptoms of VOD; these may include elevations in ALT, AST, total bilirubin, hepatomegaly, rapid weight gain, and ascites (1).
Safety and efficacy in pediatric patients with newly-diagnosed de novo AML below the age of 18 have not been established. The safety and efficacy of Mylotarg as a single agent in pediatric patients with relapsed or refractory AML has been established in patient 2 years of age and older (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Mylotarg may be considered medically necessary in patients 18 years or older with newly diagnosed acute myeloid leukemia (AML), and in patients 2 years or older with relapsed or refractory AML, and if the conditions indicated below are met.

Mylotarg is considered investigational for all other indications and ages.

Prior-Approval Requirements

Diagnoses

Age  2 years of age or older

The patient must have the following:

Relapsed or refractory CD33-positive acute myeloid leukemia (AML)

Age  18 years of age or older

The patient must have the following:

Newly-diagnosed CD33-positive acute myeloid leukemia (AML)

AND ALL of the following:

1. CD33-positive AML as detected by FDA approved test
2. Prescriber agrees to monitor ALT, AST, total bilirubin, and alkaline phosphatase prior to each dose of Mylotarg
Prior – Approval Renewal Requirements

Age   2 years of age or older

Diagnosis

The patient must have the following:

CD33-positive acute myeloid leukemia (AML)

AND ALL of the following:
   a. NO disease progression or unacceptable toxicities
   b. Prescriber agrees to monitor ALT, AST, total bilirubin, and alkaline phosphatase prior to each dose of Mylotarg

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Duration 12 months

Prior – Approval Renewal Limits

Duration 12 months

Rationale

Summary

Mylotarg is indicated for the treatment of acute myeloid leukemia (AML) which is a rapidly progressing cancer that forms in the bone marrow and results in an increased number of abnormal white blood cells in the bloodstream and bone marrow. Safety and efficacy in pediatric patients with newly-diagnosed de novo AML below the age of 18 have not been established. The safety and efficacy of Mylotarg as a single agent in pediatric patients with relapsed or refractory AML has been established in patient 2 years of age and older (1).
Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Mylotarg while maintaining optimal therapeutic outcomes.

References