Rydapt

Description

Rydapt (midostaurin)

Background
Rydapt is an oral cancer agent that inhibits multiple receptor tyrosine kinases. Rydapt is indicated for the treatment of acute myeloid leukemia (AML), an aggressive cancer of the blood and bone, and advanced systemic mastocytosis. Some patients with AML have a gene mutation in the FLT3 cell-surface receptor which can result in faster disease progression, higher relapse rate, and lower survival rates than other forms of AML. Rydapt works by blocking the FLT3 receptor signaling and cell proliferation and inducing apoptosis of certain leukemic cells (1).

Regulatory Status
FDA-approved indication: Rydapt is a kinase inhibitor indicated for the treatment of adult patients with:

1. Newly diagnosed acute myeloid leukemia (AML) that is FLT3 mutation-positive as detected by an FDA-approved test, in combination with standard cytarabine and daunorubicin induction and cytarabine consolidation (1).
2. Aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated hematological neoplasm (SM-AHN), or mast cell leukemia (MCL) (1).

Limitations of Use: (1)

1. Rydapt is not indicated as a single-agent induction therapy for the treatment of patients with AML.
Rydapt may cause fetal harm when administered to a pregnant woman. Verify the pregnancy status of females of reproductive potential within 7 days prior to initiating therapy. Advise females and males with female partners to use effective contraception during treatment with Rydapt and for 4 months after the last dose (1).

Cases of interstitial lung disease and pneumonitis, some fatal, have occurred in patients taking Rydapt. Discontinue in patients with signs or symptoms of pulmonary toxicity (1).

Safety and efficacy in pediatric patients below the age of 18 have not been established (1).

Related policies
Xospata

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

Rydapt may be considered medically necessary in patients 18 years or older with newly diagnosed acute myeloid leukemia (AML), aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated hematological neoplasm (SM-AHN), or mast cell leukemia (MCL) and if the conditions indicated below are met.

Rydapt is considered investigational in patients below 18 years of age and for all other indications.

Prior-Approval Requirements

Age
18 years of age and older

Diagnoses
The patient must have ONE of the following:

1. Newly diagnosed acute myeloid leukemia (AML)
   a. FLT3 mutation-positive AML detected by FDA-approved test
   b. Concurrent standard induction therapy with cytarabine and daunorubicin and cytarabine consolidation
2. Aggressive systemic mastocytosis (ASM)
3. Systemic mastocytosis with associated hematological neoplasm (SM-AHN)
4. Mast cell leukemia (MCL)

Prior – Approval Renewal Requirements

Age
18 years of age and older

Diagnoses

The patient must have ONE of the following:

1. Acute myeloid leukemia (AML)
2. Aggressive systemic mastocytosis (ASM)
3. Systemic mastocytosis with associated hematological neoplasm (SM-AHN)
4. Mast cell leukemia (MCL)

AND ALL of the following:
   a. NO disease progression or unacceptable toxicity

Policy Guidelines

Pre - PA Allowance
None

Prior - Approval Limits

Quantity
25 mg
672 capsules per 84 days

Duration
12 months

Prior – Approval Renewal Limits
Same as above

Rationale

Summary
Rydapt, a multikinase inhibitor, is indicated for the treatment of FLT3 mutation-positive acute myeloid leukemia and advanced systemic mastocytosis. Patients with FLT3 mutation-positive AML often have worse outcomes compared to patients with other types of AML. Rydapt works by blocking FLT3 receptor signaling and cell proliferation to slow the progression of disease (1). Prior authorization is required to ensure the safe, clinically appropriate and cost effective use of Rydapt while maintaining optimal therapeutic outcomes.

References

Policy History

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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 15, 2019 and is effective on April 1, 2019.