BlueCross BlueShield Federal Employee Program

RYDAPT (midostaurin)

RATIONALE FOR INCLUSION IN PA PROGRAM

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 indications: 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 women. 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).

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

1. Rydapt [package insert]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; May 2023.
2. NCCN Drugs & Biologics Compendium® Midostaurin 2025. National Comprehensive Cancer Network, Inc. Accessed on January 9, 2025.