Reference number
3491-A

SPECIALTY GUIDELINE MANAGEMENT

AYVAKIT (avapritinib)

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.

A. FDA-Approved Indications
   1. Gastrointestinal Stromal Tumor (GIST)
      Ayvakit is indicated for the treatment of adults with unresectable or metastatic GIST harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations.
   2. Advanced Systemic Mastocytosis (AdvSM)
      Ayvakit is indicated for the treatment of adult patients with advanced systemic mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).
      Limitations of Use: Ayvakit is not recommended for the treatment of patients with AdvSM with platelet counts of less than 50 X 10^9/L.

B. Compendial Uses
   1. Myeloid/lymphoid neoplasms with eosinophilia and FIP1L1-PDGFRA rearrangement
   2. Unresectable, recurrent, or metastatic GIST after failure on approved therapies

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. For GIST: PDGFRA exon 18 mutation testing (e.g., polymerase chain reaction [PCR]-based assay, next-generation sequencing [NGS]-based assay) results (where applicable).
B. For myeloid and/or lymphoid neoplasms with eosinophilia: Testing or analysis confirming FIP1L1-PDGFRA rearrangement and PDGFRA D842V mutation

III. CRITERIA FOR INITIAL APPROVAL

A. Gastrointestinal Stromal Tumor (GIST)
Authorization of 12 months may be granted for treatment of gastrointestinal stromal tumor (GIST) when either of the following criteria are met:
   1. The disease harbors a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations.
   2. The disease is unresectable, recurrent, or metastatic and member has failed at least four FDA-approved therapies (e.g., imatinib, sunitinib, regorafenib, and ripretinib).

B. Myeloid/Lymphoid Neoplasms with Eosinophilia
Authorization of 12 months may be granted for treatment of myeloid and/or lymphoid neoplasms with eosinophilia when all of the following criteria are met:
1. The disease harbors a PDGFRA D842V mutation which is resistant to imatinib.
2. The disease is FIP1L1-PDGFRA rearrangement-positive.

C. Advanced Systemic Mastocytosis (AdvSM), including Aggressive Systemic Mastocytosis (ASM), Systemic Mastocytosis with an Associated Hematological Neoplasm (SM-AHN) and Mast Cell Leukemia (MCL)
Authorization of 12 months may be granted for treatment of AdvSM, ASM, SM-AHN and MCL when the member's platelet count is greater than or equal to 50 X 10^9/L.

IV. CONTINUATION OF THERAPY

A. GIST
Authorization of 12 months may be granted for continued treatment of GIST when the member is receiving clinical benefit and there is no evidence of generalized (widespread, systemic) disease progression or unacceptable toxicity while on the current regimen.

B. Myeloid/Lymphoid Neoplasms with Eosinophilia and AdvSM including ASM, SM-AHN, MCL
Authorization of 12 months 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.

V. REFERENCES

1. Ayvakit [package insert]. Cambridge, MA: Blueprint Medicines Corporation; June 2021.
2. The NCCN Drugs & Biologics Compendium® © 2020 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed November 2, 2020.
3. NCCN Clinical Practice Guidelines in Oncology® Gastrointestinal Stromal Tumors (GIST) (Version 1.2021). © 2020 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed November 2, 2020.