Reference number(s)
1793-A

Specialty Guideline Management nilotinib products

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over the counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Tasigna	nilotinib hydrochloride
Danziten	nilotinib tartrate
Nilceya	nilotinib d-tartrate

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 Indications[1-4]

- Danziten, Nilceya, and Tasigna are indicated for adult patients with newly diagnosed Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) in chronic phase
- Tasigna is indicated for pediatric patients greater than or equal to 1 year of age with newly diagnosed Ph+ CML in chronic phase
- Danziten, Nilceya, and Tasigna are indicated for adult patients with chronic phase and accelerated phase Ph+ CML resistant or intolerant to prior therapy that included imatinib
- Tasigna is indicated for pediatric patients greater than or equal to 1 year of age with chronic phase and accelerated phase Ph+ CML with resistance or intolerance to prior tyrosine-kinase inhibitor (TKI) therapy.

Compendial Uses[5-9]

- Primary treatment of advanced phase CML (accelerated phase or blast phase)
- Additional therapy for CML patients after hematopoietic stem cell transplant (HSCT)
- Ph+ acute lymphoblastic leukemia or lymphoblastic lymphoma (Ph+ ALL/LL)
- Maintenance therapy for Ph+ ALL/LL patients after HSCT
- Gastrointestinal stromal tumor (GIST)
- Myeloid/lymphoid neoplasms with eosinophilia and ABL1 rearrangement in chronic or blast phase
- Pigmented Villonodular Synovitis/Tenosynovial Giant Cell Tumor (PVNS/TGCT)
- Cutaneous Melanoma

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

Documentation

The following information is necessary to initiate the prior authorization review:

- For treatment of CML or Ph+ ALL/LL: results of cytogenetic and/or molecular testing for detection of the Ph chromosome or the BCR::ABL gene
- For members requesting initiation of therapy with the requested medication for treatment of CML or ALL/LL after experiencing resistance to prior tyrosine kinase inhibitor (TKI) therapy: results of BCR::ABL1 mutation testing for T315I, Y253H, E255K/V, F359V/C/I, and G250E mutations, where applicable
- For members requesting initiation of therapy with the requested medication for treatment of myeloid and/or lymphoid neoplasms with eosinophilia: results of testing or analysis confirming ABL1 rearrangement
- For treatment of melanoma: results of molecular testing or analysis confirming c-KIT activating mutations

Coverage Criteria

Chronic Myeloid Leukemia (CML)[1-6]

Authorization of 7 months may be granted for treatment of CML that has been confirmed by detection of the Ph chromosome or BCR::ABL gene by cytogenetic and/or molecular testing when any of the following criteria are met:

- Member has not received prior therapy with a TKI (e.g., asciminib, bosutinib, dasatinib, imatinib, ponatinib)
- Member experienced toxicity or intolerance to prior therapy with a TKI
- Member experienced resistance to prior therapy with a TKI and results of BCR::ABL1 mutational testing are negative for all of the following: T315I, Y253H, E255K/V, F359V/C/I
- Member has received HSCT for CML and results of BCR::ABL1 mutational testing are negative for all of the following: T315I, Y253H, E255K/V, F359V/C/I

Ph+ Acute Lymphoblastic Leukemia (ALL)/Lymphoblastic Lymphoma (LL)[5,7,8]

Authorization of 12 months may be granted for treatment of Ph+ ALL/LL that has been confirmed by detection of the Ph chromosome or BCR::ABL gene by cytogenetic and/or molecular testing when any of the following criteria are met:

- Member has not received prior therapy with a TKI (e.g., bosutinib, dasatinib, imatinib, ponatinib)
- Member experienced toxicity or intolerance to prior therapy with a TKI
- Member experienced resistance to prior therapy with a TKI and results of BCR::ABL1 mutational testing are negative for all of the following: T315I, Y253H, E255K/V, F359V/C/I, and G250E
- Member has received HSCT for Ph+ ALL/LL and results of BCR::ABL1 mutational testing are negative for all of the following: T315I, Y253H, E255K/V, F359V/C/I, and G250E

Gastrointestinal Stromal Tumor (GIST)[5,9]

Authorization of 12 months may be granted for treatment of GIST as a single agent for residual, unresectable, tumor rupture, recurrent/progressive, or metastatic disease and the member has failed at least four FDA-approved therapies (e.g., imatinib, sunitinib, regorafenib, ripretinib).

Myeloid/Lymphoid Neoplasms with Eosinophilia[5]

Authorization of 12 months may be granted for treatment of myeloid and/or lymphoid neoplasms with eosinophilia and ABL1 rearrangement in the chronic phase or blast phase.

Pigmented Villonodular Synovitis/Tenosynovial Giant Cell Tumor (PVNS/TGCT)[5]

Authorization of 12 months may be granted for the treatment of pigmented villonodular synovitis (PVNS) or tenosynovial giant cell tumor (TGCT) as a single agent.

Cutaneous Melanoma[5]

Authorization of 12 months may be granted for treatment of cutaneous melanoma when all of the following criteria are met:

- The disease is metastatic or unresectable
- The tumor has c-KIT activating mutations
- The requested medication will be used as subsequent therapy
- Member has had disease progression, intolerance, or risk of progression with BRAF-targeted therapy
- The requested medication will be used as a single agent

Continuation of Therapy

CML

Authorization may be granted for continued treatment of CML that has been confirmed by detection of Ph chromosome or BCR::ABL gene by cytogenetic and/ or molecular testing when either of the following criteria is met:

- Authorization of 12 months may be granted when any of the following criteria is met:
    - BCR::ABL1 is less than or equal to 10% and there is no evidence of disease progression or unacceptable toxicity while on the current regimen for members who have been receiving treatment with the requested medication for 6 months or greater
    - Member has received HSCT and there is no evidence of unacceptable toxicity or disease progression while on the current regimen
- Authorization of up to 7 months may be granted when the member has completed less than 6 months of therapy with the requested medication.

Ph+ ALL/LL

Authorization of 12 months may be granted for continued treatment of ALL/LL when there is no evidence of unacceptable toxicity or disease progression while on the current regimen and either of the following criteria is met:

- Member has Ph+ ALL/LL that has been confirmed by detection of Ph chromosome or BCR::ABL gene by cytogenetic and/ or molecular testing
- Member has received HSCT for ALL/LL

Myeloid/Lymphoid Neoplasms with Eosinophilia, PVNS/TGCT, or Cutaneous Melanoma

Authorization of 12 months may be granted for continued treatment of myeloid/lymphoid neoplasms with eosinophilia, PVNS/TGCT, or cutaneous melanoma when there is no evidence of unacceptable toxicity or disease progression while on the current regimen.

GIST

Authorization of 12 months may be granted for continued treatment of GIST when there is no evidence of unacceptable toxicity while on the current regimen.

References

1. Tasigna [package insert]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; February 2024.
2. Danziten [package insert]. Woburn, MA: Azurity Pharmaceuticals, Inc.; November 2024.
3. Nilceya [package insert]. Warren, NJ: Cipla USA, Inc.; July 2025.
4. nilotinib [package insert]. Weston, FL: Apotex Corp.; March 2024
5. The NCCN Drugs & Biologics Compendium® © 2025 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed March 26, 2025.
6. NCCN Clinical Practice Guidelines in Oncology® Chronic Myeloid Leukemia (Version 3.2025). © 2025 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed March 26, 2025.
7. NCCN Clinical Practice Guidelines in Oncology® Acute Lymphoblastic Leukemia (Version 3.2024). © 2025 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed March 26, 2025.
8. Micromedex Solutions [database online]. Ann Arbor, MI: Truven Health Analytics, Inc. Updated periodically. Available at: https://www.micromedexsolutions.com [available with subscription]. Accessed March 26, 2025.
9. NCCN Clinical Practice Guidelines in Oncology® Gastrointestinal Stromal Tumors (Version 1.2025). © 2025 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed March 26, 2025.