CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Koselugo (selumetinib)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Koselugo, approved by the FDA in 2020, is a kinase inhibitor indicated for the treatment of pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable plexiform neurofibromas (PN). It works by targeting mitogen-activated protein kinase kinases 1 and 2 (MEK1/2). MEK is a component of a pathway that is often activated in certain types of cancer.
NF1 is a rare, progressive genetic condition caused by a mutation in the *NF1* gene. PN's are histologically benign peripheral-nerve sheath tumors (PNST) that occur in up to 50% of NF1 patients. For PN's that cannot be completely removed by surgery, systemic therapy may be appropriate. PN's do have a risk of malignant transformation.
Koselugo was approved based on data from the phase 2 SPRINT clinical trial in which a majority of children had durable tumor shrinkage and clinical benefit such as pain reduction with treatment.

Koselugo (selumetinib) will be considered for coverage when the following criteria are met:

Neurofibromatosis Type 1 (NF1)

For **initial** authorization:

1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with a pediatric oncologist, neurologist, or geneticist; AND
3. Member has a confirmed diagnosis of neurofibromatosis type 1 (NF1) with at least 1 of the following:
   a) Positive genetic test for NF1
   b) 6 or more café-au-lait macules (CALMs)
   c) Axillary or inguinal freckling
   d) Optic glioma
   e) 2 or more Lisch nodules
   f) A distinctive osseous lesion
   g) First degree relative with NF1; AND
4. Member has at least one measurable plexiform neurofibromas (PN) as evidenced by MRI or PET-CT scan; AND
5. The plexiform neurofibromas (PN) is inoperable and cannot be removed completely by surgery without risk for substantial morbidity due to encasement of, or close proximity to, vital structures, invasiveness, or high vascularity of the PN; AND
6. Member has significant morbidity related to the PN (e.g., disfigurement, motor dysfunction, pain, airway dysfunction, visual impairment, bladder/bowel dysfunction).
7. **Dosage allowed/Quantity limit**: 25 mg/m^2 by mouth twice daily until disease progression or unacceptable toxicity. Capsules must be swallowed whole.
   (See Table 1 in prescribing information for recommended dosage based on body surface area).

QL for 10 mg capsules: 224 per 28 days (8/day)
QL for 25 mg capsules: 112 per 28 days (4/day)

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes have been provided showing that the member has had at least a partial response (defined as ≥20% reduction in the PN volume) from baseline and no disease progression and/or
2. Clinical improvement such as reduction of tumor pain or increased physical functioning.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Koselugo (selumetinib) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/14/2020	New policy for Koselugo created.
09/19/2023	Updated template. Revised references. Added neurology, genetics as specialists. Removed upper age limit. Added criteria to define NF1 diagnosis (only need to fulfill one since PN would count as the second). Added clinical benefit to reauth.

References:

1. Koselugo [Package Insert]. Wilmington, DE: AstraZeneca Pharmaceuticals LP; 2021.
2. Gross AM, Wolters PL, Dombi E, et al. Selumetinib in Children with Inoperable Plexiform Neurofibromas [published correction appears in N Engl J Med. 2020 Sep 24;383(13):1290]. *N Engl J Med*. 2020;382(15):1430-1442. doi:10.1056/NEJMoa1912735.
3. Miller DT, Freedenberg D, Schorry E, et al. Health Supervision for Children With Neurofibromatosis Type 1. *Pediatrics*. 2019;143(5):e20190660. doi:10.1542/peds.2019-0660.
4. Pellerino A, Verdijk RM, Nichelli L, Andratschke NH, Idbaih A, Goldbrunner R. Diagnosis and Treatment of Peripheral and Cranial Nerve Tumors with Expert Recommendations: An EUropean Network for RAre CANcers (EURACAN) Initiative. *Cancers (Basel)*. 2023;15(7):1930. Published 2023 Mar 23. doi:10.3390/cancers15071930
5. Armstrong AE, Belzberg AJ, Crawford JR, Hirbe AC, Wang ZJ. Treatment decisions and the use of MEK inhibitors for children with neurofibromatosis type 1-related plexiform neurofibromas. *BMC Cancer*. 2023;23(1):553. Published 2023 Jun 16. doi:10.1186/s12885-023-10996-y
6. Legius E, Messiaen L, Wolkenstein P, et al. Revised diagnostic criteria for neurofibromatosis type 1 and Legius syndrome: an international consensus recommendation. *Genet Med*. 2021;23(8):1506-1513. doi:10.1038/s41436-021-01170-5

Effective date: 04/01/2024
Revised date: 09/19/2023