SmartPA Criteria Proposal

Drug/Drug Class:	Koselugo Clinical Edit
First Implementation Date:	November 19, 2020
Proposed Date:	June 17, 2021
Prepared for:	MO HealthNet
Prepared by:	MO HealthNet/Conduent
Criteria Status:	☒Existing Criteria ☐Revision of Existing Criteria ☐New Criteria

Executive Summary

Purpose: Ensure appropriate utilization and control of Koselugo™ (selumetinib)

Why Issue Selected: Koselugo™ (selumetinib), FDA approved on April 10, 2020, is the first treatment approved for pediatric patients aged 2 years and older with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable plexiform neurofibromas (PN). NF1 is a rare, multisystem disorder, primarily involving the skin and peripheral nervous system, caused by a pathogenic variant in the *NF1* gene. Approximately 10,000 to 20,000 children in the United States are estimated to have NF1 or 1 in 2,600-3,000 infants. PN are benign tumors that grow along the length of a nerve and typically change the color and/or texture of overlying skin. The tumors can be found in any part of the body and are often difficult to remove due to their intertwining with normal tissues and peripheral nerves. PN are present in approximately 50% of patients with NF1; however, less than 20% will require any intervention for PN during childhood. Koselugo is an inhibitor of mitogen-activated protein kinase 1 and 2 (MEK1/2). MEK1/2 proteins are upstream regulators of the extracellular signal-related kinase (ERK) pathway. MEK and ERK are critical components of the RAS-regulated RAF-MEK-ERK pathway, which is often activated in different types of cancer. Koselugo is dosed per body surface area and may cause serious side effects including cardiomyopathy, ocular toxicity, gastrointestinal toxicity, skin toxicity and increases in creatinine phosphokinase. Due to the high cost, possible adverse events, and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of Koselugo.

Program-Specific Information:

Date Range FFS 4-1-2020 to 3-31-2021			
Drug	**Claims**	**Spend**	**Avg Spend per Claim**
KOSELUGO 10 MG CAPSULE	23	$188,139.89	$8,179.99
KOSELUGO 25 MG CAPSULE	15	$155,086.77	$10,339.11

Type of Criteria:
- ☒ **Increased risk of ADE**
- ☒ **Appropriate Indications**
- ☐ **Preferred Drug List**
- ☒ **Clinical Edit**

Data Sources:
- ☐ **Only Administrative Databases**
- ☒ **Databases + Prescriber-Supplied**

Setting & Population

- Drug class for review: Koselugo™ (selumetinib)
- Age range: All appropriate MO HealthNet participants aged 2 years or older

Approval Criteria

Initial Therapy:

- Participant aged 2 years or older **AND**
- Documented diagnosis of neurofibromatosis type 1 **AND**
- Documentation of inoperable plexiform neurofibromas (PN) defined as a PN that cannot be completely removed without risk for substantial morbidity due to encasement of or close proximity to vital structures, invasiveness, or high vascularity of the PN **AND**
- Prescribed by or in consultation with an oncologist, neurologist, or other specialist familiar with the treated disease state **AND**
- Documentation of baseline left ventricular ejection fraction (LVEF) **AND**
- Documentation of baseline ophthalmic assessment **AND**
- Documentation of baseline CPK level **AND**
- Participant is not currently pregnant **AND**
- Participant (male or female of appropriate age) is utilizing concurrent birth control methods

Continuation of Therapy:

- Initial approval is for 1 year, renewal of prior authorization may be given following documentation of the following:
  - Ophthalmic examinations for ocular toxicities at least once annually **AND**
  - LVEF assessed every 3 months during the first year of treatment and then every 6 months thereafter **AND**
  - Recent CPK level **AND**
  - Documentation of benefit of therapy, examples include:
    - stabilization or reduction in PN size or number of PN
    - improved quality of life

Denial Criteria

- Therapy will be denied if all approval criteria are not met
- Documented severe hepatic impairment (Child-Pugh Class C)

Required Documentation

Laboratory Results:	X	Progress Notes:	X
MedWatch Form:		Other:	X

Disposition of Edit

Denial: Exception code “0682” (Clinical Edit)
Rule Type: CE

Default Approval Period

1 year

References

- Koselugo™ (selumetinib) [package insert]. Wilmington, DE: AstraZeneca Pharmaceuticals LP; May 2020.
- NIH: U.S. National Library of Medicine. “AZD6244 Hydrogen Sulfate for Children with Nervous System Tumors.” https://clinicaltrials.gov/ct2/show/NCT01362803?term=NCT01362803&draw=2&rank=1. Accessed May 6, 2021.
- IPD Analytics: New Drug Review: Koselugo (selumetinib). April 2020.
- American Academy of Pediatrics. “Health Supervision for Children with Neurofibromatosis Type 1.” https://pediatrics.aappublications.org/content/pediatrics/143/5/e20190660.full.pdf. Accessed May 6, 2021.