SmartPA Criteria Proposal

Drug/Drug Class:	Joenja Clinical Edit
First Implementation Date:	TBD
Proposed Date:	July 18, 2023
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 Joenja® (leniolisib).

Why Issue Selected: On March 24, 2023, the U.S. Food and Drug Administration (FDA) approved Joenja® (leniolisib), the first treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older. APDS was first described in literature in 2013 and affects fewer than 500 patients in the United States.

APDS is a primary immunodeficiency that results from pathogenic variants in genes that encode PI3Kδ. Disease severity in APSD may range from severe infections and lymphoproliferation at an early age to an asymptomatic adult patient. APDS is most often identified in early childhood with early symptoms consisting of frequent ear and sinus infections. Patients may also present with symptoms of mild developmental delay, bronchitis, immune cytopenias, splenomegaly, and lymphadenopathy. APDS should be suspected in patients with a history of recurrent respiratory infections, lymphoproliferation, and raised immunoglobulin M (IgM) levels. Because the symptoms of APDS may be associated with a variety of other disorders, patients are frequently misdiagnosed, with a median diagnostic delay of 7 years.

Joenja is an immune modulator that targets the root cause of APDS by modifying the overactive PI3Kδ pathway to reduce lymphoproliferation and improve cellular immunodysregulation. Prior to the approval of Joenja, treatment of APDS consisted of only supportive care and management of symptoms.

Due to the high cost and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of Joenja.

Program-Specific Information:

Drug	Cost per tablet (WAC)	Cost per month (WAC)	Cost per year (WAC)
JOENJA 70 MG TABLET	$750	$45,000	$547,500

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: Joenja® (leniolisib).
- Age range: All appropriate MO HealthNet participants aged 12 years and older

Approval Criteria

- Prescribed by or in consultation with a specialist in the treated disease state **AND**
- Participant aged ≥ 12 years **AND**
- Documented diagnosis of APDS **AND**
- Documentation of genetic testing results confirming APDS diagnosis **AND**
- Documentation of clinical findings consistent with APDS (e.g., lymphoproliferation, history of repeated oto-sino-pulmonary infections, organ dysfunction) **AND**
- Documentation of baseline liver function tests
- Initial approval is for 3 months

Continuation of Therapy:

- Documentation of clinical benefit of therapy (e.g., reduced signs and symptoms of disease state from baseline)
- Continuation of approval is for 1 year

Denial Criteria

- Therapy will be denied if all approval criteria are not met
- Concurrent use of immunosuppressant therapy (e.g., rituximab, sirolimus)
- Participant is currently pregnant or lactating
- Participant has documented moderate to severe hepatic impairment

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

3 months

References

- Joenja (leniolisib) tablets [package insert]. Warren, NJ: Pharming Healthcare Inc.; March 2023.
- Coulter TI, et al. The treatment of activated PI3Kδ syndrome. Front Immunol. 2018; 9:2043. Frontiers | The Treatment of Activated PI3Kδ Syndrome (frontiersin.org)