MEDICAL MUTUAL®

Drug Policy

Policy: SD	Joenja (leniolisib)	Annual Review Date: 05/16/2024 Last Revised Date: 05/16/2024

OVERVIEW

Joenja, a kinase inhibitor, is indicated for the treatment of **activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)** in adults and pediatric patients ≥12 years of age.

POLICY STATEMENT

This policy involves the use of Joenja. Prior authorization is recommended for pharmacy benefit coverage of Joenja. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Joenja as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Joenja be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Joenja is recommended in those who meet the following criteria:

1. <u>Activated Phosphoinositide 3-kinase Delta Syndrome (APDS), initial therapy</u>
Criteria. *Patient must meet the following criteria*

- **A.** Patient is ≥ 12 years of age; AND
- **B.** Patient weighs ≥ 45 kg; AND
- **C.** Patient has a genetic phosphoinositide 3-kinase delta (PI3Kδ) mutation with a variant in PIK3CD and/or PIK3R1 genes; AND
- **D.** Patient has at least one clinical finding or manifestation consistent with APDS; AND
  <u>Note</u>: Examples of clinical findings or manifestations of APDS include recurrent sinopulmonary infections, recurrent herpesvirus infections, lymphadenopathy, hepatomegaly, splenomegaly, nodular lymphoid hyperplasia, autoimmunity, cytopenias, enteropathy, bronchiectasis, and organ dysfunction.
- **E.** The medication is prescribed by or in consultation with an immunologist, pulmonologist, gastroenterologist, hematologist, or an infectious diseases physician who treats patients with primary immune deficiencies.

2. Activated Phosphoinositide 3-kinase Delta Syndrome (APDS), continuation of therapy
Criteria. *Patient must meet the following criteria*

A. Patient has been established on therapy for at least 6 months; AND
Note: A patient who has received < 6 months of therapy or who is restarting therapy should be considered under criterion 1 (Initial Therapy).
B. Patient is ≥ 12 years of age; AND
C. Patient weighs ≥ 45 kg; AND
D. Patient has a genetic phosphoinositide 3-kinase delta (PI3Kδ) mutation with a variant in PIK3CD and/or PIK3R1 genes; AND
E. Patient has had a positive clinical response in the signs and manifestations of APDS.
Note: Examples of positive clinical response in the signs and manifestations of APDS include reduction of: lymph node size, spleen size, immunoglobulin replacement therapy use, infection rate, or immunoglobulin M (IgM) levels.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 6 months
B) *Extended Approval:* 1 year

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Joenja has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational, or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Joenja® tablets [prescribing information]. Warren. NJ: Pharming; March 2023.
2. Rao V, Webster S, Sediva A, et al. A randomized, placebo-controlled phase 3 trial of the PI3Kδ inhibitor leniolisib for activated PI3Kδ syndrome. *Blood.* 2023;141(9):971-983.
3. Data on File. Leniolisib Pre-approved Product Dossier. Based on AMCP guidelines for formulary submission. Pharming; received March 23, 2023.
4. Rao VK, et al. Interim safety and efficacy analysis of an ongoing long-term open-label extension study of leniolisib for patients with activated PI3K delta syndrome (APDS). Presented at: European Society for Immunodeficiencies (ESID) 20th Biennial Meeting; Gothenburg, Sweden; October 12-15, 2022.