Clinical Policy: Leniolisib (Joenja)

Reference Number: PA.CP.PHAR.597
Effective Date: 05/2023
Last Review Date: 04/2023

Description
Leniolisib (Joenja®) is a kinase inhibitor.

FDA Approved Indication(s)
Joenja is indicated for the treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Joenja is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Activated Phosphoinositide 3-Kinase δ Syndrome (must meet all)**:**

1. Diagnosis of APDS;
2. Prescribed by or in consultation with an immunologist;
3. Age ≥ 12 years;
4. Documentation that member weights ≥ 45 kg at time of request;
5. Confirmed PI3Kδ genetic mutation of either the PIK3CD (APDS1) or PIK3R1 (APDS2) gene;
6. Documentation of at least one measurable nodal lesion on a computed tomography (CT) or magnetic resonance imaging (MRI) scan;
7. Evidence of clinical findings and manifestations compatible with APDS/PASLI (e.g., history of repeated oto-sino-pulmonary infections and/ or organ dysfunctions) (*see Appendix D)*;
8. Dose does not exceed both of the following (a and b):
    a. 140 mg per day;
    b. 2 tablets per day.

Approval duration: 3 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Activated Phosphoinositide 3-Kinase δ Syndrome (must meet all)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;

2. Documentation that member weights ≥ 45 kg at time of request;
3. Member is responding positively to therapy as evidenced by reduction in size of nodal lesions from baseline prior to initiating Joenja;
4. If request is for a dose increase, new dose does not exceed both of the following (a and b):
    a. 140 mg per day;
    b. 2 tablets per day.

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

APDS: activated phosphoinositide 3-kinase delta syndrome
CT: computed tomography
FDA: Food and Drug Administration
MRI: magnetic resonance imaging
PASLI: p110δ-activating mutation causing senescent T cells, lymphadenopathy and immunodeficiency
PI3Kδ: phosphoinositide 3-kinase delta

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

None reported

Appendix D: General Information

- Clinical findings and manifestations compatible with APDS/PASLI
    - Organ dysfunctions such as significant nonmalignant lymphoproliferation including bronchial and intestinal lymphoid hyperplasia and lymphadenopathy/splenomegaly/hepatomegaly
    - History of repeated oto-sino-pulmonary infections include bronchiectasis, upper respiratory tract infections, otitis media, sinusitis, pneumonia, bronchitis, chronic Epstein-Barr virus (EBV) and cytomegalovirus (CMV) viremia, and an increased risk of autoimmune disease including cytopenias

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
APDS	70 mg PO BID	140 mg/day

VI. Product Availability

Tablet: 70 mg

VII. References

1. Joenjia Prescribing Information. Saint Quentin Fallavier, France: Pharming Technologies B.V.; March 2023. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/217759s000lbl.pdf. Accessed March 28, 2023.
2. ClinicalTrials.gov. Study of efficacy of CDZ173 in patients with APDS/PASLI. Available at: https://clinicaltrials.gov/ct2/show/study/NCT02435173. Accessed September 27, 2022.
3. Coulter TI, Chandra A, Bacon CM, Babar J, et al. Clinical spectrum and features of activated phosphoinositide 3-kinase δ syndrome: A large patient cohort study. J Allergy Clin Immunol. 2017 Feb;139(2):597-606.e4. doi: 10.1016/j.jaci.2016.06.021.
4. Maccari ME, Abolhassani H, Aghamohammadi A, et al. Disease evolution and response to rapamycin in activated phosphoinositide 3-kinase δ syndrome: The European Society for Immunodeficiencies-Activated Phosphoinositide 3-Kinase δ Syndrome Registry. Front Immunol. 2018 Mar 16;9:543. doi: 10.3389/fimmu.2018.00543.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
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