Prior Authorization Review Panel

CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 11/01/2025**
Policy Number: PA.CP.PHAR.597	**Effective Date: 05/2023** **Revision Date: 10/2025**
Policy Name: Leniolisib (Joenja)	

Type of Submission – Check all that apply:

- ☐ **New Policy**
- ☐ **Revised Policy***
- ✓ **Annual Review - No Revisions**
- ☐ **Statewide PDL -** *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

4Q 2025 annual review: no significant changes; references reviewed and updated.

Name of Authorized Individual (Please type or print): **Craig A. Butler, MD MBA**	**Signature of Authorized Individual:**

Clinical Policy: Leniolisib (Joenja)

Reference Number: PA.CP.PHAR.597
Effective Date: 05/2023
Last Review Date: 10/2025

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 Delta Syndrome (must meet all)**:**

1. Diagnosis of APDS;
2. Prescribed by or in consultation with an immunologist;
3. Age ≥ 12 years;
4. Weight ≥ 45 kg;
5. Confirmed PI3Kδ genetic mutation of either the PIK3CD (APDS1) or PIK3R1 (APDS2) gene;
6. 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)*;
7. 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

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 Delta 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.PHARM.01) applies;
2. Weight ≥ 45 kg;
3. Member is responding positively to therapy as evidenced by one of the following:

a. Naïve B-cell percentage;
b. Decreased frequency or severity of infections/improvement in immune function;
c. Improvement of autoimmune manifestations;
d. Prevention of downstream sequelae of lymphoproliferation (ie, malignancy, persistent lymphadenopathy) and recurrent infections (ie, bronchiectasis),;
e. Provider assessment the member continues to benefit;

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: 12 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.PHARM.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
- Prolonged or intermittent herpesvirus viremia

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. Joenja Prescribing Information. Warren, NJ: Pharming Healthcare Inc.; May 2025. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2025/217759s005lbl.pdf. Accessed August 5, 2025.
2. Rao VK, Webster S, Šedivá A, Plebani A, et al. A randomized, placebo-controlled phase 3 trial of the PI3Kδ inhibitor leniolisib for activated PI3Kδ syndrome. *Blood.* 2023 Mar 2;141(9):971-983. doi: 10.1182/blood.2022018546.
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
Policy created	04/2023
4Q 2023 annual review: no significant changes; references reviewed and updated.	10/2023
Extended initial approval duration from 3 months to 6 months to allow sufficient time for full clinical response to meet reauthorization criteria; extended continued therapy approval duration from 6 months to 12 months; references reviewed and updated.	01/2024
4Q 2024 annual review: no significant changes; references reviewed and updated.	10/2024
4Q 2025 annual review: no significant changes; references reviewed and updated.	10/2025