

Applicable Drugs: Joenja (leniolisib)

Preferred: N/A

Non-preferred: N/A

Date of Origin: 8/26/2024

Date Last Reviewed / Revised: 4/1/2026

PRIOR AUTHORIZATION CRITERIA

(May be considered medically necessary when criteria I to VII are met)

- I. Diagnosis of activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) and the following criteria A through E are met:
 - A. Documentation of genetic testing results confirming APDS-associated mutation with a pathogenic variant in PIK3CD or PIK3R1 genes.
 - B. Documentation of at least one measurable enlarged lymph node lesion observed by computed tomography or magnetic resonance imaging.
 - C. Documentation of baseline naïve B cell percentage as assessed by flow cytometry.
 - D. Documentation of ≥ 1 clinical finding consistent with APDS. Examples include:
 - i. History of recurrent infections requiring long-term antibiotic or antiviral prophylaxis (eg, sinopulmonary infections, recurrent herpesvirus infections)
 - ii. Organ dysfunction (eg, bronchiectasis, liver impairment, enteropathy)
 - iii. History of nodal or extra-nodal lymphoproliferation (eg, lymphadenopathy, hepatomegaly, splenomegaly)
 - iv. Autoimmune cytopenias
 - v. Lymphoma
 - E. Documentation of treatment failure, intolerance, or contraindication of ≥ 1 agent from each of the following classes:
 - i. Systemic corticosteroids
 - ii. Immunoglobulin G replacement therapy
 - iii. Immunosuppressants (eg, rituximab, sirolimus)
- II. Minimum age requirement: 12 years old
- III. Minimum weight requirement: 45 kg
- IV. Documentation that Joenja (leniolisib) will not be used in combination with immunosuppressive therapy (eg, B lymphocyte depletion therapy, mTOR inhibitors, cyclosporine, mycophenolate, 6-mercaptopurine, azathioprine, methotrexate, or glucocorticoids at doses > 25 mg/day prednisone equivalent).

- V. Joenja (leniolisib) is prescribed by, or in consultation with, an immunologist, geneticist, or a provider specializing in the management of immunodeficiencies.
- VI. Request is for a medication with the appropriate FDA labeling, or its use is supported by current clinical practice guidelines
- VII. Refer to the plan document for the list of preferred products. If the requested agent is not listed as a preferred product, must have documented treatment failure or contraindication to the preferred product(s).

EXCLUSION CRITERIA

- N/A

OTHER CRITERIA

- N/A

QUANTITY / DAYS SUPPLY RESTRICTIONS

- Joenja (leniolisib) 70 mg tablets: 60 tablets per 30 days

APPROVAL LENGTH

- **Authorization:** 6 months
- **Re-Authorization:** 12 months with an updated letter of medical necessity or progress notes showing current medical necessity criteria are met and clinical benefits of treatment as documented by ≥ 1 of the following parameters:
 - 1) Reduction in nodal or extra-nodal lymphoproliferation from pre-treatment baseline
 - 2) Increase in naïve B cell percentage from pre-treatment baseline
 - 3) Decrease in the frequency and severity of infections

APPENDIX

N/A

REFERENCES

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2. Joenja. Prescribing Information. Pharming Technologies BV; 2025. Accessed March 22, 2026. <https://joenja-hcp.com/prescribing-information.pdf>

3. Coulter TI, Cant AJ. The treatment of activated PI3K δ syndrome. *Front Immunol*. 2018;9:2043. Published 2018 Sep 7. doi:10.3389/fimmu.2018.02043
4. Singh A, Joshi V, Jindal AK, Mathew B, Rawat A. An updated review on activated PI3 kinase delta syndrome (APDS). *Genes Dis*. 2019;7(1):67-74. Published 2019 Oct 14. doi:10.1016/j.gendis.2019.09.015
5. Rao VK, Webster S, Dalm VASH, et al. Effective "activated PI3K δ syndrome"-targeted therapy with the PI3K δ inhibitor leniolisib. *Blood*. 2017;130(21):2307-2316. doi:10.1182/blood-2017-08-801191
6. Study of Efficacy of CDZ173 in Patients With APDS/PASLI. ClinicalTrials.gov identifier: NCT02435173. Updated August 10, 2022. Accessed March 22, 2026. <https://clinicaltrials.gov/study/NCT02435173?intr=Leniolisib&rank=4>
7. Rao VK, Webster S, Šedivá A, et al. A randomized, placebo-controlled phase 3 trial of the PI3K δ inhibitor leniolisib for activated PI3K δ syndrome. *Blood*. 2023;141(9):971-983. doi:10.1182/blood.2022018546
8. Rao VK, Kulm E, Šedivá A, et al. Interim analysis: Open-label extension study of leniolisib for patients with APDS. *J Allergy Clin Immunol*. 2024;153(1):265-274.e9. doi:10.1016/j.jaci.2023.09.032
9. Extension to the Study of Efficacy of CDZ173 in Patients With APDS/PASLI. ClinicalTrials.gov identifier: NCT02859727. Updated March 12, 2025. Accessed March 22, 2026. <https://clinicaltrials.gov/study/NCT02859727?intr=Leniolisib&rank=5>

DISCLAIMER: Medication Policies are developed to help ensure safe, effective and appropriate use of selected medications. They offer a guide to coverage and are not intended to dictate to providers how to practice medicine. Refer to Plan for individual adoption of specific Medication Policies. Providers are expected to exercise their medical judgement in providing the most appropriate care for their patients.