

## PHARMACY POLICY STATEMENT

### North Carolina Marketplace

<b>DRUG NAME</b>	<b>Joenja (leniolisib)</b>
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Joenja, approved by the FDA in 2023, is a small molecule kinase inhibitor indicated for the treatment of activated phosphoinositide 3-kinase delta (PI3K $\delta$ ) syndrome (APDS) in adult and pediatric patients 12 years of age and older. It selectively targets PI3K $\delta$  signaling by inhibiting its hyperactive subunit.

APDS is also known as p110 $\delta$ -activating mutation causing senescent T cells, lymphadenopathy, and immunodeficiency (PASLI). It is an ultra-rare disease caused by variants in either of the genes encoding the PI3K $\delta$  heterodimer (*PIK3CD* in APDS1 or *PIK3R1* in APDS2) which leads to hyperactive PI3K $\delta$  signaling. This results in disrupted immune cell development and function. Clinical manifestations include infections, nonmalignant lymphoproliferation, autoimmunity (e.g., cytopenias), enteropathy, bronchiectasis, and increased risk of lymphoma.

In a Phase 3 study, Joenja met the coprimary endpoints of reducing lymphadenopathy and normalizing immune cell subsets. It is the first FDA-approved drug for APDS.

Joenja (leniolisib) will be considered for coverage when the following criteria are met:

#### Activated Phosphoinositide 3-Kinase Delta (PI3K $\delta$ ) syndrome (APDS)

For **initial** authorization:

1. Member is at least 12 years of age; AND
2. Medication must be prescribed by or in consultation with an immunologist or hematologist; AND
3. Member has a diagnosis of APDS confirmed by an APDS-associated genetic PI3K $\delta$  mutation with a documented variant in either *PIK3CD* or *PIK3R1*; AND
4. Chart notes must show clinical findings/manifestations of APDS (e.g., history of repeated oto-sino-pulmonary infections); AND
5. Chart notes must document at least 1 measurable nodal lesion by CT or MRI; AND
6. Joenja is NOT being prescribed concurrently with an immunosuppressive medication.
7. **Dosage allowed/Quantity limit:** Weight 45 kg or greater: 70 mg orally twice daily. (QL: 60 tablets per 30 days)

***If all the above requirements are met, the medication will be approved for 6 months.***

For **reauthorization**:

1. Chart notes must show positive clinical response such as reduced lymph node size, increased naïve B cell percentage (out of total B cells), reduced spleen volume, or improved cytopenias.

***If all the above requirements are met, the medication will be approved for an additional 12 months.***

**CareSource considers Joenja (leniolisib) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.**

DATE	ACTION/DESCRIPTION
04/24/2023	New policy for Joenja created.

References:

1. Joenja [prescribing information]. Pharming Technologies B.V.; 2023.
2. Rao VK, Webster S, Šedivá A, et al. A randomized, placebo-controlled phase 3 trial of the PI3K $\delta$  inhibitor leniolisib for activated PI3K $\delta$  syndrome. *Blood*. 2023;141(9):971-983. doi:10.1182/blood.2022018546
3. Rao VK, Webster S, Dalm VASH, et al. Effective "activated PI3K $\delta$  syndrome"-targeted therapy with the PI3K $\delta$  inhibitor leniolisib. *Blood*. 2017;130(21):2307-2316. doi:10.1182/blood-2017-08-801191
4. Coulter TI, Cant AJ. The Treatment of Activated PI3K $\delta$  Syndrome. *Front Immunol*. 2018;9:2043. Published 2018 Sep 7. doi:10.3389/fimmu.2018.02043
5. 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

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Revised date: 04/24/2023