| Reference number(s) |
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| 5859-A |

# SPECIALTY GUIDELINE MANAGEMENT

## JOENJA (leniolisib)

### POLICY

#### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

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

All other indications are considered experimental/investigational and not medically necessary.

#### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Testing or analysis confirming a mutation of either *PIK3CD* or *PIK3R1* gene.
B. Medical record documentation confirming the member demonstrates clinical manifestations of the disease (e.g., history of repeated oto-sino-pulmonary infections, lymphoproliferation, autoimmunity [e.g., cytopenia], enteropathy, organ dysfunction [e.g., lung, liver]).

#### III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with an immunologist or a physician who specializes in the treatment of APDS.

#### IV. CRITERIA FOR INITIAL APPROVAL

**Activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)**
Authorization of 6 months may be granted when all of the following criteria are met:
A. Member's diagnosis is confirmed by detection of mutation of either *PIK3CD* or *PIK3R1* gene.
B. Member has clinical manifestations compatible with APDS (e.g., history of repeated oto-sino-pulmonary infections, lymphoproliferation, autoimmunity [e.g., cytopenia], enteropathy, organ dysfunction [e.g., lung, liver]).
C. Member is 12 years of age and older weighing greater than or equal to 45 kg.

#### V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in all members (including new members) who are currently receiving the requested medication and who are experiencing benefit from therapy as evidenced by disease stability or disease improvement.

## VI. REFERENCES

1. Joenja [package insert]. Warren, NJ: Pharming Technologies B.V.; March 2023.
2. Rao VK, Webster S, Šedivá 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. doi:10.1182/blood.2022018546.