

Patient Experiences with Joenja

Look inside to learn about Joenja and see how it impacted patients with APDS who were observed for 6 years.¹



APDS, activated PI3K δ syndrome; PI3K δ , phosphoinositide 3-kinase delta.

Indications and Usage

JOENJA[®] (leniolisib) is a kinase inhibitor indicated for the treatment of activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) in adult and pediatric patients 12 years of age and older.

Select Safety Information

Verify pregnancy status in females of reproductive potential prior to initiating treatment with JOENJA.

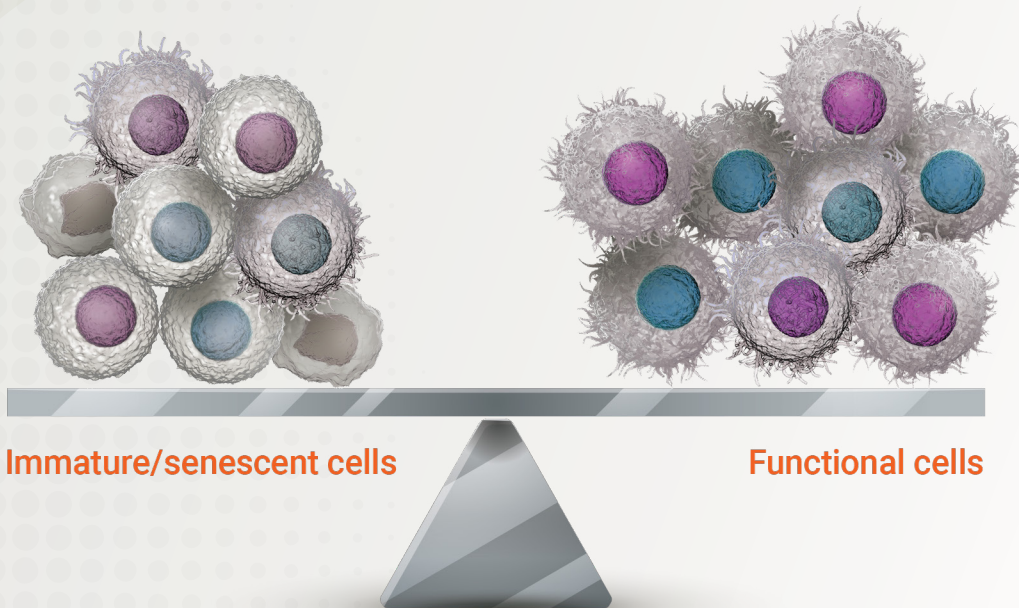
Please see Important Safety Information on page 23. Before prescribing JOENJA, please read the full Prescribing Information.

Joenja is the first and only selective PI3K δ inhibitor indicated for the treatment of APDS²⁻⁵



Joenja helps address both immune deficiency AND immune dysregulation⁶

Joenja facilitates a balanced PI3K δ pathway to support proper immune function^{2,7}



This is a graphical representation of a complex biological process.



Joenja is an oral, selective PI3K δ inhibitor that is designed to help regulate the hyperactive signaling pathway^{2,5}

Select Safety Information

JOENJA may cause fetal harm when administered to a pregnant woman. Advise patients of the potential risk to a fetus and to use highly effective methods of contraception during treatment with JOENJA and for 1 week after the last dose of JOENJA.

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Joenja pivotal clinical trials^{2,5,8}

Joenja[®]
leniolisib

PART 1 Dose-finding

12 weeks
N=6

- Nonrandomized, open-label, dose-finding study in 6 patients with APDS; dose range was 10 mg, 30 mg, and 70 mg BID for 4 weeks at each dose
- Oral dose 70 mg BID selected for part 2

PART 2 Efficacy and safety evaluation

Randomized period
12 weeks
N=31

- Randomized, triple-blinded (patient, caregiver, investigator), placebo-controlled, fixed-dose study of 70 mg BID
- Co-primary efficacy end points (improvement in lymphoproliferation and normalization of immunophenotype)
 - Change from baseline in the \log_{10} -transformed SPD of index lesions
 - Change from baseline in percentage of naive B cells out of total B cells
- Secondary and exploratory end point assessments
- Safety assessment

Open-label extension (OLE) study

N=37

- An open-label, nonrandomized extension study to evaluate the long-term safety, tolerability, efficacy, and pharmacokinetics of Joenja in patients with APDS
 - 35 patients from parts 1 and 2
 - 2 patients previously treated with an investigational PI3K δ inhibitor
- Primary outcome measure: long-term safety and tolerability

BID, twice a day; SPD, sum of product diameters.

Select Safety Information

Live, attenuated vaccinations may be less effective if administered during JOENJA treatment.

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JOENJA

SAFETY

OLE

CASE 1

CASE 2

CASE 3

CASE 4

CASE 5

CASE 6

Significant reductions in lymphadenopathy²



Log₁₀-transformed SPD of index lesions (excluding patients with 0 lesions at baseline) at week 12^{2*}

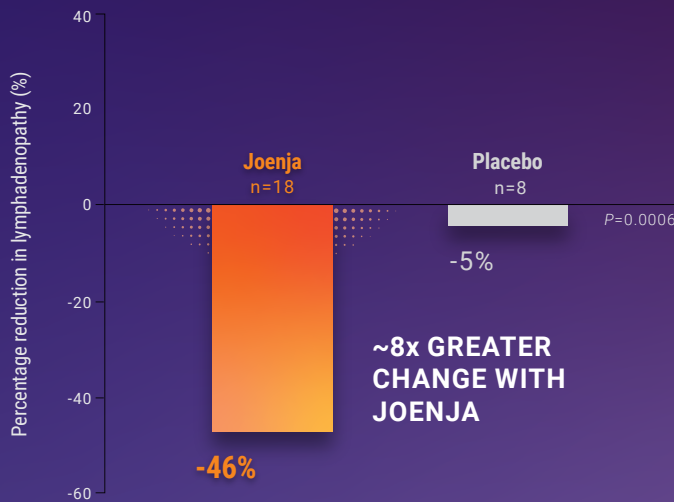
	Joenja (n/N=18/21) [†]	Placebo (n/N=8/10) [†]	
Baseline mean (SD)	3.03 (0.42)	3.05 (0.39)	
Change from baseline, LS mean (SE)	-0.27 (0.04)	-0.02 (0.05)	
Difference vs placebo (95% CI)		-0.25 (-0.38, -0.12)	P=0.0006

*The LS mean change from baseline, difference in LS mean change from baseline between Joenja and placebo and its P value, were obtained from an ANCOVA model with treatment, glucocorticoid use, and IRT at baseline, and baseline measurement as covariates.²

[†]The analysis excluded 2 patients from each treatment group due to protocol deviations and 1 Joenja patient having complete resolution of the index lesion identified at baseline.²

- Improvement in lymphoproliferation as measured by a change from baseline in lymphadenopathy measured by the log₁₀-transformed SPD of index lymph nodes²

At week 12, patients saw a significant reduction in lymphadenopathy[‡] with Joenja vs placebo^{2,3}



46%
REDUCTION WITH JOENJA VS 5% WITH PLACEBO^{2,3}

Reduction computed based on estimates for the adjusted mean changes.²

[‡]Change in index lesion size was measured using the log₁₀-transformed SPD of the largest lymph nodes (maximum of 6) identified as per the Cheson criteria on CT/MRI.²

ANCOVA, analysis of covariance; CI, confidence interval; CT, computed tomography; IRT, immunoglobulin replacement therapy; LS, least squares; MRI, magnetic resonance imaging; SD, standard deviation; SE, standard error.

Select Safety Information

Use of JOENJA in patients with moderate to severe hepatic impairment is not recommended. There is no recommended dosage for patients weighing less than 45 kg.

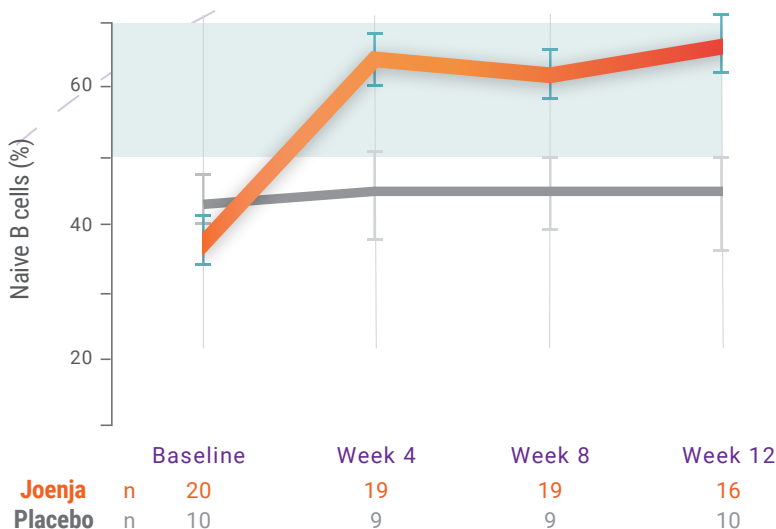
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Significant increase in naive B cells⁶

Significantly improved immunophenotype vs placebo at week 12^{2,6}

- In patients with <48% of naive B cells at baseline,* the adjusted mean difference between Jojenja (n=8) and placebo (n=5) in the percentage of naive B cells out of total B cells was 37.30 (95% CI: 24.06, 50.54); P=0.0002[†]
- The adjusted mean change from baseline (SE) for Jojenja was 37.39 (5.34) and 0.09 (6.66) for placebo

Absolute percentage of naive B cells over time^{2,6}



NORMAL RANGE FOR PERCENTAGE OF NAIVE B CELLS INDICATED BY SHADED BAR IN GRAPH

MEAN NAIVE B-CELL LEVELS WITHIN NORMAL RANGE BY WEEK 4 AND MAINTAINED THROUGH WEEK 12 WITH JOENJA⁶

*Cell surface markers used to distinguish naive B cells on flow cytometry were CD19+, CD27-, and CD10-. Baseline is defined as the arithmetic mean of the baseline and day 1 values when both were available, and if either value was missing, the existing value was used.^{2,6}

[†]The analysis excluded 2 patients from each treatment group due to protocol deviations, 5 Jojenja patients and 3 placebo patients with ≥48% naive B cells at baseline, 5 Jojenja patients with no day 85 measurement, and 1 Jojenja patient with no baseline measurement.²



Jojenja effectively helped normalize immune balance in patients with APDS^{2,6}

Select Safety Information

The most common adverse reactions (incidence >10%) seen in clinical trials were headache, sinusitis, and atopic dermatitis.

Please see Important Safety Information on page 23. Before prescribing JOENJA, please read the full Prescribing Information.

Joenja safety profile^{2,6}



- The safety of Joenja is based on data from both the placebo-controlled pivotal trial and the interim results from the OLE study

Adverse reactions reported by ≥2 Joenja-treated patients and more frequently than placebo

	Joenja (n=21) n (%)	Placebo (n=10) n (%)
Headache	5 (24)	2 (20)
Sinusitis	4 (19)	0
Dermatitis atopic*	3 (14)	0
Tachycardia [†]	2 (10)	0
Diarrhea	2 (10)	0
Fatigue	2 (10)	1 (10)
Pyrexia	2 (10)	0
Back pain	2 (10)	0
Neck pain	2 (10)	0
Alopecia	2 (10)	0

*Dermatitis atopic: including dermatitis atopic and eczema.

[†]Tachycardia: including tachycardia and sinus tachycardia.

- No serious adverse drug reactions were reported
- No patients withdrew due to an adverse drug reaction
- The most common adverse reactions (>10%) were headache, sinusitis, and dermatitis atopic

Select Safety Information

Seven (33%) patients receiving JOENJA developed an absolute neutrophil count (ANC) between 500 and 1500 cells/microL. No patients developed an ANC <500 cells/ microL and there were no reports of infection associated with neutropenia.

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Additional safety results from an interim analysis^{2,8,9}

At the data cutoff (December 2021)

- 37 of 38 patients received Joenja 70 mg orally twice daily for at least 25 weeks; 66% were exposed for 96 weeks or longer
- Median duration of Joenja treatment was approximately 2 years
- 4 patients had more than 5 years of Joenja exposure

Most common AEs

	n
Upper respiratory tract infection	9
Headache	6
Pyrexia	6
Otitis externa	5
Weight increase	5
COVID-19 positive	5

Distribution and grades* of AEs

- 32 of 37 patients had ≥1 AE (333 AEs reported)
- 78.4% were grade 1, 48.6% were grade 2, and 27% were grade 3
- No grade 4 AEs reported
- One grade 5 patient with significant baseline comorbidities suffered cardiac arrest, resulting in death on day 879; investigator determined that the death was not related to study drug
- No serious AEs were related to Joenja treatment

AEs associated with Joenja

- The AEs reported as related to study drug were weight increase (3 patients), arthralgia (1 patient), hyperglycemia (1 patient), and decreased neutrophil count (1 patient)

*CTCAE were used to determine AE grade. If CTCAE grading did not exist for an AE, the following definitions were used: 1, mild; 2, moderate; 3, severe; 4, life-threatening; 5, death.

AE, adverse event; CTCAE, common terminology criteria for adverse events.

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The open-label extension study is ongoing¹

Joenja[®]
leniolisib

Additional findings are still being observed in the open-label extension study, including¹:

- Annual infection rates
- Immunoglobulin replacement therapy reductions and discontinuations



Not actual patients

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JOENJA

SAFETY

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CASE 1

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CASE 6

Take a closer look at 6 patients from the open-label extension trial¹

See how Joenja treatment helped them over 6 years¹

- These patient cases are from a retrospective analysis of 6 patients enrolled in the dose-finding trial and the open-label extension study. These patients were not enrolled in the pivotal efficacy and safety evaluation trial for Joenja¹
- People with APDS suffer from a wide range of symptoms and response to treatment varies^{3,10-12}
- The clinical significance of these patient case observations is not known. Study limitations included variable timing of patient visits and treatment gaps up to roughly 1 year¹
- The clinician-reported questionnaire utilized was subject to recall and investigator bias, and the data captured were subjective by nature, which limited generalizability¹
- For all patients discussed, names and images were changed to protect their identity
- Individual results may vary

REGULAR ARTICLE
Check for updates



Long-term treatment with selective PI3K δ inhibitor leniolisib in adults with activated PI3K δ syndrome

V. Konetl Rao,¹ Elaine Kulm,² Jennifer Grossman,³ David Buchbinder,⁴ Hey Chong,⁵ Jason Bradt,⁶ Sharon Webster,¹ Anna Šedivá,⁷ Virgil A. Dalm,⁸ and Gulbu Uzel¹

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Key Points

• HRQoL improved in all patients with APDS receiving selective PI3K δ inhibitor leniolisib for up to 6 years, with minimal toxicities.

• Patients with APDS receiving leniolisib saw

Activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) is an inborn error of immunity that manifests as immune deficiency and dysregulation; symptoms include frequent infections and lymphoproliferation. In our dose-finding and phase 3 placebo-controlled trials, treatment with the selective PI3K δ inhibitor leniolisib reduced lymphoproliferation and normalized lymphocyte subsets. Here, we present 6 years of follow-up from the 6 adult patients in the original dose-finding trial receiving leniolisib. We used data from the ongoing open-label extension study, which was supplemented at later time points by investigators, including health-related quality of life (HRQoL) assessed through a clinician-reported questionnaire. We observed improvements in HRQoL: 5 of 6 patients experienced an increase in physical capabilities and socialization, and a decrease in respiratory medications. Immune subsets improved in all patients, except transitional B

[Read the full analysis](#)

Blood Advances is a registered trademark of the American Society of Hematology, Inc.

Select Safety Information

Live, attenuated vaccinations may be less effective if administered during JOENJA treatment.

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Meet a 24-year-old male with APDS

whose progress was followed in the Joenja open-label extension study for 6 years¹

- In clinical studies, patients taking Joenja saw a reduction in lymph node size and an improvement in naive B-cell counts^{2,6}
- Joenja has not been proven to impact quality of life

At the most recent check-in



No longer struggling with school attendance, this patient has graduated from high school and an online university



He has a full-time job, can walk and drive without difficulty, and manages his own medical care

Select Safety Information

Use of JOENJA in patients with moderate to severe hepatic impairment is not recommended. There is no recommended dosage for patients weighing less than 45 kg.

Please see Important Safety Information on page 23. Before prescribing JOENJA, please read the full Prescribing Information.

Before and after starting Joenja treatment¹

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none"> Experienced fatigue from IRT infusions, anxiety, and difficulty coping with treatment burden Hospitalized yearly for infections Frequently prescribed antibiotics 	<ul style="list-style-type: none"> Stopped IRT infusions and fatigue got better No hospitalizations He had 7 infections, none of which returned Only doctor he visits regularly is his immunologist
Clinical manifestations	<ul style="list-style-type: none"> Low blood platelet counts Damaged lung airways Gastrointestinal issues and migraines 	<ul style="list-style-type: none"> Blood platelet count increased Damaged lung airways did not get worse

Additional information¹

Over the course of the trial, he received 14 prescription medicines, receiving 2 at year 6.

He experienced some side effects within the first 2 years of treatment that were mild and cleared up, including:

- Gastroparesis (slowed stomach emptying)
- Hypertriglyceridemia (too much fat in the blood)

Select Safety Information

The most common adverse reactions (incidence >10%) seen in clinical trials were headache, sinusitis, and atopic dermatitis.

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Meet a 27-year-old male with APDS

whose progress was followed in the Joenja open-label extension study for 6 years¹

- In clinical studies, patients taking Joenja saw a reduction in lymph node size and an improvement in naive B-cell counts^{2,6}
- Joenja has not been proven to impact quality of life

At the most recent check-in



No longer struggling with shortness of breath, this patient can go on extended walks and his outlook on life has improved noticeably



His family members saw differences in his physical capacities and noted that he has become more social

Select Safety Information

Seven (33%) patients receiving JOENJA developed an absolute neutrophil count (ANC) between 500 and 1500 cells/microL. No patients developed an ANC <500 cells/ microL and there were no reports of infection associated with neutropenia.

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Before and after starting Joenja treatment¹

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none"> • Recurrent infections which occasionally required hospitalization • Infectious pneumonia • Discontinued IRT 	<ul style="list-style-type: none"> • No hospitalizations • Infectious pneumonia improved within a year, but infiltrates (substances that stay in the lungs, such as blood) were present
Clinical manifestations	<ul style="list-style-type: none"> • Low body weight for his age • Low red and white blood cell counts • Damaged lung airways (due to infectious pneumonia) 	<ul style="list-style-type: none"> • Gained weight • Red blood cell count improved within 6 weeks of starting the extension trial • White blood cell counts improved through year 6 • Damaged lung airways did not worsen and stabilized at year 6

Additional information¹

Although 5 medications were prescribed during the 6-year period, only 3 medications were taken regularly and he was down to 2 medications by year 6.

He experienced some side effects, including:

- Lyme disease in the first year, which was mild and cleared up
- Low white blood cell counts, which occurred shortly after the open-label extension trial started and resolved around year 2

Select Safety Information

Verify pregnancy status in females of reproductive potential prior to initiating treatment with JOENJA.

Please see Important Safety Information on page 23. Before prescribing JOENJA, please read the full [Prescribing Information](#).



Meet a 31-year-old female with APDS

whose progress was followed in the Joenja open-label extension study for 6 years¹

- In clinical studies, patients taking Joenja saw a reduction in lymph node size and an improvement in naive B-cell counts^{2,6}
- Joenja has not been proven to impact quality of life

At the most recent check-in



After being homeschooled due to illness, she is continuing her education and now has a full-time job



Now with more energy, she takes aerobics classes and walks. She's traveled overseas to visit friends and manages her own medical care

Select Safety Information

JOENJA may cause fetal harm when administered to a pregnant woman. Advise patients of the potential risk to a fetus and to use highly effective methods of contraception during treatment with JOENJA and for 1 week after the last dose of JOENJA.

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Before and after starting Joenja treatment¹

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none"> • Frequent nose and lower respiratory infections • Diagnosed with stage IV diffuse large B-cell lymphoma at 19 years old, and had a full remission before starting Joenja* <p>* No known recurrence of lymphoma during the study period.</p>	<ul style="list-style-type: none"> • She had 2 infections during the first 2 years of treatment
Clinical manifestations	<ul style="list-style-type: none"> • Low blood platelet counts • Low white blood cell counts 	<ul style="list-style-type: none"> • Improved blood platelet counts • White blood cell counts were within normal limits by year 2 and remained normal

Additional information¹

She has received 8 prescription medicines, receiving 3 at year 6, and visits her local doctor regularly.

She experienced some side effects, including:

- Hay fever, dental cavities, low white blood cell counts, and elevated liver enzymes
- These side effects were mild and cleared up

She also experienced some side effects that were more serious, but cleared up:

- Alanine aminotransferase (increase in liver enzymes)
- Reactive arthritis (which happened in year 4)

Select Safety Information

Live, attenuated vaccinations may be less effective if administered during JOENJA treatment.

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Meet a 39-year-old male with APDS

whose progress was followed in the Jojenja open-label extension study for 6 years¹

- In clinical studies, patients taking Jojenja saw a reduction in lymph node size and an improvement in naive B-cell counts^{2,6}
- Jojenja has not been proven to impact quality of life

At the most recent check-in



No longer affected by fatigue, he is able to participate in activities with his children



Within 2 months of starting treatment, he returned to work and socializes with colleagues after work

Select Safety Information

Use of JOENJA in patients with moderate to severe hepatic impairment is not recommended. There is no recommended dosage for patients weighing less than 45 kg.

Please see Important Safety Information on page 23. Before prescribing JOENJA, please read the full [Prescribing Information](#).

Before and after starting Joenja treatment¹

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none"> • Recurrent respiratory tract infections • Continually taking antibiotics • Malignancy of the lymph nodes at age 20, with a full remission before starting Joenja 	<ul style="list-style-type: none"> • Discontinued IRT at year 4 after 2 years of dose adjustments. After stopping IRT, he had 4 mild infections and the frequency and severity of infections declined • Periodic infections through year 4 • 2 confirmed cases of COVID-19, 1 in year 5 (with mild inflammation of nasal passages) and 1 in year 6 • He visits immunology-pulmonology clinic yearly, and remains under the care of his otolaryngologist. He sees them less frequently
Clinical manifestations	<ul style="list-style-type: none"> • Low blood platelet counts 	<ul style="list-style-type: none"> • Low blood platelet counts resolved around the end of year 1

Additional information¹

He received 14 prescription medications over 6 years. He received 2 at year 6 and no longer needed preventative antibiotics.

He experienced some side effects that were mild and cleared up, including hearing loss, nosebleeds, diarrhea, headache, inflammation at his bone-anchored hearing aid, jaw pain, sore throat, tickling cough, painful wrist, reflux esophagitis, and Fuchs heterochromic iridocyclitis (swelling and inflammation of the middle layer of the eye).

Select Safety Information

The most common adverse reactions (incidence >10%) seen in clinical trials were headache, sinusitis, and atopic dermatitis.

Please see Important Safety Information on page 23. Before prescribing JOENJA, please read the full [Prescribing Information](#).



Meet a 23-year-old female with APDS

whose progress was followed in the Jojenja open-label extension study for 6 years¹

- In clinical studies, patients taking Jojenja saw a reduction in lymph node size and an improvement in naive B-cell counts^{2,6}
- Jojenja has not been proven to impact quality of life

At the most recent check-in



She graduated from college and started working full-time as a schoolteacher



With her anxiety better controlled, she became more assertive, remained active, and expanded her circle of friends. She discontinued her anxiety medication and regularly visits her therapist

Select Safety Information

Seven (33%) patients receiving JOENJA developed an absolute neutrophil count (ANC) between 500 and 1500 cells/microL. No patients developed an ANC <500 cells/ microL and there were no reports of infection associated with neutropenia.

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Before and after starting Joenja treatment¹

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none"> • Frequent throat and nose infections that required consistent antibiotics • Recurrent Epstein-Barr virus infections 	<ul style="list-style-type: none"> • 12 infections occurred that were managed efficiently • Highest Epstein-Barr virus levels were clinically significant, but no infection was observed • Contracted COVID-19 but had mild symptoms (cough and runny nose) that resolved • She visits her immunologist and pulmonologist less frequently
Clinical manifestations	<ul style="list-style-type: none"> • Low white blood cell counts 	<ul style="list-style-type: none"> • Low white blood cell counts resolved around the end of year 1

Additional information¹

She received 17 prescription medications, receiving 7 at year 6, with pulmonary medications prescribed as needed.

She experienced some side effects that were mild and cleared up, including rash, sunburn, hair loss, oral ulcer, seborrheic dermatitis (dandruff, scaly patches, and/or inflamed skin), and temporomandibular joint pain (pain and discomfort in the jaw joint).

Weight gain and myalgia (muscle aches and pain) were unresolved side effects.

Select Safety Information

Verify pregnancy status in females of reproductive potential prior to initiating treatment with JOENJA.

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Meet a 32-year-old male with APDS

whose progress was followed in the Joenja open-label extension study for 6 years¹

- In clinical studies, patients taking Joenja saw a reduction in lymph node size and an improvement in naive B-cell counts^{2,6}
- Joenja has not been proven to impact quality of life

At the most recent check-in



He works a full-time, labor-intensive job. As symptoms improved, he experienced positive changes in his mood



He did not report any changes in shortness of breath, but he has airway damage from lymphoma chemotherapy in 2011

Select Safety Information

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Before and after starting Joenja treatment¹

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none"> • IRT • Repeated airway infections • Prophylactic antibiotics • Diagnosed with Hodgkin lymphoma at 11 years old and had a full remission before starting Joenja 	<ul style="list-style-type: none"> • IRT dose was reduced • Frequency and severity of infections reduced with 8 relapses of respiratory infections • Prophylactic antibiotics stopped • Acute infections cleared more quickly • He is only monitored by his pneumologist and visits have decreased
Clinical manifestations	<ul style="list-style-type: none"> • Low white blood cell counts • Bronchiectasis • Rash, joint stiffness, and swelling • Gastrointestinal issues • Shortness of breath 	<ul style="list-style-type: none"> • Low white blood cell counts resolved by year 2 • Bronchiectasis remained stable out to year 6 • Rash and joint pain resolved • Gastrointestinal conditions improved

Additional information¹

He received 22 prescription medications, receiving 7 at year 6, 3 of which were deemed crucial.

He experienced some side effects that were mild and cleared up, including pruritus (itching), sore throat, seborrheic dermatitis (dandruff, scaly patches, and/or inflamed skin), diarrhea, productive cough (produces mucus), and anxiety.

He experienced some unresolved side effects, including COVID-19–associated mild dyspnea (shortness of breath), hepatopathy (abnormal liver), and hypercholesterolemia. The latter 2 side effects improved.

Select Safety Information




Live, attenuated vaccinations may be less effective if administered during JOENJA treatment.

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opening doors to help your patients with APDS move forward

APDS Assist can help:

-  Enroll your patients with APDS in our comprehensive program
-  Share information about patient-specific insurance requirements for Joenja
-  Provide information about our support services, including through our APDS Clinical Educators (ACEs)

Visit [Joenja.com](https://joenja.com) to enroll your patients today.

Want to hear more patient stories?

Learn about [other patients](#) and what Joenja treatment did for them

Select Safety Information

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Indications and Usage

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The most common adverse reactions (incidence >10%) seen in clinical trials were headache, sinusitis, and atopic dermatitis.

Seven (33%) patients receiving JOENJA developed an absolute neutrophil count (ANC) between 500 and 1500 cells/microL. No patients developed an ANC <500 cells/microL and there were no reports of infection associated with neutropenia.

Before prescribing JOENJA, please read the full [Prescribing Information](#).

References: 1. Rao VK, Kulm E, Grossman J, et al. Long-term treatment with selective PI3K δ inhibitor leniolisib in adults with activated PI3K δ syndrome. *Blood Adv.* 2024;8(12):3092-3108. doi:10.1182/bloodadvances.2023011000 2. Joenja (leniolisib). Prescribing information. Pharming Healthcare, Inc; 2023. 3. Data on file. Pharming Healthcare, Inc. 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;9:543. doi:10.3389/fimmu.2018.00543 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. 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 7. Nunes-Santos CJ, Uzel G, Rosenzweig SD. PI3K pathway defects leading to immunodeficiency and immune dysregulation. *J Allergy Clin Immunol.* 2019;143(5):1676-1687. 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. Rao VK. Interim safety and efficacy analysis of an ongoing long-term open-label extension study of leniolisib for patients with activated PI3K delta syndrome (APDS). Presented at the European Society for Immunodeficiencies; October 12-15, 2022; Gothenburg, Sweden. 10. Jamee M, Moniri S, Zaki-Dizaji M, et al. Clinical, immunological, and genetic features in patients with activated PI3K δ syndrome (APDS): a systematic review. *Clin Rev Allergy Immunol.* 2020;59(3):323-333. doi:10.1007/s12016-019-08738-9 11. Deau M-C, Heurtier L, Frange P, et al. A human immunodeficiency caused by mutations in the PIK3R1 gene. *J Clin Invest.* 2014;124(9):3923-3928. 12. Lucas CL, Chandra A, Nejentsev S, Condliffe AM, Okkenhaug K. PI3K δ and primary immunodeficiencies. *Nat Rev Immunol.* 2016;16(11):702-714. doi:10.1038/nri.2016.93

Prescribe twice-daily oral administration with Joenja²

Joenja[®]
leniolisib



Recommended administration of Joenja

- 70 mg, orally
- Twice daily, ~12 hours apart
- In adult and pediatric patients ≥12 years of age and weighing ≥45 kg

Joenja can be taken **with or without food**.

Advise patients that if a dose is missed by more than 6 hours, **wait and take the next dose at the usual time**.

Advise patients that if vomiting occurs

- **Within 1 hour** after taking Joenja, take Joenja as soon as possible
- **More than 1 hour** after taking Joenja, wait and take the next dose at the usual time

Joenja oral tablets can be taken anytime, anywhere²

- Joenja should be taken twice a day, approximately 12 hours apart

Select Safety Information

The most common adverse reactions (incidence >10%) seen in clinical trials were headache, sinusitis, and atopic dermatitis.

**Please see Important Safety Information on page 23.
Before prescribing JOENJA, please read the full
Prescribing Information.**



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SAFETY

OLE

CASE 1

CASE 2

CASE 3

CASE 4

CASE 5

CASE 6