

Joenja[®] (leniolisib)

The first and only FDA-approved targeted therapy for APDS

APDS, activated PI3K delta syndrome; FDA, Food and Drug Administration.

What's inside?

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TAKING JOENJA >

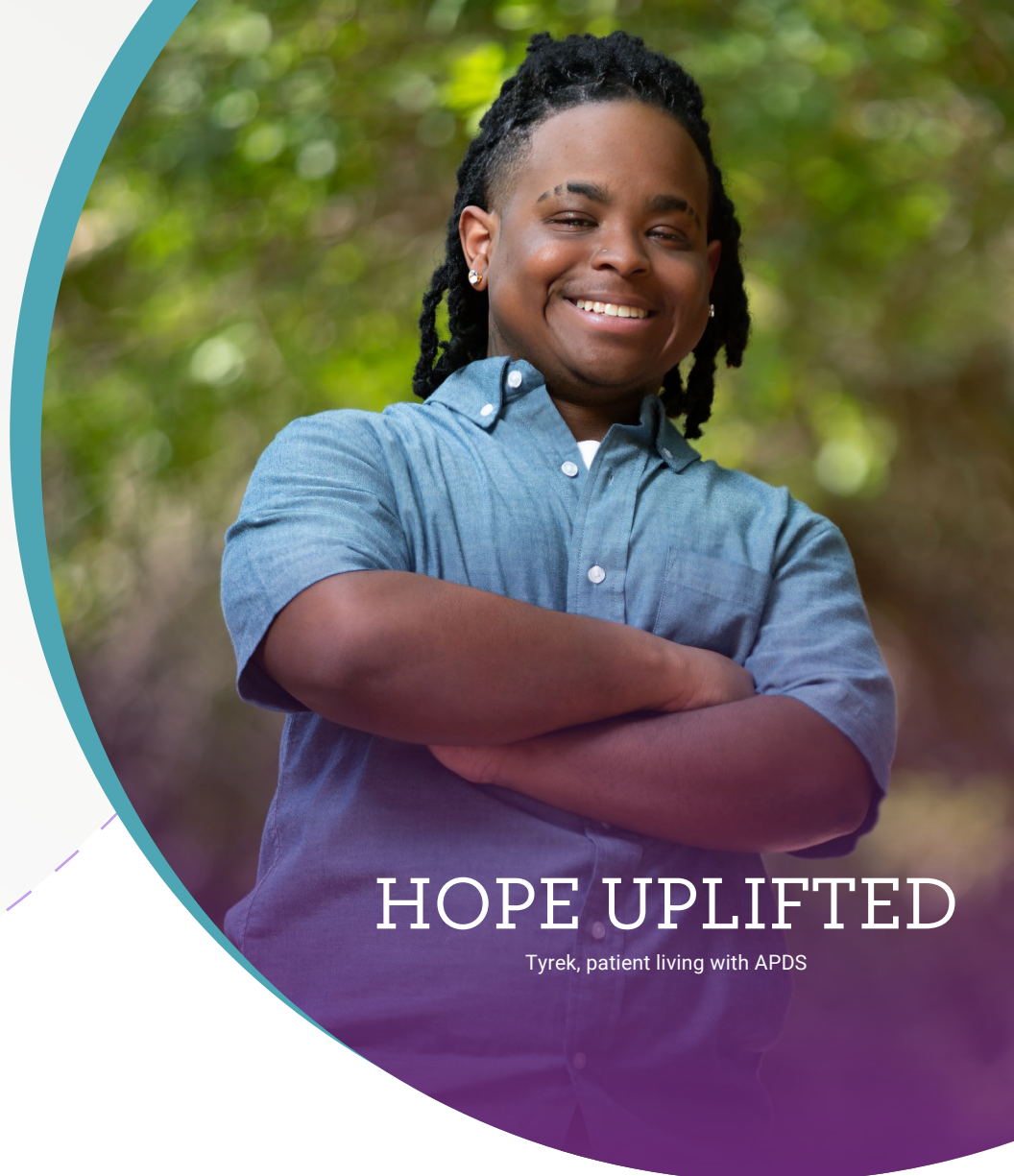
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HOPE UPLIFTED

Tyrek, patient living with APDS

Approved Use

JOENJA[®] (leniolisib) is a prescription medicine that is used to treat activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adults and children 12 years of age and older.

Select Safety Information

Tell your healthcare provider if you are pregnant or plan to become pregnant. JOENJA may harm your unborn baby. Your healthcare provider will do a pregnancy test before you start receiving JOENJA.

Please see additional Important Safety Information throughout.
For more information, please see the full [Prescribing Information](#).


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Understanding APDS



What is APDS?

- APDS is an inherited, rare primary immunodeficiency (PI) that is often overlooked or mistaken for other conditions*
- PIs are genetic disorders that cause parts of the body's immune system to be absent or not function properly



What causes APDS?

- It starts with changes in genes *PIK3CD* or *PIK3R1*
- These changes cause an immune pathway (PI3K delta) to become hyperactive. This means the body's immune cells (B and T cells) may not grow and work properly
- For people with APDS, this can create too many immature cells and not enough healthy ones for your body, which is why it can be hard to fight off infections

APDS is a progressive disease

It could continue to progress, or worsen, over time.
This could lead to permanent, life-threatening organ damage or lymphoma.

*APDS is a primary immunodeficiency, also known as an inborn error of immunity (or IEI).

Select Safety Information (cont.)

Use effective birth control to prevent pregnancy while taking JOENJA and for one week after you stop taking JOENJA.

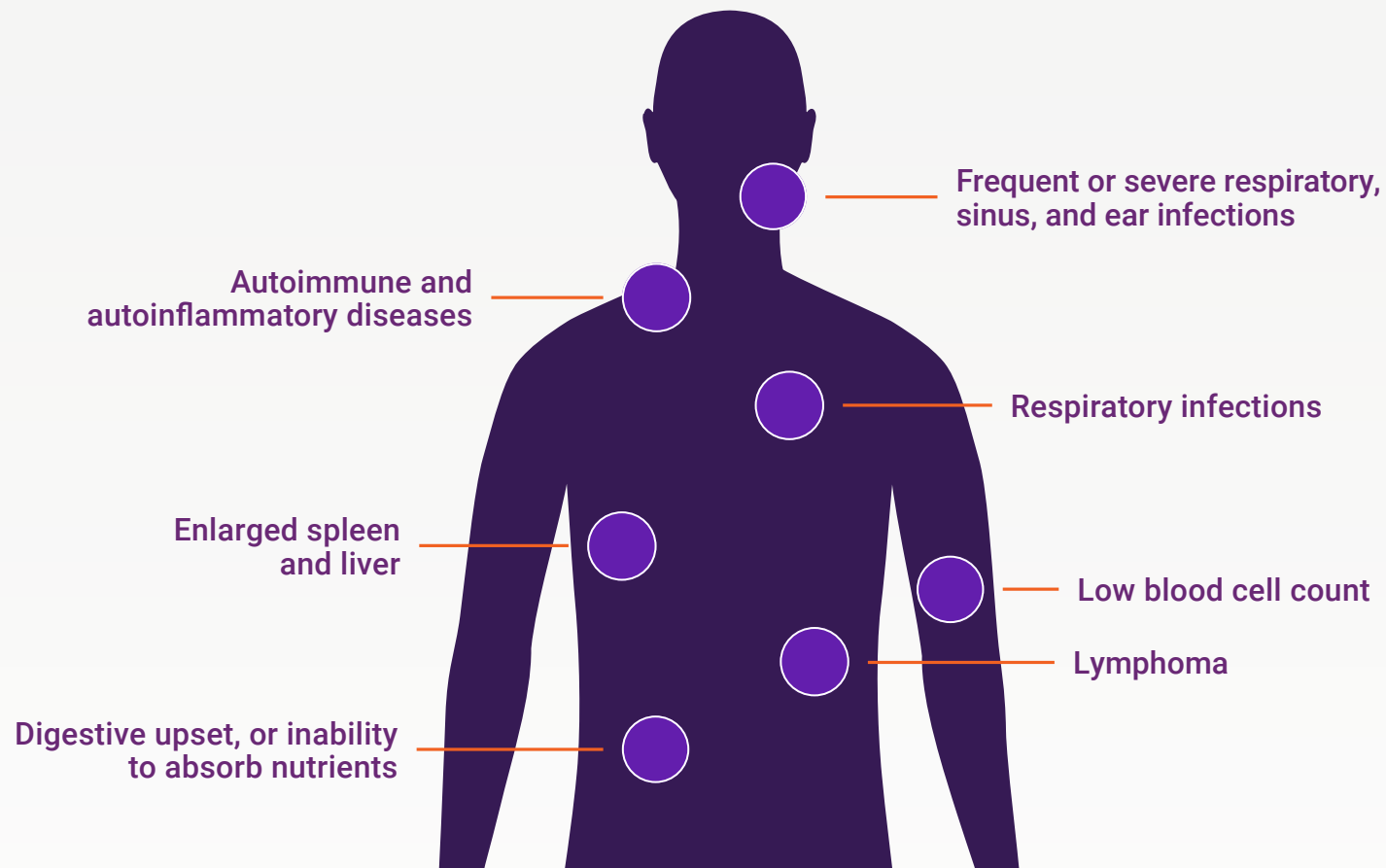
If you plan to become pregnant, continue taking your birth control for one week after stopping JOENJA. Talk to your doctor about what type of birth control method is right for you while taking JOENJA.

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People with APDS can suffer from a wide range of signs and symptoms



Select Safety Information (cont.)

It is not known if JOENJA passes into your breast milk. Talk to your doctor about the best way to feed your baby if you take JOENJA.

Please see additional Important Safety Information throughout.

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Diagnosing APDS

Genetic testing can help your doctor diagnose APDS

APDS symptoms are not the same for everyone, even within members of the same family, which can make it difficult to diagnose.

A genetic test can help confirm if you or a family member has APDS.

If you have been diagnosed with APDS, it's important for your relatives to also get tested. Genetic testing is available for eligible family members for whom your doctor requests a test.



Ask about sponsored genetic testing

Some patients may be eligible for sponsored genetic testing and counseling. **Genetic testing can help inform an APDS diagnosis.** Ask your doctor if you qualify.

Tyler, patient living with APDS



Sometimes, a patient does not inherit APDS but is **simply born with the condition**. This has been reported to happen in about 20% of people with APDS.

If one parent is affected, there is a
50%
chance that their child will inherit the disease

Select Safety Information (cont.)

Tell your healthcare provider and pharmacist about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. JOENJA and certain other medicines may affect each other.

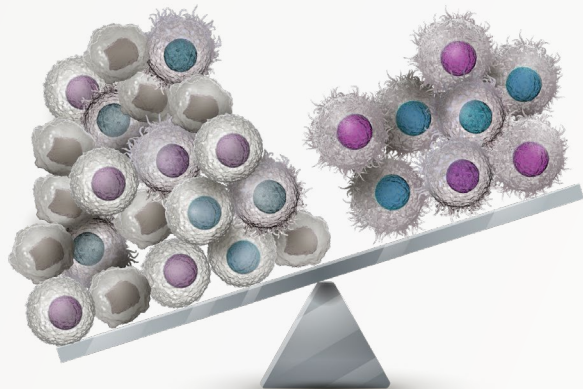
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Joenja works to help restore balance to the immune system

Joenja showed meaningful results across all age groups studied



Immature cells

Functional cells

This is a graphical representation of a complex biological process.

In people with APDS, a genetic variant causes an imbalance of functional and immature B and T cells. This makes it difficult for the body to fight infections and may cause APDS symptoms.

Joenja works to treat APDS at the source of the imbalance in your immune system caused by PI3K delta hyperactivity.

“Starting Joenja felt like a new chapter in my life, which led to controlling my symptoms a lot better. And it also led to less hospital visits for me.”

— Mariana, patient living with APDS

As with all medications, individual patient experiences may vary.



Select Safety Information (cont.)

Tell your healthcare provider if you are scheduled to receive an immunization (vaccine). JOENJA may affect how well a vaccine works.

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How Joenja was studied



Duration

12 weeks



Population

31 participants



Age

12 years and older



Dosing

Each participant took a fixed 70-mg dosage of Joenja or placebo twice a day



Conditions

The participants, sponsor staff, and trial staff did not know what treatment each participant took. This prevented participants from influencing some of the results

Select Safety Information (cont.)

Discontinue JOENJA and seek immediate medical attention if you develop any signs and symptoms of serious allergic reactions including itching, skin redness, hives, rash, difficulty breathing, or swallowing.

Please see additional Important Safety Information throughout.

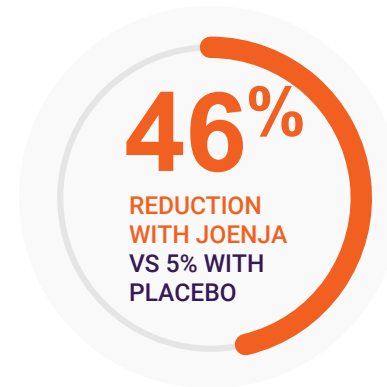
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The size of the most swollen lymph nodes was reduced at week 12

- The size of swollen lymph nodes for each person in the trial was studied before and after treatment
- The results showed that lymph nodes shrank significantly more in people taking Joenja vs those receiving placebo

Reduction of swollen lymph nodes^{*†‡§}



That is an ~8x greater improvement
with Joenja vs placebo

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

†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 immunoglobulin replacement therapy at baseline, and baseline measurement as covariates.

‡Change in index lesion size was measured using the \log_{10} -transformed SPD of the largest lymph nodes (maximum of 6) identified as per the Cheson criteria on CT/MRI.

§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. ANCOVA, analysis of covariance; CT, computed tomography; LS, least squares; MRI, magnetic resonance imaging; SPD, sum of product diameters.

Select Safety Information (cont.)

The most common side effects of JOENJA include headache, inflammation of sinuses, weight gain, and dry, itchy, and inflamed skin (eczema). These are not all the possible side effects of JOENJA. Tell your healthcare provider about any side effect that bothers you or does not go away.

You can also report negative side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch, or Pharming Medical Affairs at (800) 930-5221.

Please see additional Important Safety Information throughout.

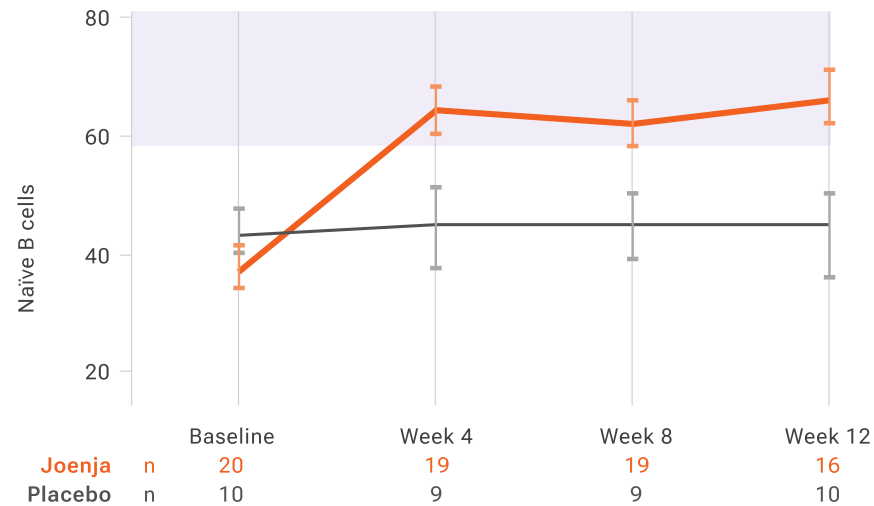
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Increase in functioning B cells that was maintained over 12 weeks

- Some participants began the trial with fewer than 48% of their B cells functioning properly*
- The increase in functional B cells helped restore balance to the immune system†
- B cells are a type of white blood cell that help the body fight infections

Absolute percentage of naïve B cells over time*



Functional B-cell levels returned to normal range within 4 weeks and remained steady through week 12‡

*Normal range for percentage of naïve B cells indicated by shaded bar in graph.

†Cell surface markers used to distinguish naïve 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 was missing, the existing value was used.

‡The analysis at week 12 excluded 2 patients from each treatment group due to protocol deviations, 5 Joenja patients and 3 placebo patients with $\geq 48\%$ naïve B cells at baseline, 5 Joenja patients with no day 85 measurement, and 1 Joenja patient with no baseline measurement.

Select Safety Information (cont.)











Tell your healthcare provider if you are pregnant or plan to become pregnant. JOENJA may harm your unborn baby. Your healthcare provider will do a pregnancy test before you start receiving JOENJA.

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Possible side effects of Joenja



	Joenja 21 participants n (%)	Placebo 10 participants n (%)
 Headache	5 (24%)	2 (20%)
 Sinus infection	4 (19%)	0 (0%)
 Skin rash	3 (14%)	0 (0%)
 Elevated heart rate	2 (10%)	0 (0%)
 Diarrhea	2 (10%)	0 (0%)
 Fatigue	2 (10%)	1 (10%)
 Fever	2 (10%)	0 (0%)
 Back pain	2 (10%)	0 (0%)
 Neck pain	2 (10%)	0 (0%)
 Hair loss	2 (10%)	0 (0%)

If you have concerns or would like to know more about potential side effects, please talk with your doctor about Joenja.

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Use effective birth control to prevent pregnancy while taking JOENJA and for one week after you stop taking JOENJA. If you plan to become pregnant, continue taking your birth control for one week after stopping JOENJA. Talk to your doctor about what type of birth control method is right for you while taking JOENJA.

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Take Joenja twice a day, at home or on the go, anywhere

- Take Joenja **exactly as your healthcare provider** tells you to take it
- Take one Joenja **tablet in the morning**, wait about 12 hours, and take the **second tablet**. Joenja can be taken with or without food
- If you miss a dose of Joenja by more than 6 hours, **take the next dose at your usual time**
- If vomiting occurs within 1 hour after taking Joenja, take another tablet as soon as you are able
- If vomiting occurs more than 1 hour after dosing, wait and take the next dose at the usual time



**Even if Joenja makes you feel better,
do not stop taking Joenja unless directed
by your healthcare provider.**

**Always speak with your healthcare provider
before making any treatment decisions.**

Select Safety Information (cont.)

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Supporting you
throughout your
treatment journey

“My ACE helped find a doctor
in my area who specialized in
treating APDS, and also helped
me navigate insurance to ensure
I didn’t run out of medication.”

— Liam, patient living
with APDS



APDS Clinical Educators (ACEs)*

Your dedicated ACE provides one-on-one support with APDS and genetic testing education, and can connect you with helpful resources.

Meet your personal ACE and get the conversation started.

- Call 1-877-796-APDS (2737)
- 8 AM-8 PM ET, Monday-Friday



APDS Assist Specialty Pharmacists

Specialty Pharmacists can help answer questions about prescription coordination and fulfillment, prior authorization, and taking medication as prescribed.



APDS Assist Care Coordinators

Your Care Coordinator is dedicated to supporting you with insurance coverage/access needs and information on the Joenja Starter program.



Connect with
APDS Assist
Sign up for updates

*Please note that the APDS Assist team does not offer medical or treatment advice or replace discussions with your physician, who will always be your primary source of information.

Select Safety Information (cont.)

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Meet our community

Having a rare disease like APDS may feel like you're the only one with it, but you are not alone.

Meet our patient ambassadors and hear about their journey to diagnosis and Joenja.



Visit [Joenja.com](https://joenja.com) to hear their stories

“You're not alone and don't be afraid. It will get better. It's just going to take some time.”

— Tyrek, patient living with APDS

Annie (patient living with APDS), Tyler (patient living with APDS), Mariana (patient living with APDS), Liam (patient living with APDS), Tyrek (patient living with APDS), and Twana (caregiver)

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