Soar where life takes you

Joenja is the **first** and **only** FDA-approved treatment for APDS that works on the source of the disease itself, **not just the symptoms**.

APDS, activated PI3K delta syndrome.

What's inside?

WHAT IS APDS?

DIAGNOSING APDS

WHAT IS JOENJA?

JOENJA RESULTS

TAKING JOENJA

PATIENT SUPPORT

Not actual patients.

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

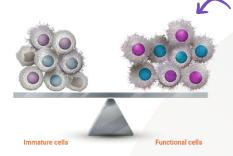
Tell your healthcare provider if you are pregnant or plan to become pregnant. JOENJA may harm your unborn baby. Your healthcare provider will do a pregnancy test before you start receiving JOENJA.

Please see additional Important Safety Information throughout. For more information, please see the <u>full Prescribing Information</u>.



What is **APDS**?

APDS is a rare, inherited disease*



Imature cells

This is a graphical representation of a complex biological process.

In people without APDS.

the PI3K delta pathway regulates the development, growth, and division of white blood cells (made up of B and T cells). These white blood cells can help the body fight infections. When the PI3K delta pathway is working properly, there is a healthy **balance** of immature and functional cells.

In people with APDS,

a genetic variant causes the PI3K delta pathway to be hyperactive. This means the development of B and T cells can be altered and create an **imbalance** of immature and functional cells. This makes it difficult for the body to fight infections and is thought to cause APDS symptoms.

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

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

- Frequent and severe respiratory tract infections, sinus infections, or ear infections
- Swollen lymph nodes
- Enlarged spleen and liver
- Diarrhea, abdominal pain, cramping, blood in stool, or inability to absorb nutrients
- Low blood cell count (red blood cells, white blood cells, platelets), which can make you fatigued or result in infections
- Difficulty breathing, coughing up phlegm (mucus), wheezing, and chest pain, which may lead to permanent lung damage over time
- Lymphoma (a type of blood cancer that affects the lymph nodes)

APDS is a progressive disease

If left untreated, it could continue to progress, or worsen, over time. This could lead to permanent, life-threatening organ damage or lymphoma.



Diagnosing **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, your relatives should speak with their healthcare provider about getting a genetic test.

If one parent is affected, there is a

50% chance that their child will inherit the disease 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.

Mother with APDS

It's important to test for APDS

Not actual patients.

Son

Son with

A confirmed diagnosis can help you or a family member get APDS under control. **Make sure to talk to your healthcare provider about genetic testing.**



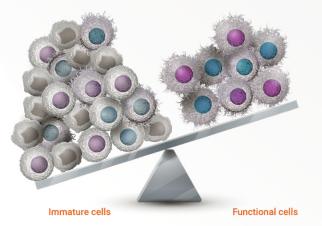
Select Safety Information (cont.)

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.

Please see additional Important Safety Information throughout. For more information, please see the <u>full Prescribing Information</u>. Father

Joenja is the **first** and **only** FDA-approved treatment for APDS

Joenja works on the source of the disease itself, not just the symptoms



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 control the hyperactive PI3K delta pathway, which helps to rebalance the immune system.

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. For more information, please see the <u>full Prescribing Information</u>.

Focus on the things you love

Joenja can help get your APDS under control

Not actual patients.



Joenja works to help **restore balance to your immune system**

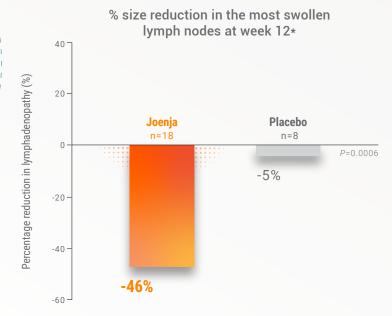
Lymph nodes before

How Joenja was studied

- Joenja was studied in a clinical trial for a period of 12 weeks, involving 31 participants
- Each participant took a fixed 70 mg dose of Joenja or placebo twice a day
- The participants, sponsor staff, and trial staff did not know what treatment each participant took. This prevented participants from influencing some of the results

In the clinical trial, patients taking Joenja saw their most swollen lymph nodes decrease in size

The size of the participants' most swollen lymph nodes was studied before and after treatment. Overall, these lymph nodes shrank more in participants who took Joenja than those who took placebo. This was a meaningful difference.



Reduction computed based on estimates for the adjusted mean changes

*Improvement in lymphoproliferation as measured by a change from baseline in lymphadenopathy measured by loq,,-transformed SPD of index lymph nodes.

Lymph nodes

after

Index lymph nodes were the largest 6 or fewer lymph nodes as measured by MRI/CT imaging at baseline and were selected per the Cheson criteria.

The ANCOVA model was used with treatment as a fixed effect and log₁₀-transformed baseline as a covariate for index and non-index lesions. The use of both glucocorticoids and IV Ig at baseline was included as categorical (yes/no) covariates.

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; Ig, immune globulin; IV, intravenous; SPD, sum of product diameters.

Select Safety Information (cont.)

Tell your healthcare provider 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.

Please see additional Important Safety Information throughout. For more information, please see the full Prescribing Information.



Joenja works to help **restore balance** to your immune system

Patients in the clinical trial also experienced an increase in functioning B cells

Absolute percentage of naïve B cells over time



*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 value was missing, the existing value was used.

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

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

Some participants began the trial with fewer than 48% of their B cells functioning properly.* This graph shows the average change in the percentage of functional B cells in these participants' blood from before treatment through 12 weeks of treatment.⁺ Not an actual patient. SECONDARY END POINT: Patients in the clinical trial with an

feren

enlarged spleen also saw an average 27% decrease in the size of their spleen

LEARN MORE

See additional safety findings from an ongoing open-label extension study of Joenja

LEARN MORE

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.

Please see additional Important Safety Information throughout. For more information, please see the full Prescribing Information.



Taking Joenja is as **easy as...**



Establish a treatment routine with Joenja

- Take Joenja around 12 hours apart at a typically convenient time
- Set 2 reminders, one in the morning and another at night
- Keep your Joenja tablets in the same place so you always know where they are

Joenja^{*} leniolisib) tablets **70 mg**

Take Joenja exactly as your healthcare provider tells you to take it

Never Miss a dose with the free Joenja reminder tone!

DOWNLOAD TONE

Learn More in the Joenja

administration guide

DOWNLOAD NOW

Select Safety Information (cont.)

1 tablet 2X a Day

Know the medicines you take. Keep a list of your medicines and show it to your healthcare provider and pharmacist when you get a new medicine.



Please see additional Important Safety Information throughout. For more information, please see the <u>full Prescribing Information</u>.



Opening doors to help you move forward

We're here to help

- Understand and navigate our program services
- Understand your insurance requirements for Joenja
- Connect you with additional resources, including our ACEs

Connect with an APDS Assist Care Coordinator

Call 1-877-796-APDS (2737)
8 ам-8 рм ЕТ, Monday-Friday







APDS Clinical Educators (ACEs)

Here to support you throughout your journey with Joenja. Your dedicated ACE can provide education and available resources, either live or by phone.

APDS Assist Specialty Pharmacists

Provide you access to a Specialty Pharmacist with deep knowledge about rare disease treatment. They are available to help answer questions about Joenja.

APDS Assist Care Coordinators

Here to help you navigate coverage, access, and financial support options when you are prescribed Joenja. They are also able to connect you with your ACE.

Please note that ACEs do not offer medical treatment advice or replace discussions with your physician, who will always be your primary source of information.

Select Safety Information (cont.)

The most common side effects of JOENJA include headache, inflammation of sinuses, 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 <u>www.fda.gov/medwatch</u>, or Pharming Medical Affairs at (800) 930-5221.

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