



PATIENT & CAREGIVER EDUCATION

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This information from UpToDate® Lexidrug™ explains what you need to know about this medication, including what it's used for, how to take it, its side effects, and when to call your healthcare provider.

What is this drug used for?

- It is used to treat people with Wiskott-Aldrich Syndrome (WAS).
- This drug is used as part of a treatment plan. Be sure you know about the warnings, benefits, and risks of the other parts of the treatment plan. If you have questions, talk with the doctor.

What do I need to tell my doctor BEFORE I take this drug?

- If you are allergic to this drug; any part of this drug; or any other drugs, foods, or substances. Tell your doctor about the allergy and what signs you had.
- If you have had a stem cell transplant or stem cell gene therapy.
- If you are taking a drug for certain viruses (HIV, HTLV).

Do not take one of these drugs within 1 month before mobilization or after treatment with this drug until your doctor tells you to.

- If you are pregnant or may be pregnant. Do not take this drug if you are pregnant.
- If you are breast-feeding. Do not breast-feed while you take this drug.

This is not a list of all drugs or health problems that interact with this drug.

Tell your doctor and pharmacist about all of your drugs (prescription or OTC, natural products, vitamins) and health problems. You must check to make sure that it is safe for you to take this drug with all of your drugs and health problems. Do not start, stop, or change the dose of any drug without checking with your doctor.

What are some things I need to know or do while I take this drug?

- Tell all of your health care providers that you take this drug. This includes your doctors, nurses, pharmacists, and dentists.
- Have your blood work and other lab tests checked as you have been told by your doctor.
- Talk with your doctor if you are planning to get a vaccine

after treatment with this drug. Some vaccines need to be avoided for some time after treatment with this drug.

- Low blood cell counts have happened with this drug and the treatment plan. This may last for several weeks. If blood cell counts get very low, this can lead to bleeding problems, infections, or anemia. If you have questions, talk with the doctor.
- You may have more chance of getting an infection. Wash hands often. Stay away from people with infections, colds, or flu.
- You may bleed more easily. Be careful and avoid injury. Use a soft toothbrush and an electric razor.
- If you need to be tested for HIV after getting this drug, talk with your doctor. False test results have happened with some types of HIV tests.
- Do not donate blood, organs, tissues, or cells at any time after getting this drug.
- This drug is made using substances that come from humans and cows. To lower the risk of disease, this drug is tested to see if it carries infection. If you have questions, talk with the doctor.
- The chance of cancer is higher after using this drug. You will need to be watched closely for the rest of your life. If you have questions, talk with your doctor.

- Talk with the doctor about how drugs used for conditioning may affect being able to get pregnant or father a child later in life.
- A pregnancy test will be done to show that you are NOT pregnant before starting this drug.
- If you may become pregnant, use birth control before starting this drug, while taking it, and for at least 6 months after taking it. Ask your doctor when to start using birth control and what kind to use. If you get pregnant, call your doctor right away.
- If your sex partner may become pregnant, use birth control before starting this drug, while taking it, and for at least 6 months after taking it. Ask your doctor when to start using birth control and what kind to use. If your sex partner gets pregnant, call the doctor right away.
- If you plan to get pregnant or breast-feed after getting this drug, talk with your doctor.

What are some side effects that I need to call my doctor about right away?

WARNING/CAUTION: Even though it may be rare, some people may have very bad and sometimes deadly side effects when taking a drug. Tell your doctor or get medical help right away if you have any of the following signs or symptoms that may be related to a very bad side effect:

- Signs of an allergic reaction, like rash; hives; itching; red, swollen, blistered, or peeling skin with or without fever; wheezing; tightness in the chest or throat; trouble breathing, swallowing, or talking; unusual hoarseness; or swelling of the mouth, face, lips, tongue, or throat.
- Signs of infection like fever, chills, very bad sore throat, ear or sinus pain, cough, more sputum or change in color of sputum, pain with passing urine, mouth sores, or wound that will not heal.
- Signs of bleeding like throwing up or coughing up blood; vomit that looks like coffee grounds; blood in the urine; black, red, or tarry stools; bleeding from the gums; abnormal vaginal bleeding; bruises without a cause or that get bigger; or bleeding you cannot stop.
- Signs of liver problems like dark urine, tiredness, decreased appetite, upset stomach or stomach pain, light-colored stools, throwing up, or yellow skin or eyes.
- Signs of electrolyte problems like mood changes; confusion; muscle pain, cramps, or spasms; weakness; shakiness; change in balance; an abnormal heartbeat; seizures; loss of appetite; or severe upset stomach or throwing up.
- Feeling very tired or weak.
- Pinpoint red spots on the skin.

- Swollen gland.

What are some other side effects of this drug?

All drugs may cause side effects. However, many people have no side effects or only have minor side effects. Call your doctor or get medical help if any of these side effects or any other side effects bother you or do not go away:

- Diarrhea or constipation.
- Throwing up.
- Mouth irritation or mouth sores.
- Stomach pain.
- Headache.
- Runny nose.
- Mouth or throat pain or irritation.

These are not all of the side effects that may occur. If you have questions about side effects, call your doctor. Call your doctor for medical advice about side effects.

You may report side effects to your national health agency.

You may report side effects to the FDA at 1- 800-332-1088. You may also report side effects at <https://www.fda.gov/medwatch>.

How is this drug best taken?

Use this drug as ordered by your doctor. Read all information given to you. Follow all instructions closely.

- It is given as an infusion into a vein over a period of time.
- Other drugs may be given before this drug to help avoid side effects.

What do I do if I miss a dose?

- This drug will be given in a health care setting.

How do I store and/or throw out this drug?

- This drug will be given in a healthcare setting. You will not store it at home.

General drug facts

- If your symptoms or health problems do not get better or if they become worse, call your doctor.
- Do not share your drugs with others and do not take anyone else's drugs.
- Keep all drugs in a safe place. Keep all drugs out of the reach of children and pets.
- Throw away unused or expired drugs. Do not flush down a toilet or pour down a drain unless you are told to do so. Check with your pharmacist if you have questions

about the best way to throw out drugs. There may be drug take-back programs in your area.

- Some drugs may have another patient information leaflet. If you have any questions about this drug, please talk with your doctor, nurse, pharmacist, or other health care provider.
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- If you think there has been an overdose, call your poison control center or get medical care right away. Be ready to tell or show what was taken, how much, and when it happened.

Consumer Information Use and Disclaimer

This generalized information is a limited summary of diagnosis, treatment, and/or medication information. It is not meant to be comprehensive and should be used as a tool to help the user understand and/or assess potential diagnostic and treatment options. It does NOT include all information about conditions, treatments, medications, side effects, or risks that may apply to a specific patient. It is not intended to be medical advice or a substitute for the medical advice, diagnosis, or treatment of a health care

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If you have questions or concerns, contact your healthcare provider. A member of your care team will answer Monday through Friday from 9 a.m. to 5 p.m. Outside those hours, you can leave a message or talk with another MSK provider. There is always a doctor or nurse on call. If you're not sure how to reach your healthcare provider, call 212-639-2000.

For more resources, visit www.mskcc.org/pe to search our virtual library.

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