PATIENT & CAREGIVER EDUCATION

Etuvetidigene Autotemcel

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.

Brand Names: US

Waskyra

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 the doctor BEFORE my child takes this drug?

- If your child is allergic to this drug; any part of this drug; or any other drugs, foods, or substances. Tell the doctor about the allergy and what signs your child had.
- If your child has had a stem cell transplant or stem cell gene therapy.
- If your child is taking a drug for certain viruses (HIV, HTLV). Do not give one of these drugs within 1 month before mobilization or after treatment with this drug until the doctor tells you to.

If your child is pregnant or breast-feeding a baby:

- Do not give this drug to your child during pregnancy.
- If your child is breast-feeding a baby. Be sure your child does not breast-feed while taking this drug.

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

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

What are some things I need to know or do while my child takes this drug?

- Tell all of your child's health care providers that your child is taking this drug. This includes your child's doctors, nurses, pharmacists, and dentists.
- Have your child's blood work and other lab tests checked as you have been told by the doctor.
- Talk with the doctor if your child is going 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.
- Your child may have more chance of getting infections. Have your child wash hands often. Avoid crowds and people with infections, colds, or flu.
- Your child may bleed more easily. Make sure your child is careful and avoids injury. Be sure your child has a soft toothbrush.
- If your child needs to be tested for HIV after getting this drug, talk with the doctor. False test results have happened with some types of HIV tests.

- Be sure your child does 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. Your child will need to be watched closely for the rest of their life. If you have questions, talk with the 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.
- If your child is of childbearing age, a pregnancy test will need to be done before starting this drug to make sure your child is not pregnant.

If your child is or may be sexually active:

- If your child may become pregnant, have your child use birth control while taking this drug and for at least 6 months after the last dose. If your child becomes pregnant, call the doctor right away.
- If your child's sex partner may become pregnant, have them use birth control while taking this drug and for at least 6 months after the last dose. If your child's partner gets pregnant, call the doctor right away.
- If your child plans to get pregnant or breast-feed a baby after getting this drug, talk with the doctor.

What are some side effects that I need to call my child's 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 child's doctor or get medical help right away if your child has 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 child's doctor or get medical help if any of these side effects or any other side effects bother your child 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 child's doctor. Call your child's doctor for medical advice about side effects.

You may report side effects to your national health agency.

How is this drug best given?

Give this drug as ordered by your child's 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 my child misses 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 child's symptoms or health problems do not get better or if they become worse, call your child's doctor.
- Do not share your child's drug with others and do not give anyone else's drug to your child.
- 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 child's doctor, nurse, pharmacist, or other health care provider.
- 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.

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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 provider based on the health care provider's examination and assessment of a patient's specific and unique circumstances. Patients must speak with a health care provider for complete information about their health, medical questions, and treatment options, including any risks or benefits regarding use of medications. This information does not endorse any treatments or medications as safe, effective, or approved for treating a specific patient. UpToDate, Inc. and its affiliates disclaim any warranty or liability relating to this information or the use thereof. The use of this information is governed by the Terms of Use, available at https://www.wolterskluwer.com/en/know/clinical-effectiveness-terms.

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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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