PATIENT & CAREGIVER EDUCATION

Pariglasgene Brecaparvovec

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

Genglycos >87kg; Genglycos 15 to <21kg; Genglycos 21 to <27kg; Genglycos 27 to <33kg; Genglycos 33 to <39kg; Genglycos 39 to <45kg; Genglycos 45 to <51kg; Genglycos 51 to <57kg; Genglycos 57 to <63kg; Genglycos 63 to <69kg; Genglycos 69 to <75kg; Genglycos 75 to <81kg; Genglycos 81 to <87kg

What is this drug used for?

- It is used to help lower daily cornstarch intake in people with glycogen storage disease type 1a (GSD1a).

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 antibodies to AAV8.
- If you have a liver problem, including liver infection.
- If you have had a vaccine in the past month.
- If you are pregnant or may be pregnant. Do not use this drug if you are pregnant.

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.
- High triglyceride levels have happened with this drug. Tell your doctor if you have ever had high triglyceride levels.
- Talk with your doctor before getting any vaccines. Use of some vaccines with this drug may either raise the chance of an infection or make the vaccine not work as well.
- This drug may be passed to others through body fluids after the infusion. This happens through blood, saliva, urine, stools, and semen. Talk with your doctor about how to avoid spreading this drug to others. Be sure to wash your hands well and dispose of anything that comes into contact with body fluids as you have been told. You will need to do this for 3 months after you get this drug.
- There is a chance that this drug could raise the risk of a tumor. Talk with the doctor if you have questions.
- Do not donate semen for at least 6 months after you get this drug.
- If you may become pregnant, use birth control for at least 12 months after you get this drug. If you get pregnant, call your doctor right away.
- If your sex partner may become pregnant, use birth control for 6 months after you get this drug. If your partner gets pregnant, call the doctor right away.

- Tell your doctor if you are breast-feeding. You will need to talk about any risks to your baby.

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 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 a weak adrenal gland like a severe upset stomach or throwing up, severe dizziness or passing out, muscle weakness, feeling very tired, mood changes, decreased appetite, or weight loss.
- Signs of Cushing's syndrome like weight gain in the upper back or belly, moon face, severe headache, or slow healing.
- Signs of high blood sugar like confusion, feeling sleepy, unusual thirst or hunger, passing urine more often, flushing, fast breathing, or breath that smells like fruit.
- Infusion reactions have happened with this drug. Sometimes these can be severe. Tell your doctor right away if you have signs of high or low blood pressure like headache, dizziness, passing out, or change in eyesight. Tell your doctor right away if you have fever, chills, flushing, flu-like signs, trouble breathing, chest tightness, upset stomach, throwing up, stomach pain, or any other bad effect while this drug is being given.

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:

- Pimples (acne).
- Constipation.
- Headache and upset stomach are common with this drug. However, if they happen during the infusion, they may be part of an infusion reaction. Tell your doctor if you have a headache or upset stomach while this drug is being given.

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 will be given with this drug to help avoid side effects.

What do I do if I miss a dose?

- Call your doctor to find out what to do.

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

- If you need to store this drug at home, talk with your doctor, nurse, or pharmacist about how to store it.

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.
- Some drugs may have another patient information leaflet. Check with your pharmacist. If you have any questions about this drug, please talk with your 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.

Consumer Information Use and Disclaimer

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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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