Memorial Sloan Kettering Cancer Center

PATIENT & CAREGIVER EDUCATION

Onasemnogene Abeparvovec

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

Itvisma; Zolgensma

Brand Names: Canada

Zolgensma

Warning

- Liver problems have happened with this drug. Some liver problems have been deadly. The risk of liver problems may be higher if you already have liver problems. You will need to have liver function checked as you have been told by your doctor.

What is this drug used for?

- It is used to treat spinal muscular atrophy.

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 an infection.

- If you are dehydrated or have poor nutrition.
- If you have ever had another drug that has the same drug in it.

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.
- Raised troponin levels (a protein from heart muscle) have happened with this drug. A heart doctor may need to check your heart. If you have questions, talk with the doctor.
- Make sure you are up to date with all your vaccines before treatment with this drug.
- This drug is used with a steroid drug like prednisolone. Talk with your doctor before getting any vaccines.
- Having an infection before or after infusion of this drug could lead to a more serious problem. Caregivers and close contacts need to take steps to prevent infections. These include washing hands often, taking care when coughing or sneezing, and limiting contact. Call the doctor right away if you have signs of infection like coughing, wheezing, sneezing, runny nose, sore throat, fever, chills, ear or sinus pain, more sputum or change in color of sputum, pain with passing urine, mouth sores, or wound that will not heal.
- Take special care when handling and throwing away the patient's stools.

Throw diapers away in household trash sealed in trash bags. Be sure to wash your hands well if you come into contact with body fluids like saliva, mucus, urine, or stools. Do this for 1 month after treatment with this drug. If you have questions, talk with the doctor.

- There is a chance this drug may raise the risk of cancer. Cases of cancer have been seen in people who had been treated with this drug. It is not known if this drug may be the cause. If you have questions, talk with the doctor.
- Do not donate eggs for 6 months after getting this drug.
- Do not donate sperm for 3 months after getting this drug.
- 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 for 6 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 3 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.

- A severe health problem called thrombotic microangiopathy (TMA) has happened with this drug. TMA can be life-threatening or deadly. Call the doctor right away if you have signs like feeling very tired or weak; any unexplained bruising or bleeding; seizures; change in the amount of urine passed; or signs of high blood pressure like severe headache or dizziness, passing out, or change in eyesight.
- Numbness, tingling, prickling, or pain in the arms, hands, legs, or feet have started about 3 weeks after getting this drug. Call the doctor right away if you have any of these effects.

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:

- Throwing up.
- Headache.

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 a shot into the spine.
- A steroid drug like prednisolone will be given before and after this drug to help avoid side effects. Tell the doctor if the steroid drug is not used as you

have been told.

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

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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