

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 your child already has liver problems. Your child will need to have liver function checked as you have been told by your child's doctor.

What is this drug used for?

- It is used to treat spinal muscular atrophy.

What do I need to tell the doctor BEFORE my child takes this drug?

All products:

- 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 an infection.
- If your child is dehydrated or has poor nutrition.
- If your child has ever had another drug that has the same drug in it.

Injection (if given in the vein):

- If your child is a premature newborn. This drug is not for use in certain ages of premature newborns.

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.
- Raised troponin levels (a protein from heart muscle) have happened with this drug. A heart doctor may need to check your child's heart. If you have questions, talk with the doctor.
- Make sure your child is up to date with all vaccines before treatment with this drug.

- This drug is used with a steroid drug like prednisolone. Talk with your child's doctor before your child gets 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 your child has 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.

If your child is or may be sexually active:

- A pregnancy test will be done to show that your child is NOT pregnant before starting this drug. If your child gets pregnant while taking this drug, call the doctor right away.
- If your child may become pregnant, birth control must be used for 6 months after your child gets this drug. If your child gets pregnant, call the doctor right away.
- If your child's sex partner may become pregnant, birth control must be used for 3 months after your child gets this drug. If your child's partner gets pregnant, call the doctor right away.
- Tell the doctor if your child is breast-feeding a baby. You will need to talk about any risks to the baby.

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:

All products:

- 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 your child has 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.

Injection (if given in the vein):

- Infusion reactions have happened during the infusion. Tell your child's doctor right away if your child has rash; itching; throwing up; shortness of breath or other breathing problems; fast, slow, or abnormal heartbeat; signs of low blood pressure like severe dizziness or passing out; or any other bad effects.

Injection (if given into the spine):

- 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 your child has 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 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:

All products:

- Throwing up.

Injection (if given into the spine):

- Headache.

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.

Injection (if given in the vein):

- It is given as an infusion into a vein over a period of time.
- 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.

Injection (if given into the spine):

- 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 my child misses a dose?

- Call your child's 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 child's doctor, nurse, or pharmacist about how to store it.

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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