

What is Xenpozyme?

Xenpozyme is a prescription medicine used to treat Acid Sphingomyelinase Deficiency (ASMD) in patients. It helps with the problems ASMD causes in the body, but not the problems that affect the brain or nerves. The non-proprietary (generic) name is olipudase alfa-rpcp. Xenpozyme is manufactured by Sanofi.

How It Works

ASMD happens when your body doesn't make enough of a natural enzyme called acid sphingomyelinase (ASM). This enzyme usually breaks down a cholesterol-like substance called sphingomyelin. When there isn't enough ASM, sphingomyelin builds up in different parts of the body, like the liver, spleen, and lungs, causing damage.

Xenpozyme is a man-made version of this missing ASM enzyme. It helps your body break down the extra sphingomyelin, which can reduce its buildup and help your organs work better.

Why It Matters

Xenpozyme helps manage the non-neurologic symptoms of ASMD by reducing the buildup of sphingomyelin in your organs. This can lead to improvements in organ size (like the spleen and liver), better lung function, and increased platelet counts. The goal is to help you feel better and improve your overall health.

How to Take Xenpozyme

Form: Liquid solution

Route: Given directly into a vein (intravenous or IV infusion)

Where: You will receive this medicine at a clinic or hospital. After a period of time, some patients may be able to receive infusions in their home.

Food: Not applicable (given by IV)

Dosing Schedule

Your doctor or nurse will determine your dose based on your body weight. The medicine is given slowly through an IV line by a healthcare professional every two weeks. The dose is slowly increased over the first several months of infusions until your clinician determines you have reached a stable dose that is appropriate for you. A sample schedule for increasing the dose is provided below.

Xenpoyzme Dose Escalation Schedule

Time Period	Dose	Frequency	Administration Notes
Weeks 1-2	Starting dose*	Every 2 weeks	Healthcare professional will determine initial dose based on age and size of the individual
Weeks 3-8	Gradual increase*	Every 2 weeks	Dose slowly increased based on tolerance
Weeks 9-16	Further increase*	Every 2 weeks	Continued escalation as appropriate
Week 17+	Target dose*	Every 2 weeks	Maintenance dose individualized for patient

*Specific doses determined by healthcare team based on individual patient weight, age, and treatment response

Important Notes:

- All doses given as IV infusion by healthcare professionals
- Escalation timeline may be adjusted based on individual tolerance
- Your healthcare team will monitor you closely during dose escalation

What If You Miss a Dose?

If you miss an appointment for your infusion, contact your doctor or healthcare team right away to reschedule

Common Side Effects

Reactions during or after the infusion (headache, fever, rash, nausea, vomiting, dizziness, chills, flushing, feeling short of breath) • Stomach pain • Diarrhea • Feeling tired

Important Safety Information

- **Infusion Reactions:** Some people may have reactions during or after the infusion. These can be mild or more serious. Your healthcare team will monitor you for reactions and may manage these reactions and may slow down the infusion or give you other medicines if needed
- **Allergic Reactions:** Serious allergic reactions are possible. Tell your healthcare team immediately if you feel unwell during or after your infusion
- **Antibodies:** Your body might make antibodies (proteins that fight off foreign substances) against olipudase alfa. Your doctor may check for these
- **Pregnancy and Breastfeeding:** Tell your doctor if you are pregnant, planning to become pregnant, or breastfeeding

Who Should NOT Take It?

- Anyone who has had a severe allergic reaction to olipudase alfa or any of its ingredients in the past
- Always tell your doctor about all your health conditions and all the medicines you are taking, including over-the-counter drugs, vitamins, and herbal supplements

Storage & Safety Tips

- This medicine is stored and prepared by your healthcare team at the clinic or hospital. For patients receiving home infusions, some patients may store this medication in their refrigerator.
- Keep all your scheduled appointments for infusions and follow-up visits
- Report any side effects or concerns to your doctor or nurse

Reporting Side Effects

In addition to reporting any side effects to your healthcare providers, you are encouraged to report side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Questions?

Always talk to your doctor or pharmacist if you have questions about your medicine.

If you ever have questions about Xenpozyme - how to take it, side effects, or anything else - please call your doctor or pharmacist.

For more information see xenpozyme.com