



UNDERSTANDING INFUSIONS WITH XENPOZYME

XENPOZYME IS GIVEN AS AN INTRAVENOUS INFUSION ONCE EVERY 2 WEEKS. THIS GUIDE CONTAINS IMPORTANT INFORMATION TO KNOW BEFORE YOU OR YOUR CHILD RECEIVES TREATMENT WITH XENPOZYME.

INDICATION

XENPOZYME[®] (olipudase alfa-rpcp) is indicated for treatment of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients.

IMPORTANT SAFETY INFORMATION

WARNING: SEVERE ALLERGIC REACTIONS

Allergic Reactions Including Anaphylaxis


Allergic reactions, including severe reactions that may be serious or life-threatening (known as anaphylaxis), have occurred during and after XENPOZYME treatment. Tell your healthcare provider right away if you develop any reactions, and seek immediate medical care if severe reactions occur. If a severe allergic reaction occurs, your doctor may decide to discontinue XENPOZYME immediately and provide appropriate medical care. Appropriate medical support measures may be administered, and you may require close observation during and after XENPOZYME administration.

Please see [Important Safety Information](#) and full [Prescribing Information](#) for complete details, including **Boxed WARNING**.

WHAT IS ASMD?

HISTORICALLY KNOWN AS NIEMANN-PICK DISEASE TYPES A, A/B, AND B, ASMD IS AN INHERITED CONDITION WITH MULTIORGAN SYMPTOMS THAT CAN WORSEN OVER TIME

ASMD IS CAUSED BY REDUCED ACTIVITY OF AN ENZYME CALLED ACID SPHINGOMYELINASE (ASM)



Enzymes help your body break down substances.





When you do not have enough ASM, your body cannot adequately break down the sphingomyelin in your cells.

This may lead to a buildup of sphingomyelin in tissues in the body.

ASMD=acid sphingomyelinase deficiency.

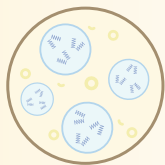
WHAT IS XENPOZYME?

THE FIRST AND ONLY DISEASE-SPECIFIC TREATMENT FOR ASMD (NON-CNS MANIFESTATIONS)

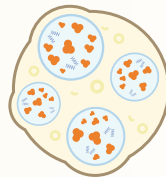
-  XENPOZYME is an enzyme replacement therapy that provides the ASM enzyme that is deficient in people with ASMD.
-  The effectiveness of XENPOZYME has been evaluated in 3 clinical trials including adults and children with ASMD.
-  The safety of XENPOZYME has been evaluated across 3 clinical trials including adults and children with ASMD.
-  XENPOZYME is administered in 2 phases, dose escalation followed by maintenance, with a potential option of home infusion during the maintenance phase. The decision to move to home infusion can only be made after evaluation and recommendation by your prescribing doctor.

HOW DOES XENPOZYME WORK?

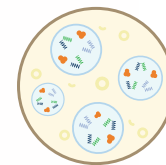
XENPOZYME provides the enzyme that is deficient or missing, helping to reduce the buildup of a substance called sphingomyelin in cells. XENPOZYME does not impact symptoms related to the central nervous system.



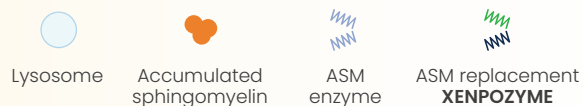
Healthy cell



ASMD causes a buildup of sphingomyelin in the cells.



XENPOZYME replaces the ASM enzyme and helps reduce the buildup of sphingomyelin.



CNS=central nervous system.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Allergic Reactions (Including Anaphylaxis) and Infusion-Associated Reactions (IARs)

See Boxed WARNING for more information. Your doctor may decide to give you antihistamine, anti-fever, and/or steroid medications before your infusions. Signs of allergic reactions and infusion-associated reactions (IARs) included hives, itchy skin, skin redness, rash, swelling underneath the skin, tender bumps under the skin, and localized swelling, as well as headache, vomiting, nausea, fever, and diarrhea.

Reactions may occur during and/or after the infusion. Tell your healthcare provider right away if you experience any reactions. Your healthcare provider may slow or stop the infusion or may lower the next dose.

Please see [Important Safety Information](#) and full [Prescribing Information](#) for complete details, including Boxed WARNING.

HOW TO TAKE XENPOZYME

- XENPOZYME is given as an intravenous infusion once every 2 weeks—the dose is based on body weight.
- XENPOZYME must always be administered by a trained healthcare provider.

BEFORE INITIATING XENPOZYME

- XENPOZYME dosage initiation or escalation, at any time during pregnancy, is not recommended.
- If you are a female of reproductive potential, your doctor will verify your pregnancy status before starting treatment with XENPOZYME.
- Use effective contraception during treatment with XENPOZYME and for 14 days after the last dose if XENPOZYME is discontinued.
- If you are pregnant or plan to become pregnant, tell your doctor right away.
- Your doctor will order a baseline liver enzyme level measurement for you or your child 1 month prior to the start of treatment.
- Prior to infusion, your doctor may decide to pretreat you or your child with anti-fever, anti-allergy, and/or steroid medications.

XENPOZYME TREATMENT OCCURS IN 2 DOSING PHASES TO REDUCE THE RISK OF SIDE EFFECTS AT TREATMENT INITIATION

The first phase is called the dose escalation phase. You or your child will start with a low dose that will gradually increase throughout the dose escalation phase, so the body has time to adjust to XENPOZYME.



- The dose escalation phase lasts at least 14 weeks for adults and at least 16 weeks for children.*
- Gradual dose escalation is essential to reduce the risk of infusion-associated reactions (IARs) and elevated liver enzyme levels when first starting treatment.
- The dose escalation period takes place in a clinical setting to manage for the possibility of severe reactions.

The second phase is called the maintenance phase. After dose escalation, you or your child will reach the maintenance dose. The target maintenance dose is 3 mg/kg once every 2 weeks.



- In people with ASMD, the body is unable to make enough of the ASM enzyme; therefore, it is important to keep taking XENPOZYME every 2 weeks.
- If 3 or more doses are missed, you will have to repeat the dose escalation phase before resuming your maintenance dose.
- Once you or your child starts receiving the maintenance dose, there is a potential option of receiving XENPOZYME at home if the doctor recommends it.

*In clinical trials, all but 1 of the pediatric patients completed the dose escalation up to the target maintenance dose of 3 mg/kg within 22 weeks.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Elevated Transaminases Levels

XENPOZYME may be associated with elevated liver enzymes (known as transaminases) within 24 to 48 hours after infusion. Your doctor should check your liver enzyme levels with a blood test:

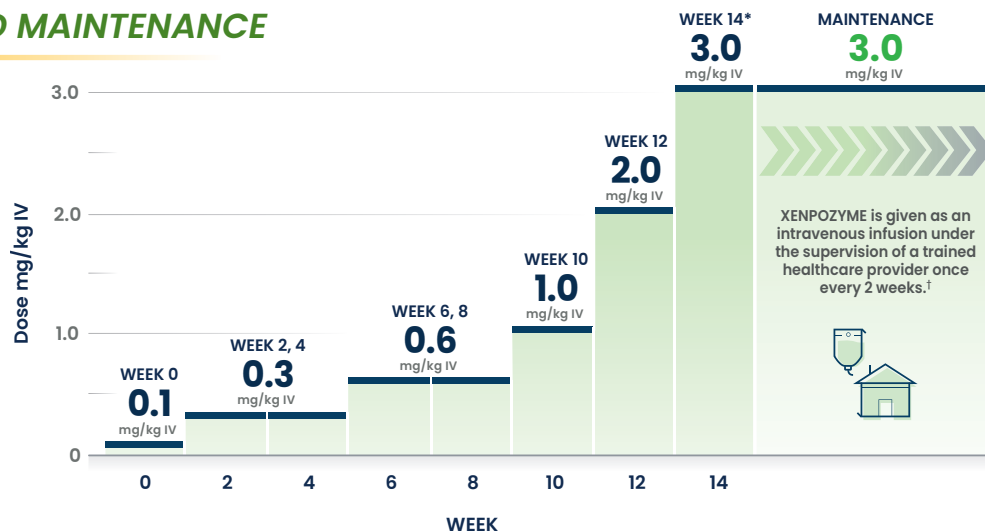
- within one month before starting XENPOZYME;
- within 72 hours before any infusion during the dose escalation phase, or before your next scheduled XENPOZYME infusion if you missed a dose.

Based on the results of your blood tests, your doctor may make changes to your dose or infusion schedule. Upon reaching the recommended maintenance dose, your doctor may continue to monitor your liver enzyme levels.

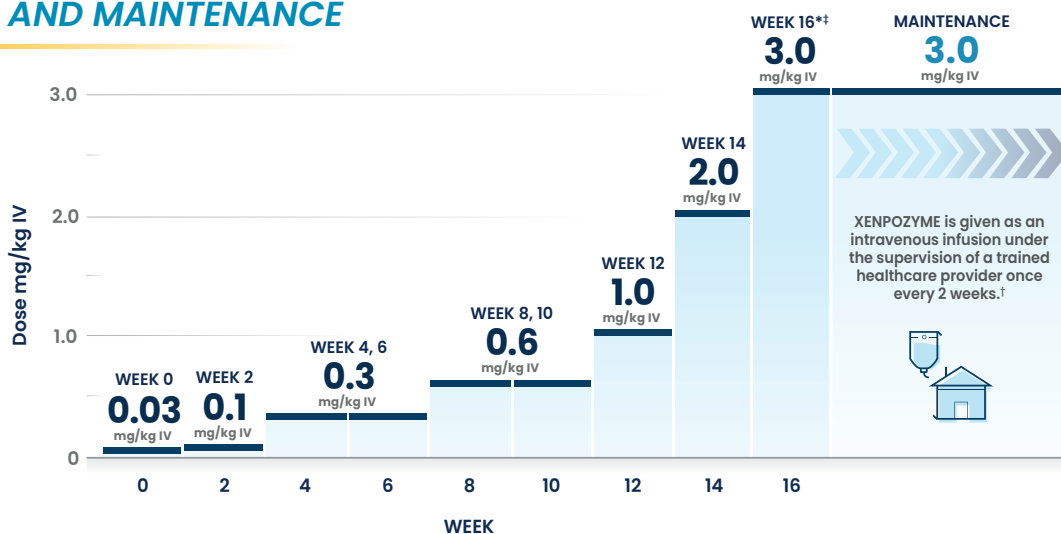
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HOW TO TAKE XENPOZYME

ADULT DOSE ESCALATION AND MAINTENANCE



PEDIATRIC DOSE ESCALATION AND MAINTENANCE



STAY ON TRACK WITH TREATMENT

- Make sure you set aside enough time in your calendar for regular, biweekly infusions. Infusions during the maintenance phase may take up to 3.5–4 hours.
- Be sure to allow enough time on the day of the infusion to get to your appointment.

Check out the [Dosing Adherence Flashcard](#) to help you stay on track.

*The dose escalation phase includes the first 3 mg/kg dose.

†The dose is based on body weight. Once you or your child starts receiving the maintenance dose, there is a potential option of receiving XENPOZYME at home if your doctor recommends it.

‡In clinical trials, all but 1 of the pediatric patients completed the dose escalation up to the target maintenance dose of 3 mg/kg within 22 weeks.



MISSED INFUSIONS: If 3 or more infusions are missed, you or your child will need to return to dose escalation. Dose escalation should take place in a clinical setting.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Risk to Unborn Babies

Starting or increasing the dose of XENPOZYME is not recommended in a pregnant female as it may cause harm (birth defects) to the developing baby. If you are pregnant or plan to become pregnant, tell your doctor right away.

If you are a female of reproductive potential, your doctor will verify your pregnancy status before you start treatment with XENPOZYME. You should use effective contraception during XENPOZYME treatment and for 14 days after your last dose if XENPOZYME is discontinued.

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IF YOU OR YOUR CHILD IS TOLERATING INFUSIONS WELL ON THE MAINTENANCE DOSE, YOUR DOCTOR MAY DECIDE THAT INFUSIONS CAN BE GIVEN AT HOME UNDER THE SUPERVISION OF A TRAINED HEALTHCARE PROVIDER

- The decision to move to home infusions is only made after evaluation and recommendation by your doctor.
- Once in the home setting, in case of missed doses or delayed infusion, the doctor should be contacted.



HOW BEING INFUSED AT HOME IS SIMILAR TO BEING INFUSED IN A CLINICAL SETTING

- XENPOZYME is still prescribed by your doctor.
- XENPOZYME is still administered under the supervision of a trained nurse.

HOW BEING INFUSED AT HOME IS DIFFERENT FROM BEING INFUSED IN A CLINICAL SETTING

- XENPOZYME may be prepared by a pharmacy and shipped to your home OR your nurse will prepare it while they are at your home.
- XENPOZYME and infusion supplies will be received, stored, and possibly ordered by you, unlike the infusions you received at the hospital or infusion center.

IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

- Most frequently reported adverse drug reactions in adults (incidence $\geq 10\%$) were headache, cough, diarrhea, low blood pressure, and redness in the eye.
- Most frequently reported adverse drug reactions in pediatric patients (incidence $\geq 20\%$) were fever, cough, diarrhea, runny nose, abdominal pain, vomiting, headache, hives, nausea, rash, joint pain, itchy skin, fatigue, and sore throat.

PREPARING FOR HOME INFUSIONS



IF YOUR DOCTOR RECOMMENDS RECEIVING XENPOZYME AT HOME, HERE IS WHAT YOU NEED TO KNOW:

- **FIRST:** Your home environment must be able to accommodate the home infusion therapy by providing an appropriate space to receive the infusion and ample storage space for supplies.
 - Unlike infusions in a clinical setting, you will be responsible for receiving, storing, and possibly ordering XENPOZYME and supplies for your infusions.
- **SECOND:** Your doctor will decide the appropriate dose and discuss logistics with you:
 - You will be educated on the risks of home infusions, the logistics of interacting with a home-healthcare agency, and the importance of keeping your regular follow-up appointments with your doctor.
 - XENPOZYME at-home infusions must always be given under the supervision of a trained healthcare provider who will ensure that appropriate medical support measures, including cardiopulmonary resuscitation equipment, are readily available during XENPOZYME administration.



Whether in the clinic or at home, XENPOZYME must always be given as an intravenous infusion under the supervision of a trained healthcare provider—once every 2 weeks.

WARNINGS AND PRECAUTIONS

Allergic Reactions (Including Anaphylaxis) and Infusion-Associated Reactions (IARs)

See Boxed WARNING for more information. Your doctor may decide to give you antihistamine, anti-fever, and/or steroid medications before your infusions. Signs of allergic reactions and infusion-associated reactions (IARs) included hives, itchy skin, skin redness, rash, swelling underneath the skin, tender bumps under the skin, and localized swelling, as well as headache, vomiting, nausea, fever, and diarrhea.

Reactions may occur during and/or after the infusion. Tell your healthcare provider right away if you experience any reactions. Your healthcare provider may slow or stop the infusion or may lower the next dose.

UNDERSTANDING REACTIONS AND SIDE EFFECTS

HYPERSENSITIVITY AND INFUSION-ASSOCIATED REACTIONS (IARs)

- Some adults and children experienced mild, moderate, or severe hypersensitivity reactions with XENPOZYME, meaning their immune systems had an exaggerated response to the medication such as developing hives, redness of the skin, or itchy skin. Your doctor might consider pretreatment with other medications to lessen the chance of a hypersensitivity reaction. The doctor will also monitor you or your child and will be prepared to follow the appropriate course of action if a reaction occurs.
- XENPOZYME is given as an intravenous infusion. Some adults and children experienced side effects in the clinical trials that may have been associated with the XENPOZYME infusion. These are called IARs and they may occur during and after the infusion.

IAR ALERT: During or after an infusion, you or your child may experience a reaction to the infusion, which must be treated immediately.

CONSULT WITH YOUR DOCTOR IF ANY OF THE FOLLOWING SIGNS AND SYMPTOMS APPEAR OR WORSEN AFTER INFUSION.

IN THE CLINICAL TRIAL FOR XENPOZYME, THE MOST FREQUENT ADVERSE REACTIONS IN ≥10% OF ADULTS INCLUDED:

- Headache
- Cough
- Diarrhea
- Low blood pressure
- Redness in the eye

IN CLINICAL TRIALS FOR XENPOZYME, THE MOST FREQUENT ADVERSE REACTIONS IN ≥20% OF CHILDREN INCLUDED:

- Fever
- Cough
- Diarrhea
- Runny nose
- Abdominal pain
- Vomiting
- Headache
- Hives
- Nausea
- Rash
- Joint pain
- Itchy skin
- Fatigue
- Sore throat

LIVER ENZYME ELEVATION

- During the dose escalation phase of XENPOZYME treatment, levels of liver enzymes (called transaminases) can increase.
- Your doctor will check your liver enzymes within 1 month before the start of the treatment with XENPOZYME.
- Your doctor will also check your liver enzymes throughout the dose escalation phase—every 2 weeks, within 3 days before the next infusion. If liver enzymes are elevated during the dose escalation phase, the doctor may delay an infusion, repeat a dose, or lower the dose for the next infusion.
- Testing results will help your doctor decide whether it is safe for you to receive the next dose of XENPOZYME.
- Upon reaching the recommended maintenance dose, testing for liver enzyme levels is recommended to be continued as part of routine clinical management of ASMD.

CERTAIN SIDE EFFECTS: If a severe hypersensitivity reaction or severe IAR occurs, your doctor may stop your infusion immediately and provide appropriate medical care. If a mild or moderate hypersensitivity reaction or IAR occurs, the infusion rate may be slowed or temporarily withheld, and/or the XENPOZYME dose reduced.

DURING THE INFUSION, YOUR HEALTHCARE PROVIDER WILL MONITOR FOR IARs.
If you or your child is having a reaction to the infusion, tell them right away.

careconnect™ PERSONALIZED SUPPORT SERVICES

CareConnect Personalized Support Services provides personalized support to eligible individuals. From diagnosis and treatment journey to insurance coverage and community connections, CareConnect has someone available to help.



› **SPEAKING ASMD**

ASMD can be hard to understand or explain. CareConnect offers disease and treatment education—both in-person and virtually—for you, family members, and important members of your community.



› **CARE COORDINATION**

It is important to stay connected to ASMD healthcare providers. CareConnect can assist you in finding appropriate medical facilities. If you relocate or travel, CareConnect can also help with logistics.



› **INSURANCE COVERAGE**

It is important to understand insurance coverage. CareConnect can help eligible patients navigate the healthcare system and know more about their options.



› **FINANCIAL ASSISTANCE**

CareConnect can help eligible patients understand and manage treatment costs, follow up on insurance claims, and find assistance programs.

Access personalized support and resources at [CareConnectPSS.com](https://www.CareConnectPSS.com)

or call **1-800-745-4447** (toll free), Option 3,

or email Info@CareConnectPSS.com.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

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INDICATION AND IMPORTANT SAFETY INFORMATION



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IMPORTANT REMINDERS ABOUT TREATMENT WITH XENPOZYME



- › Contact your healthcare team and prescriber immediately if you do not feel well or experience any side effects during or after infusion.
- › Report any change in pregnancy status (if applicable) to your healthcare team and prescriber.
- › If you receive your infusion at home, discuss and report any missed doses to your healthcare team and prescriber.
- › Schedule regular follow-up consultations.

For any questions relating to CareConnect, please contact 1-800-745-4447, Option 3, email Info@CareConnectPSS.com, or visit [CareConnectPSS.com](https://www.CareConnectPSS.com).

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LEARN MORE AT [XENPOZYME.COM](https://www.XENPOZYME.COM)

Xenpozyme®
(olipudase alfa-rpcp)
For Injection, 20 mg

sanofi

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