HOME INFUSION WITH XENPOZYME™

A GUIDE FOR TRANSITIONING TO HOME INFUSION

INDICATIONS AND USAGE
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 HYPERSENSITIVITY REACTIONS

Hypersensitivity Reactions Including Anaphylaxis

Hypersensitivity reactions, including severe reactions known as anaphylaxis, may occur during and after XENPOZYME treatment. You should seek immediate medical care if hypersensitivity reactions (including anaphylaxis) occur. If a severe hypersensitivity 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 throughout this brochure and full Prescribing Information, including Boxed WARNING for complete details.
WHAT IS XENPOZYME?

XENPOZYME IS THE FIRST AND ONLY DISEASE-SPECIFIC TREATMENT FOR NON–CENTRAL NERVOUS SYSTEM (NON–CNS) MANIFESTATIONS OF ACID SPHINGOMYELINASE DEFICIENCY (ASMD)

WHEN TRANSITIONING TO HOME INFUSION, IT IS IMPORTANT TO KNOW WHAT IS AHEAD SO YOU CAN BE PREPARED.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions Including Anaphylaxis

Your doctor may decide to give you antihistamine, anti-fever, and/or steroid medications before your infusions.

• If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, your doctor should discontinue XENPOZYME immediately and initiate appropriate medical treatment.

• If a mild or moderate hypersensitivity reaction occurs, your doctor may adjust or temporarily withhold your infusion rate or dose of XENPOZYME.

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IS HOME INFUSION AN OPTION?

- You or your child has completed the dose escalation phase of treatment, during which your doctor started you or your child on a low dose of XENPOZYME and gradually escalated the dose over at least 14 weeks (for adults) or at least 16 weeks (for children) to the target maintenance dose of 3 mg/kg.

- If you or your child is tolerating infusions well on the maintenance dose, your doctor may decide that treatment can be given at home under the supervision of a nurse.

HOW TO GET STARTED WITH HOME INFUSION

STEP 1: GET HELP FINDING A HOME INFUSION AGENCY

Your CareConnectPSS® Case Manager and/or Patient Education Liaison (PEL) can assist in finding a home health agency that is capable of providing home infusion.

STEP 2: RECEIVE AND STORE SUPPLIES

Unlike infusions in a clinical setting, you will be responsible for receiving, storing, and possibly ordering XENPOZYME and supplies needed for your home infusions. Before your first home infusion occurs, you will be mailed a large box of medical supplies and a smaller box with XENPOZYME that will be necessary for your infusions.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Hypersensitivity Reactions Including Anaphylaxis (continued)

Hypersensitivity reactions, including anaphylaxis, have been reported in olipudase alfa-treated patients.

- Signs of hypersensitivity reactions in adults included hives, itchy skin, skin redness, rash, swelling underneath the skin, and tender bumps under the skin.

- Hypersensitivity reactions in pediatric patients included hives, itchy skin, rash, and localized swelling.

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HOW IS RECEIVING INFUSIONS AT HOME DIFFERENT FROM RECEIVING INFUSIONS IN A CLINICAL SETTING?

HOW IT IS SIMILAR:

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

HOW IT IS DIFFERENT:

• XENPOZYME may be prepared by a pharmacy and shipped to your home OR your nurse may 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

WARNINGS AND PRECAUTIONS (CONTINUED)

Infusion-Associated Reactions

Your doctor may decide to give you antihistamine, anti-fever, and/or steroid medications before your infusions to reduce the risk of infusion-associated reactions (IARs). However, IARs may still occur after receiving these medications.

• If severe IARs occur, your doctor should discontinue XENPOZYME immediately and initiate appropriate medical treatment.
• If a mild or moderate IAR occurs, your doctor may adjust or temporarily withhold your infusion rate or dose of XENPOZYME.

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HOW IMPORTANT IS IT TO ADHERE TO THE HOME INFUSION SCHEDULE?

IT IS VERY IMPORTANT TO FOLLOW YOUR INFUSION SCHEDULE

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 to continue to help reduce the buildup of sphingomyelin in the cells.

• Contact your doctor for help in scheduling a time and day of the week that work best for you or your child to stay on track.

If you or your child misses 3 or more infusions in a row, returning to the dose escalation phase will be required before resuming the maintenance dose.

• You or your child might need to go back to the hospital or infusion center to receive infusions until you reach the maintenance dose again.

• There may also be additional logistical delays related to missed doses. If you miss a scheduled dose, contact your doctor to discuss returning to your regular dosing schedule.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Infusion-Associated Reactions (continued)

The most frequent IARs in:

• adult patients were headache, rash, vomiting, and hives;

• pediatric patients were hives, swelling, headache, nausea, fever, and vomiting.

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PREPARING FOR HOME INFUSION

IF YOUR DOCTOR GIVES YOU THE OPTION OF 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 treatment at home, the associated risks, and which medical personnel will administer the home infusion.
   • 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.

BEFORE YOUR FIRST HOME INFUSION, YOU WILL RECEIVE 2 BOXES IN THE MAIL:

1. A large box of medical supplies
2. A smaller box with XENPOZYME that will be necessary for your infusion

WHEN THE LARGE BOX OF MEDICAL SUPPLIES ARRIVES:

› OPEN THE PACKAGE: Look through the contents to see if there are any special storage instructions in the large box that you need to follow.
› DO NOT SHAKE.
› MAKE SURE nothing has been damaged or leaked in shipment.
› CHECK the contents against the shipping list to make sure you received the correct supplies.
› STORE IN THE REFRIGERATOR: Some of these supplies must be refrigerated between 2 °C/36 °F and 8 °C/46 °F.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Infusion–Associated Reactions (continued)
An acute phase reaction (APR), an acute inflammatory response accompanied by elevations in inflammatory protein concentrations from blood tests, was observed.
• Most of the APRs occurred at 48 hours post infusion during the dose escalation period.
• The most common symptoms of APRs were fever, vomiting, and diarrhea.
• Your doctor can manage APRs like other IARs you may experience.

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PREPARING FOR HOME INFUSION

WHEN THE SMALLER BOX WITH XENPOZYME ARRIVES, IT MUST BE REFRIGERATED, NOT FROZEN, BETWEEN 2 °C/36 °F AND 8 °C/46 °F AND HANDLED WITH CARE.

YOU MUST:

› RECEIVE MEDICATION SHIPMENT: Be at home to receive the medication shipment, or make arrangements to have it delivered at a different time or to an alternate location (such as a neighbor’s house).

› STORE IN REFRIGERATOR: Be sure you have enough space in your refrigerator for the amount of XENPOZYME that will be shipped.

STORAGE TIPS:

› Some people find it helpful to use a mini refrigerator just for XENPOZYME.

› Some people store the other supplies in drawers reserved for medical supplies, small drawer organizers, or in an over-the-door shoe rack.

WHAT ELSE MAY BE NEEDED?

Because your dosing is based on your weight, you may need to have a scale available for periodic weight checks per your nurse’s orders or home health agency policies. Your dosing may change periodically if there are significant changes in your weight.

Your home environment must be able to accommodate the preparation and administration of infusions by providing an appropriate space to receive the infusions and ample storage space for supplies.

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.

• Elevated transaminase levels were reported in patients during the XENPOZYME dose escalation phase in clinical trials.

To manage the risk of elevated transaminase levels, 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 levels of transaminases from your blood tests, your doctor may make changes to your dose or infusion schedule.

Upon reaching the recommended maintenance dose, transaminase testing is recommended to be continued as part of routine clinical management of ASMD.

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WHAT TO EXPECT ON THE DAY OF YOUR HOME INFUSION

The first home infusion may take longer than those you received at the hospital or infusion center. The infusion nurse will check over your supplies, review your therapy orders, and have a discussion with you regarding your infusion process.

BEFORE YOUR INFUSION BEGINS, THE NURSE WILL:

▷ Give you pretreatment medication, if needed.
▷ Allow your XENPOZYME to come to room temperature.
▷ Perform a clinical assessment.
▷ Make sure they are able to gain access to a vein (or medication port, if you have one).

Once those steps are complete, your nurse will prepare your medication and perform any other necessary steps prior to infusion.

THE NURSE WILL REMAIN WITH YOU THROUGHOUT THE INFUSION and for an appropriate time after your infusion is completed.

IMPORTANT: During the infusion, always be sure to let the nurse know if you or your child experiences any changes in how you are feeling. These may be signs of a reaction.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Risk of Fetal Malformations During Dosage Initiation or Escalation in Pregnancy

XENPOZYME dosage initiation or escalation, for a female at any time during her pregnancy, is not recommended as it may increase risk of defects in the fetus. The decision to continue or discontinue XENPOZYME maintenance dosing, if you are a pregnant female, should be determined by you and your doctor and should consider your need for XENPOZYME, the potential drug-related risks to the fetus, and the potential risks due to untreated maternal ASMD disease.

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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FOLLOW THESE STEPS TO ESTABLISH A ROUTINE FOR YOUR INFUSIONS:

› Make sure you set aside enough time in your calendar for regular, biweekly infusions.
› On the day of your infusion, prepare a space in a room where you will feel comfortable.
› Allow additional time for preparation and post-infusion observation. Each infusion may last approximately between 3.5 and 4 hours.
› Establish a routine and plan some light activities you enjoy (e.g., reading a book or watching TV) to help pass the time.

EACH INFUSION may last approximately between 3.5 and 4 hours

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

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WHAT TO LOOK OUT FOR DURING INFUSION

Hypersensitivity reactions: In clinical trials, some adults and children experienced mild, moderate, or severe hypersensitivity reactions associated with the XENPOZYME infusion, such as hives, redness to the skin, or itchy skin.

Infusion-associated reactions (IARs): Some adults and children experienced side effects in the clinical trials that may have been associated with the XENPOZYME infusion. These typically occurred between the time of infusion and up to 24 hours after the infusion was complete.

If you or your child experiences hypersensitivity, IARs, or any other side effects, tell your doctor right away.

IMMEDIATELY SEEK URGENT MEDICAL ATTENTION IF ANY OF THE FOLLOWING SIGNS AND SYMPTOMS APPEAR OR WORSEN AFTER INFUSION:

- Headache
- Itching
- Vomiting
- Shortness of breath
- Hives/rash
- Skin redness
- Fever
- Swelling

OTHER IMPORTANT REMINDERS:

- Contact your nurse and prescriber immediately if you or your child does not feel well or experiences any side effects during or after infusion.

- XENPOZYME dosage initiation or escalation, at any time during pregnancy, is not recommended. Based on findings from animal studies, treatment with XENPOZYME may cause defects in the fetus.

- 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.

- When you receive the infusion at home:
  - Discuss any missed doses with the nurse providing care, as a different nurse may be assigned during subsequent home infusions. Report the missed doses to your healthcare team and prescriber.
  - Testing for liver enzyme levels is recommended to be continued as part of routine clinical management of ASMD.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions Including Anaphylaxis

Your doctor may decide to give you antihistamine, anti-fever, and/or steroid medications before your infusions.

- If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, your doctor should discontinue XENPOZYME immediately and initiate appropriate medical treatment.

- If a mild or moderate hypersensitivity reaction occurs, your doctor may adjust or temporarily withhold your infusion rate or dose of XENPOZYME.

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CareConnectPSS® Case Manager

Your CareConnectPSS Case Manager is your primary point of contact for questions about health insurance and reimbursement, and can help with care coordination such as scheduling infusions.

CareConnectPSS Patient Education Liaison (PEL)

Your CareConnectPSS PEL acts as a resource for ongoing questions, concerns, and support for you and your healthcare team. Your PEL can help make sure you have all the information you need, including:

- Disease and product in-service training from the Home Infusion Nursing Agency and Specialty Pharmacy
- Help with understanding what is required for home infusion regarding storage and delivery of supplies and medication
- Product and disease education, and acting as a resource for ongoing questions, concerns, and support for you and your healthcare team

Access personalized support and resources at CareConnectPSS.com.
Services are also available at 1-800-745-4447 (toll free), Option 3, or email Info@CareConnectPSS.com.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONTINUED)

Hypersensitivity Reactions Including Anaphylaxis (continued)

Hypersensitivity reactions, including anaphylaxis, have been reported in olipudase alfa-treated patients.

- Signs of hypersensitivity reactions in adults included hives, itchy skin, skin redness, rash, swelling underneath the skin, and tender bumps under the skin.
- Hypersensitivity reactions in pediatric patients included hives, itchy skin, rash, and localized swelling.

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Infusion-Associated Reactions

Your doctor may decide to give you antihistamine, anti-fever, and/or steroid medications before your infusions to reduce the risk of infusion-associated reactions (IARs). However, IARs may still occur after receiving these medications.

- If severe IARs occur, your doctor should discontinue XENPOZYME immediately and initiate appropriate medical treatment.
- If a mild or moderate IAR occurs, your doctor may adjust or temporarily withhold your infusion rate or dose of XENPOZYME.

The most frequent IARs in:

- adult patients were headache, rash, vomiting, and hives;
- pediatric patients were hives, swelling, headache, nausea, fever, and vomiting.

An acute phase reaction (APR), an acute inflammatory response accompanied by elevations in inflammatory protein concentrations from blood tests, was observed.

- Most of the APRs occurred at 48 hours post infusion during the dose escalation period.
- The most common symptoms of APRs were fever, vomiting, and diarrhea.
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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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BE READY FOR HOME INFUSIONS WITH XENPOZYME

YOUR XENPOZYME TREATMENT CHECKLIST

- Talk to your CareConnectPSS® Case Manager and Patient Education Liaison (PEL) for help arranging home infusions.
- Follow your infusion and monitoring schedule!
- Plan ahead so you’re ready for your appointment.
- If you have questions, have them ready to discuss with your doctor or infusion nurse.
- Prepare a clean space for your infusion and have some light activities to do to pass the time.
- Track your progress.
- Confirm your next infusion and monitoring appointments.
- Contact your doctor if you experience any side effects or infusion-associated reactions (IARs).

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14