Xenpozyme™
(olipudase alfa-rpcp)

A treatment option for non-CNS manifestations of ASMD (acid sphingomyelinase deficiency):

Patient and HCP perspectives on the transforming ASMD landscape

Thursday, February 23, 2023

6:15 AM – 7:15 AM, FLORIDA ROOM

FEATURED PHYSICIAN SPEAKERS

Melissa Wasserstein, MD
Chief, Department of Pediatrics Division of Pediatric Genetic Medicine
Albert Einstein College of Medicine, Montefiore Medical Center
New York, New York

Jaya Ganesh, MD
Associate Professor, Division of Medical Genetics, Department of Genetics and Genomic Sciences
Icahn School of Medicine
New York, New York

Following the presentation, Dr Wasserstein and Dr Ganesh will also be joined by a patient with ASMD for an engaging panel discussion

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

Patients treated with XENPOZYME have experienced hypersensitivity reactions, including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during XENPOZYME administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, XENPOZYME should be discontinued immediately, and appropriate medical treatment should be initiated. In patients with severe hypersensitivity reaction, a desensitization procedure to XENPOZYME may be considered.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions Including Anaphylaxis

Prior to XENPOZYME administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during XENPOZYME administration.

- If a *severe* hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue XENPOZYME immediately and initiate appropriate medical treatment. Consider the risks and benefits of re-administering XENPOZYME following severe hypersensitivity reactions (including anaphylaxis).
- If a *mild or moderate* hypersensitivity reaction occurs, the infusion rate may be slowed or temporarily withheld, and/or the XENPOZYME dose reduced.

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

- Hypersensitivity reactions in adults included urticaria, pruritus, erythema, rash, rash erythematous, eczema, angioedema, and erythema nodosum.
- Hypersensitivity reactions in pediatric patients included urticaria, pruritus, rash, erythema and localized edema.

Infusion-Associated Reactions

Antihistamines, antipyretics, and/or corticosteroids may be given prior to XENPOZYME administration to reduce the risk of infusion-associated reactions (IARs). However, IARs may still occur in patients after receiving pretreatment.

- If *severe* IARs occur, discontinue XENPOZYME immediately and initiate appropriate medical treatment. Consider the risks and benefits of re-administering XENPOZYME following severe IARs.
- If a *mild or moderate* IAR occurs, the infusion rate may be slowed or temporarily withheld, and/or the XENPOZYME dosage may be reduced.

The most frequent IARs in:

- adult patients were headache, pruritus, vomiting and urticaria;
- pediatric patients were urticaria, erythema, headache, nausea, pyrexia, and vomiting.

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

- Most of the APRs occurred at 48 hours post infusion during the dose escalation period.
- Elevations of C-reactive protein, calcitonin, and IL-6, and reduction of serum iron were observed.
- The most common clinical symptoms associated with APRs were pyrexia, vomiting, and diarrhea. APRs can be managed as other IARs.

Elevated Transaminases Levels

XENPOZYME may be associated with elevated transaminases (ALT, AST, or both) within 24 to 48 hours after infusion.

- Elevated transaminase levels were reported in patients during the XENPOZYME dose escalation phase in clinical trials.
- At the time of the next scheduled infusion, these elevated transaminase levels generally returned to levels observed prior to the XENPOZYME infusion.

To manage the risk of elevated transaminase levels, assess ALT and AST:

- within one month prior to initiation of XENPOZYME,
- within 72 hours prior to any infusion during dose escalation, which includes the first 3 mg/kg dose, or prior to the next scheduled XENPOZYME infusion upon resuming treatment following a missed dose.
- See full Prescribing Information for additional information on assessment and management of elevated transaminases.

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

Risk of Fetal Malformations During Dosage Initiation or Escalation in Pregnancy

XENPOZYME dosage initiation or escalation, at any time during pregnancy, is not recommended as it may lead to elevated sphingomyelin metabolite levels that may increase the risk of fetal malformations. The decision to continue or discontinue XENPOZYME maintenance dosing in pregnancy should consider the female's need for XENPOZYME, the potential drug-related risks to the fetus, and the potential adverse outcomes from untreated maternal ASMD disease.

Verify pregnancy status in females of reproductive potential prior to initiating XENPOZYME treatment. Advise females of reproductive potential to use effective contraception during XENPOZYME treatment and for 14 days after the last dose if XENPOZYME is discontinued.

ADVERSE REACTIONS

- Most frequently reported adverse drug reactions in adults (incidence ≥10%) were headache, cough, diarrhea, hypotension, and ocular hyperemia.
- Most frequently reported adverse drug reactions in pediatric patients (incidence ≥20%) were pyrexia, cough, diarrhea, rhinitis, abdominal pain, vomiting, headache, urticaria, nausea, rash, arthralgia, pruritus, fatigue, and pharyngitis.

Please see accompanying full Prescribing Information, including Boxed WARNING, for complete details.

This event is open to healthcare professionals only. Seating is limited and available on a first-come, first-served basis. In accordance with Sanofi's policies and the PhRMA Code on Interactions with Healthcare Professionals, attendance at this educational program is limited to healthcare professionals.

This Satellite Symposium is not part of the official WORLD*Symposium*™ program, and WORLD*Symposium* does not approve or endorse any commercial products or services discussed during the Satellite Symposium or offered for sale by the corporate supporter of the Satellite Symposium. This Satellite Symposium is not approved for CME by WORLD*Symposium* or any other entity.

Eligibility: Registration for WORLD*Symposium* 2023 and an official name badge are required to attend this satellite symposium.

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