Xenpozyme (olipudase alfa-rpcp)

Override(s)	Approval Duration
Prior Authorization	Initial requests: 6 months Continuation requests: 1 year

Medications	Dosing Limit
Xenpozyme (olipudase alfa-rpcp) for injection, for intravenous use	3 mg/kg every 2 weeks

APPROVAL CRITERIA

Initial requests for Xenpozyme (olipudase alfa-rpcp) may be approved if the following criteria are met:

I. Individual has a diagnosis of acid sphingomyelinase deficiency (ASMD); **AND**
II. Individual has a clinical presentation consistent with ASMD type B OR ASMD type A/B; **AND**
III. Documentation is provided that diagnosis has been confirmed by (McGovern 2017):
    A. Pathogenic sphingomyelin phosphodiesterase-1 (SMPD1) gene mutation; **OR**
    B. Deficiency in acid sphingomyelinase (ASM) activity as measured in fibroblasts, leukocytes or dried blood spot;
    AND
IV. Individual is using for the treatment of non-central nervous system disease manifestations.

Continuation requests for Xenpozyme (olipudase alfa-rpcp) may be approved if the following criterion is met:

I. There is confirmation of clinically significant improvement or stabilization in clinical signs and symptoms of disease (including but not limited to improvement in splenomegaly, hepatomegaly, pulmonary function or platelet count).

Requests for Xenpozyme (olipudase alfa-rpcp) may not be approved for the following:

I. Individual has a clinical presentation consistent with ASMD type A; **OR**
II. Individual has a diagnosis of Niemann-Pick disease type C; **OR**
III. May not be approved when the above criteria are not met and for all other indications.

NOTE:
Xenpozyme has a black box warning for severe hypersensitivity reactions including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during Xenpozyme administration. If a severe

hypersensitivity reaction occurs, Xenpozyme should be discontinued immediately, and appropriate medical treatment should be initiated.

Key References:

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: December 7, 2022.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
4. McGovern MM, Dionisi-Vici C, Giugliani R, et al. Consensus recommendation for a diagnostic guideline for acid sphingomyelinase deficiency. *Genet Med*. 2017;19(9):967-974.
5. Patterson MC. Overview of Niemann-Pick disease. Updated: December 21, 2020. In: UpToDate, Post TW (Ed), UpToDate, Waltham, MA. Accessed: December 7, 2022.
6. Wasserstein M, Lachmann R, Hollak C, et al. A randomized, placebo-controlled clinical trial evaluating olipudase alfa enzyme replacement therapy for chronic acid sphingomyelinase deficiency (ASMD) in adults: One-year results. *Genet Med*. 2022;24(7):1425-1436.