PHARMACY POLICY STATEMENT
North Carolina Marketplace

DRUG NAME	Xenpozyme (olipudase alfa-rpcp)
BILLING CODE	J3490, J3590; NDC: 58468-0050-1
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Background statement: Xenpozyme is a recombinant human acid sphingomyelinase enzyme initially approved by the FDA in 2022 for the treatment of non-central nervous system (non-CNS) manifestations of

Initial dose : Recommended starting dose is 0.1 mg/kg for adults and 0.03 mg/kg for pediatrics administered as an intravenous infusion. Xenpozyme is administered via intravenous infusion every 2 weeks following the dose escalation regimen in the package insert.
Maintenance dose: The recommended maintenance dos e of Xenpozyme in pediatrics and adults is 3 mg/kg via intravenous infusion every 2 weeks.

If all the above requirements are met, the medication will be approved for 6 months.

For reauthorization :

1. Chart notes must show improvement from baseline in at least one of the following: improved lung function (increased % predicted DLco), reduction of splenomegaly, reduction of hepatomegaly, increased platelet count, or improvement in children experiencing growth delay; AND
2. Chart notes must show that ALT and AST are continuing to be monitored.

If all the above requirements are met, the medication will be approved for an additional 1 year.

CareSource considers Xenpozyme (olipudase alfa-rpcp) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off- Label policy.

DATE	ACTION/DESCRIPTION
11/18/2022	New policy for Xenpozyme created.

References:

1. Xenpozyme. Package Insert. Sanofi; 2022. Accessed October 21, 2022.
2. Xenpozyme. Prescribing information. Sanofi; 2022. Accessed October 21, 2022. https://products.sanofi.us/xenpozyme/xenpozyme.pdf
3. Xenpozyme: olipudase alfa-rpcp. Accessed October 21, 2022. https://www.xenpozyme.com/
4. Keam SJ. Olipudase Alfa: First Approval. Drugs. 2022 Jun;82(8):941-947. doi: 10.1007/s40265-022-01727-x. PMID: 35639287.
5. Xenpozyme (olipudase alfa-rpcp) approved by FDA as first disease-specific treatment for ASMD (non-CNS manifestations). Sanofi. Accessed October 21, 2022. https://www.news.sanofi.us/2022-08-31-XenpozymeTM-olipudase-alfa-rpcp-approved-by-FDA-as-first-disease-specific-treatment-for-ASMD-non-CNS-manifestations?
6. Diagnosing ASMD. ASMD. Updated March 2022. Accessed October 21, 2022. https://www.asmdfacts.com/hcp/diagnosing-asmd
7. Xenpozyme. Lexi-Drugs. Lexicomp Online. Wolters Kluwer Health, Inc. October 3, 2022. Accessed October 21, 2022. http://online.lexi.com
8. Diaz GA, Jones SA, Scarpa M, et al. One-year results of a clinical trial of olipudase alfa enzyme replacement therapy in pediatric patients with acid sphingomyelinase deficiency. *Genetics in Medicine.* 2021; 23 (8): 1543-1550. doi:10.1038/s41436-021-01156-3
9. 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. *Genetics in Medicine.* 2022; 24 (7): 1425-1436. doi:10.1016/j.gim.2022.03.021
10. McGovern M, Dionisi-Vici C, Giugliani R, et al. Consensus recommendation for a diagnostic guideline for acid sphingomyelinase deficiency. *Genetics in Medicine.* 2017; 19: 967–974. doi:10.1038/gim.2017.7

Effective date: 04/01/2023
Revised date: 11/18/2022