Medical Policy

Xenpozyme™ (olipudase alfa)	
MEDICAL POLICY NUMBER	MED_Clin_Ops_128
CURRENT VERSION EFFECTIVE DATE	01/01/2024
APPLICABLE PRODUCT AND MARKET	Individual Family Plan: ALL Small Group: ALL Medicare Advantage: ALL

Brand New Day/Central Health Medicare Plan develops policies and makes coverage determinations using credible scientific evidence including but not limited to MCG™ Health Guidelines, the ASAM Criteria™, and other third party sources, such as peer-reviewed medical literature generally recognized by the relevant medical community, physician specialty society recommendations, and expert opinion as relevant to supplement those sources. Brand New Day/Central Health Medicare Plan Medical Policies, MCG™ Guidelines, and the ASAM Criteria™ are not intended to be used without the independent clinical judgment of a qualified health care provider considering the individual circumstances of each member's case. The treating health care providers are solely responsible for diagnosis, treatment, and medical advice. Members may contact Brand New Day/Central Health Medicare Plan Customer Service at the phone number listed on their member identification card to discuss their benefits more specifically. Providers with questions about this Brand New Day/ Central Health Medicare Plan policy may contact the Health Plan. Brand New Day/Central Health Medicare Plan policies and practices are compliant with federal and state requirements, including mental health parity laws.

If there is a difference between this policy and the member specific plan document, the member benefit plan document will govern. For Medicare Advantage members, Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), govern. Refer to the CMS website at http://www.cms.gov for additional information.

Brand New Day/Central Health Medicare Plan medical policies address technology assessment of new and emerging treatments, devices, drugs, etc. They are developed to assist in administering plan benefits and do not constitute an offer of coverage nor medical advice. Brand New Day/Central Health Medicare Plan medical policies contain only a partial, general description of plan or program benefits and do not constitute a contract. Brand New Day/Central Health Medicare Plan does not provide health care services and, therefore, cannot guarantee any results or outcomes. Treating providers are solely responsible for medical advice and treatment of members. Our medical policies are updated based on changes in the evidence and healthcare coding and therefore are subject to change without notice. CPT codes, descriptions and materials are copyrighted by the American Medical Association (AMA). MCG™ and Care Guidelines® are trademarks of MCG Health, LLC (MCG).

PURPOSE

To promote consistency between reviewers in clinical coverage decision-making by providing the criteria that generally determine the medical necessity of Xenpozyme™ (olipudase alfa) therapy.

POLICY/CRITERIA

Prior Authorization and Medical Review is required.

Coverage will be provided for 12 months and may be renewed.

Max Units (per dose and over time) [HCPCS Unit]:

- 340 mg every 14 days

Medical Policy

Initial

1. Provider attests that female patients of reproductive potential will have pregnancy status verified prior to start of therapy and will use effective contraception during treatment and for 14 days after the last dose if therapy is discontinued; **AND**
2. Documentation has been submitted for the following baseline measurements(necessary for renewal):
    a. Percent predicted diffusion capacity of the lungs for carbon monoxide (DLco) or other age-appropriate pulmonary function testing,
    b. Spleen volume,
    c. Liver volume,
    d. Plasma lyso-sphingomyelin
    e. Platelet count
    f. Note - height Z-score and skeletal maturation (pediatric patients); **AND**
3. Documented baseline transaminase (alanine aminotransferase [ALT] and aspartate aminotransferase [AST]) levels within 1 month prior to treatment initiation, within 72 hours prior to any infusion during dose escalation, and periodically throughout therapy; **AND**
4. Patient should not require invasive ventilatory support OR non-invasive ventilatory support while awake and for >12 hours a day; **AND**
5. Patient has a definitive diagnosis of Acid Sphingomyelinase Deficiency (ASMD) as confirmed and documented by the following:
    a. Detection of biallelic pathogenic mutations in the SMPD1 gene by molecular genetic testing; **OR**
    b. Deficiency of acid sphingomyelinase enzyme activity <10% of controls as measured in peripheral leukocytes, cultured fibroblasts, or lymphocytes; **AND**
6. Patient has a clinical diagnosis consistent with Niemann-Pick disease type **B** (**NPD-B**) or **A/B** (**NPD-A/B**); **AND**
7. Xenpozyme will be used for **non**-CNS manifestations of disease.

Renewal

1. Patient continues to meet initial criteria; **AND**
2. Absence of unacceptable toxicity from the drug including, but not limited to, anaphylaxis and severe hypersensitivity reactions, severe infusion-associated reactions, severely elevated liver transaminases, etc.; **AND**
3. Patient has not experienced progressive/irreversible severe cognitive impairment; **AND**
4. Disease response with treatment as defined by improvement or stability from pre-treatment baseline by the following:
    a. Improvement in or stability in the percent predicted diffusion capacity of the lungs for carbon monoxide (DLco) or other age-appropriate pulmonary function testing; **OR**
    b. Improvement in or stability of spleen and/or liver volumes; **OR**
    c. Reduction in plasma lyso-sphingomyelin; **OR**
    d. Improvement in or stability of platelet count; **OR**
    e. Improvement in linear growth progression as measured by mean height Z-scores (pediatric patients only)

LIMITATIONS/EXCLUSIONS

1. Any indication other than those listed above due to insufficient evidence of therapeutic value

CODING

Applicable NDC Codes	
58468-0050 -01	Xenpozyme 20 mg lyophilized powder for reconstitution in a single-dose vial

Applicable Procedure Code	
J3590	Unclassified biologics

Applicable ICD-10 Codes	
E75.241	Niemann-Pick disease type B
E75.244	Niemann-Pick disease type A/B

EVIDENCE BASED REFERENCES

1. Xenpozyme [package insert]. Cambridge, MA; Genzyme Corporation, Inc.; August 2022. Accessed October 2022.

POLICY HISTORY

Revision History	Month Day, Year	Updates
Original Effective Date	NOVEMBER 8, 2022	
Revision	MARCH 1, 2023	Adopted by MA UMC
P&T Committee Endorsement	NOVEMBER 8, 2022	
Revision	JANUARY 1, 2024	Updated to Brand New Day/Central Health Medicare Plan/Central Health Medicare Plan