

## Medical Policy:

### Xenpozyme (olipudase alfa-rpcp), intravenous infusion

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.366	January 2, 2024	November 10, 2022

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## Definitions

XENPOZYME is indicated for treatment of non–central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients.

## Length of Authorization

12 months

## Dosing Limits [Medical Benefit]

Dosing is weight-based. For patients with a body mass index (BMI) of  $\leq 30$  kg/m<sup>2</sup>, actual body weight is used. For patients with a BMI  $> 30$  kg/m<sup>2</sup> adjusted body weight is used (adjusted body weight in kg = [actual height in meters]<sup>2</sup> x 30). Home infusion of Xenpozyme under the supervision of a healthcare provider may be considered for patients on a maintenance dose and who are tolerating the infusion well. The decision to have patients moved to home infusion should be made after evaluation and recommendation by a physician.

The recommended starting dose in adults is 0.1 mg/kg via intravenous (IV) infusion. The dose is titrated every 2 weeks over a period of 14 weeks to a maintenance dose of 3 mg/kg every 2 weeks (Table 1). In pediatric patients, the recommended starting dose is 0.03 mg/kg via IV infusion.<sup>1</sup> The dose is titrated every 2 weeks over a period of 16 weeks to a maintenance dose of 3 mg/kg every 2 weeks (Table 2). To reduce the risk of hypersensitivity and infusion-related reactions or elevated transaminase levels, the dose escalation regimen outlined in Tables 1 and 2 below should be followed. A dose is considered “missed” when it is not administered within 3 days of the scheduled date.<sup>1</sup> Refer to Table 3 for missed doses.

**Table 1. Xenpozyme Dose Escalation Regimen for Adults (> 18 Years of Age).<sup>1</sup>**

First dose (Day 1/Week 0)	0.1 mg/kg
Second dose (Week 2)	0.3 mg/kg
Third dose (Week 4)	0.3 mg/kg
Fourth dose (Week 6)	0.6 mg/kg
Fifth dose (Week 8)	0.6 mg/kg
Sixth dose (Week 10)	1 mg/kg
Seventh dose (Week 12)	2 mg/kg
Eighth dose (Week 14) <sup>†</sup>	3 mg/kg

<sup>†</sup> The dose escalation phase includes the first 3 mg/kg dose.

**Table 2. Xenpozyme Dose Escalation Regimen for Pediatric Patients.<sup>1</sup>**

First dose (Day 1/Week 0)	0.03 mg/kg
Second dose (Week 2)	0.1 mg/kg
Third dose (Week 4)	0.3 mg/kg
Fourth dose (Week 6)	0.3 mg/kg
Fifth dose (Week 8)	0.6 mg/kg
Sixth dose (Week 10)	0.6 mg/kg
Seventh dose (Week 12)	1 mg/kg
Eighth dose (Week 14) <sup>†</sup>	2 mg/kg
Ninth dose (Week 16) <sup>†</sup>	3 mg/kg

<sup>†</sup> The dose escalation phase includes the first 3 mg/kg dose.

**Table 3. Dosing Recommendations for Xenpozyme Missed Doses\*.<sup>1</sup>**

Consecutive Missed Doses In:	Escalation Phase	Maintenance Phase
1 missed dose	<u>First dose after a missed dose:</u> Administer last tolerated dose.  <u>Second and subsequent doses after missed dose:</u> Resume dose escalation at next infusion according to Table 1 for adult patients or Table 2 for pediatric patients.	<u>First and subsequent doses after missed dose:</u> Administer maintenance dose.
2 consecutive missed doses	<u>First dose after missed dose:</u> Administer 1 dose below the last tolerated dose.  <u>Second and subsequent doses after missed dose:</u> Resume dose escalation according to Table 1 for adults or Table 2 for pediatric patients.	<u>First dose after missed dose:</u> Administer 1 dose below the maintenance dose.  <u>Second and subsequent doses after missed dose:</u> Resume the maintenance dose.
≥ 3 consecutive missed doses	<u>First and subsequent doses after missed doses:</u> Resume dose escalation at 0.3 mg/kg and follow Table 1 for adults or Table 2 for pediatric patients.	<u>First and subsequent doses after missed doses:</u> Restart dosing at 0.3 mg/kg and follow Table 1 for adult patients or Table 2 for pediatric patients.

\*At scheduled infusion after a missed dose, if the dose administered is 0.3 mg/kg or 0.6 mg/kg, administer that dose twice as per Table 1 and 2.

Limit: 3mg/kg every 2 weeks; 340 billable units (340 mg) every 14 days

## Guideline

1. **Acid Sphingomyelinase Deficiency (ASMD).** Approve if the patient meets the following criteria (A, B, C, and D):
  - A. The diagnosis of ASMD meets ALL of the following (i, ii, and iii):
    - i. The diagnosis of ASMD has been established by acid sphingomyelinase (ASM) enzymatic assay testing; **AND**
    - ii. The diagnosis of ASMD has been confirmed by mutation testing; **AND**
    - iii. A diagnosis of Gaucher disease has been excluded; **AND**  
*Note: ASMD has historically been known as Niemann-Pick Disease.*
  - B. Patient meets **ONE** of the following criteria (i or ii):
    - i. Patient has ASMD type B; **OR**
    - ii. Patient has ASMD type A/B; **AND**
  - C. Patient has **TWO** or more non-central nervous system signs of ASMD type B or type A/B according to the prescriber; **AND**  
*Note: Examples of non-central nervous system signs of ASMD type B or type A/B include but are not limited to hepatosplenomegaly, interstitial lung disease, decreased diffusing capacity of the lungs, progressive liver disease with cirrhosis or fibrosis, dyslipidemia, osteopenia, thrombocytopenia, anemia, leukopenia.*
  - D. The medication is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders

## Applicable Procedure Codes

Code	Description
J0218	Injection, olipudase alfa-rpcp, 1 mg; 1 billable unit = 1 mg

## Applicable NDCs

Code	Description
58468-0050-01	Xenpozyme (olipudase alfa-rpcp) 20mg vial

## ICD-10 Diagnoses

Code	Description
E75.241	Niemann-Pick disease type B
E75.244	Niemann-Pick disease type A/B

## Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	1/2/2024	Annual Review: No criteria changes
EmblemHealth & ConnectiCare	5/02/2023	Annual Review: Added code J0218, removed code J3590, Removed ICD-10 codes E75.29 and E75.24, added E75.241 and E75.244.  <u>Under ASMD initial Criteria-</u> Removed the Statement "A.The diagnosis of ASMD is established by enzymatic assay; AND" and replaced it with the statement "A) The diagnosis of ASMD meets ALL of the following (i, ii, and

		<p>iii):</p> <p>i.The diagnosis of ASMD has been established by acid sphingomylinase (ASM) enzymatic assay testing; AND</p> <p>ii.The diagnosis of ASMD has been confirmed by mutation testing; AND</p> <p>iii.A diagnosis of Gaucher disease has been excluded; AND</p> <p>Note: ASMD has historically been known as Niemann-Pick Disease.”</p> <p>Removed the statement “C.Patient has signs of ASMD type B or type A/B (e.g., hepatosplenomegaly, decreased diffusing capacity of the lungs, progressive liver disease with cirrhosis or fibrosis, dyslipidemia, osteopenia, and thrombocytopenia), according to the prescriber; AND” and replaced it with “C) Patient has two or more non-central nervous system signs of ASMD type B or type A/B according to the prescriber; AND</p> <p>Note: Examples of non-central nervous system signs of ASMD type B or type A/B include but are not limited to hepatosplenomegaly, interstitial lung disease, decreased diffusing capacity of the lungs, progressive liver disease with cirrhosis or fibrosis, dyslipidemia, osteopenia, thrombocytopenia, anemia, leukopenia.”</p>
EmblemHealth & ConnectiCare	11/10/2022	New Policy

## References

1. Xenpozyme™ intravenous infusion [prescribing information]. Cambridge, MA: Genzyme; August 2022.