

## **Xenpozyme™ (olipudase alfa-rpcp intravenous infusion)**

When requesting Xenpozyme™ (olipudase alfa-rpcp intravenous infusion), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

### **FDA-approved indication**

Xenpozyme is indicated for the treatment of non-central nervous system (CNS) manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients.

### **Coverage Guidelines**

#### **Acid Sphingomyelinase Deficiency (ASMD)**

The individual must meet all of the following criteria for authorization:

- Diagnosis is established by acid sphingomyelinase (ASM) enzymatic assay and mutation testing;
- A diagnosis of Gaucher disease is excluded;
- Has ASMD type B OR ASMD type A/B;
- Has two or more non-central nervous system signs of ASMD type B or type A/B (e.g., hepatosplenomegaly, interstitial lung disease, decreased diffusing capacity of the lungs, progressive liver disease with cirrhosis or fibrosis, dyslipidemia, osteopenia, thrombocytopenia, anemia, leukopenia);
- 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.

**Approval duration:** 12 months

## Dosing Recommendations

Recommended starting dose of Xenpozyme in adults is 0.1mg/kg via intravenous infusion every 2 weeks. Dose escalation is as follows:

**Table 1: XENPOZYME Dose Escalation Regimen for Adult Patients\***

| Adult Patients (18 years and older) |                                        |
|-------------------------------------|----------------------------------------|
| 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)†              | 3 mg/kg (recommended maintenance dose) |

\*Use actual body weight for patients with a BMI less than or equal to 30. For patients with a BMI greater than 30, calculate adjusted body weight (kg) = (actual height in m)<sup>2</sup> × 30.

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

Recommended starting dose of Xenpozyme in pediatric patients is 0.03 mg/kg via intravenous infusion every 2 weeks. Dose escalation is as follows:

**Table 2: XENPOZYME Dose Escalation Regimen for Pediatric Patients\***

| Pediatric Patients (0-17 years) |                                        |
|---------------------------------|----------------------------------------|
| 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)†          | 2 mg/kg                                |
| Ninth dose (Week 16)†           | 3 mg/kg (recommended maintenance dose) |

\*Use actual body weight for patients with a BMI less than or equal to 30. For patients with a BMI greater than 30, calculate adjusted body weight (kg) = (actual height in m)<sup>2</sup> × 30.

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

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## References

1. Xenpozyme™ intravenous infusion [prescribing information]. Cambridge, MA: Genzyme; December 2023.
2. Wasserstein M, Lachmann R, Hollack C, et al. A randomized, placebo-controlled clinical trial evaluating olipudase alfa enzyme replacement for chronic acid sphingomyelinase deficiency (ASMD) in adults: One-year results. *Genet Med.* 2022;24(7):1425-1436.
3. 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. *Genet Med.* 2021;23:154-1550.
4. Geberhiwot T., Wasserstein M, Wanninayake, S. et al. Consensus clinical management guidelines for acid sphingomyelinase deficiency (Niemann-Pick disease types A, B, and A/B). *Orphanet J Rare Dis* 18,85 (2023). Available at: <https://doi.org/10.1186/s13023-023-02686-6>. Accessed August 31, 2023.