

Xenpozyme

Qatar · access guide

How to access Xenpozyme from Qatar, the named-patient import pathway, 2026

By Reserve Meds · Clinical & regulatory team · Last reviewed 2026-04-24

A Qatar patient with confirmed acid sphingomyelinase deficiency (ASMD), historically referred to as Niemann-Pick disease type B, and in some presentations as type A/B, may receive a prescription for Xenpozyme (olipudase alfa) from their treating metabolic specialist, paediatric geneticist, or pulmonologist. Xenpozyme is FDA-approved in the United States as an enzyme-replacement therapy for non-central-nervous-system manifestations of ASMD in paediatric and adult patients. Because ASMD is ultra-rare and Xenpozyme is not yet routinely stocked in Qatar hospital pharmacies, your specialist may be coordinating a named-patient import pathway on your behalf.

This guide explains the legal pathway, what documentation your physician needs, typical timing and cost bands, and where Reserve Meds fits in.

The clinical situation

Xenpozyme is an intravenous recombinant human acid sphingomyelinase. It is administered via a dose-titration protocol, starting at a low dose and escalating every two weeks to the target maintenance dose, to reduce the risk of infusion-associated reactions that can accompany the clearance of sphingomyelin stores early in treatment. Once maintenance is reached, infusions are given every two weeks. The manufacturer is Sanofi. Eligibility requires biochemical and/or genetic confirmation of ASMD (SMPD1 variant and/or deficient acid sphingomyelinase activity). Your specialist will confirm the diagnosis, assess organ-system burden (lung diffusion capacity, spleen and liver volume, platelet count, lipid profile), and coordinate the titration and monitoring plan per FDA labeling.

Is Xenpozyme legally importable into Qatar?

Yes, through the Qatar Ministry of Public Health (MOPH) named-patient import framework. The Qatar has a mature named-patient mechanism that has supported cross-border access to rare-disease enzyme-replacement therapies for many years.

The MOPH named-patient route allows a Qatar-licensed physician to request import of a medicine that is not locally registered when: (a) the medicine has been approved by a recognised reference authority (FDA qualifies), (b) there is no clinically equivalent locally registered alternative, (c) the treating physician takes clinical responsibility for use, and (d) the importing party documents chain of custody from the US source to the administering facility. Applications are reviewed by the MOPH Drug Sector. For ultra-rare conditions like ASMD, where no local alternative exists and the clinical rationale is diagnosis-driven, the submission is straightforward to frame.

How the pathway works, step by step

1. **Consultation with your treating specialist.** The decision to prescribe Xenpozyme is a clinical one, based on ASMD confirmation and organ-system burden. Your specialist documents the rationale and the titration plan.
2. **Infusion-facility identification.** A Qatar tertiary metabolic or paediatric centre equipped for bi-weekly infusions with infusion-reaction management capability accepts the case.
3. **MOPH named-patient application.** Your physician or the hospital's importing pharmacy files an application including clinical rationale, diagnostic confirmation, patient identifier, product details, titration schedule, and chain-of-custody plan.
4. **US-side sourcing.** Reserve Meds coordinates with our US-licensed specialty wholesale partner to secure product from the manufacturer's authorised distribution chain under DSCSA.
5. **Cold-chain shipment.** Xenpozyme requires refrigerated handling and ships with validated cold-chain packaging and continuous temperature logging end to end.
6. **Arrival and titration start.** The infusion centre receives the product and begins the dose-titration protocol under your specialist's care. Reserve Meds coordinates the next cycle ahead of depletion.

What documentation your physician needs

Your physician will typically need to provide:

- A clinical rationale letter confirming ASMD diagnosis (SMPD1 variant and/or enzyme assay), organ-system status, and Xenpozyme as the indicated therapy
- Verification of their Qatar medical licence (SCFHS / MOH)
- A current prescription naming the product, the titration schedule, and the target maintenance dose
- Patient identifier (anonymised reference preferred)
- The identified infusion facility and its cold-chain and infusion-reaction handling capability

Reserve Meds provides a physician documentation kit bundling the templates MOPH reviewers expect to see for named-patient import of cold-chain enzyme-replacement therapies with titration protocols.

Costs and timing

Xenpozyme's US cash-pay reference price is weight-dependent and scales with the titration protocol. A typical adult annual cost sits in an indicative 2026 range of roughly USD 600,000-900,000 for ongoing bi-weekly infusions at maintenance dose, with the first several months lower because of the titration ramp. Cold-chain logistics, MOPH documentation handling, and concierge coordination add incremental cost; Reserve Meds issues a full transparent quote at the start of intake, with a drug-only reference figure separated from service charges so your family can see the breakdown.

Indicative timing for first shipment arrival after cohort intake opens is 7-14 days from the moment a complete MOPH application is submitted. Subsequent cycles are scheduled to match the bi-weekly titration and maintenance calendar.

Fulfillment availability is limited to our first cohort, and all timelines published on this site are indicative. If your clinical situation is time-sensitive, tell us at intake. We triage accordingly.

A brief culturally-aware note: ASMD has a higher reported prevalence in certain regional communities, and Qatar tertiary metabolic centres have experience managing lysosomal storage disorders. Ramadan and Hajj seasons can affect infusion scheduling; our concierge team coordinates cycle timing with your family's preferences and your hospital's calendar.

Reserve Meds's role

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. For Xenpozyme specifically, we provide:

- **Sourcing.** Through our US-licensed specialty wholesale partner, operating under DSCSA chain-of-custody.
- **Documentation.** Regulatory documentation package for your physician and for MOPH review, including diagnostic-attestation templates for ASMD.
- **Logistics.** Cold-chain shipment and chain-of-custody coordination with temperature logging.
- **Concierge case lead.** A named point of contact for your family and your physician throughout the process.

What we do not do: we are not the prescriber, we do not practise medicine, and we are not the dispensing pharmacy. All clinical decisions remain with your treating specialist and the infusion facility.

Frequently asked

Is this legal in Qatar? Yes, when executed through the MOPH named-patient framework with appropriate documentation and a licensed administering facility. See our trust and compliance page for our methodology.

Does Xenpozyme treat the neurological form of Niemann-Pick? Xenpozyme addresses non-CNS manifestations of ASMD. It is not approved for the neuronopathic (type A) form. Your specialist will confirm the subtype and what the therapy is expected to address.

Why the dose-titration protocol? Gradual dose escalation is built into Xenpozyme's labeling to reduce the risk of infusion-associated reactions that can occur as sphingomyelin is cleared. Your infusion team follows the manufacturer's titration schedule.

What about paediatric patients? Xenpozyme is approved for both paediatric and adult patients. Paediatric dosing follows the same titration framework adjusted for weight. Your paediatric metabolic specialist will lead the plan.

Will private insurance cover this? Cash-pay is the default. Some Qatar private insurers reimburse named-patient imports for ultra-rare enzyme-replacement therapies on a case-by-case basis; we supply documentation for your submission but do not process insurance claims directly.

Reserve Meds's role

US-based concierge coordinator for cross-border specialty medicine. We are not the prescriber, not the dispensing pharmacy, and not the manufacturer. All clinical decisions remain with your treating physician.

Reserve Meds

reserved for you.

Composite case examples. This document is for general information only and does not constitute medical advice. Please consult your treating physician.

Reserve Meds is in pre-launch. Published timelines and cost ranges are indicative, not guarantees.

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