Aldurazyme

Qatar · access guide

Aldurazyme (laronidase) for a Qatari family: what the pathway looks like in 2026

By Reserve Meds clinical & regulatory team. Last reviewed 2026-05-20.

Qatari families looking into Aldurazyme for a child with mucopolysaccharidosis type I, MPS I, are in a workable position. The therapy has a long track record in the region. Sidra Medicine in Doha is a dedicated paediatric specialty centre with established rare-disease infrastructure and is the natural infusion home for paediatric MPS I patients in Qatar. Hamad Medical Corporation handles the wider paediatric and adult metabolic population. The Qatar MOPH framework for rare-disease ERT is mature.

This page is meant to be the first honest read you get on Aldurazyme in Qatar, written by the team that would coordinate around your child's case if you decided you wanted operational support on the workup, the documentation, the import logistics, or the long-term cost picture.

We will be specific about MPS I, what the workup decides, the regulatory pathway in 2026, the cost in QAR and US dollars, the Sidra Medicine and HMC pathways, and where Reserve Meds adds value.

What MPS I actually is, in plain terms

MPS I is a lysosomal storage disorder caused by deficiency of the enzyme alpha-L-iduronidase (IDUA). The deficiency leads to progressive accumulation of dermatan sulfate and heparan sulfate in lysosomes across the body. Presentation spans a clinical spectrum: severe Hurler syndrome with infant-onset multisystemic disease and progressive cognitive decline, intermediate Hurler-Scheie with somatic features but preserved cognition, and attenuated Scheie often diagnosed in adolescence or adulthood.

Aldurazyme is recombinant alpha-L-iduronidase, administered as a weekly intravenous infusion at 0.58 mg/kg over 3 to 4 hours. The therapy is disease-modifying for non-CNS manifestations: cardiac valves, pulmonary function, hepatosplenomegaly, joint mobility, sleep apnea, corneal clouding (variable response). It does not cross the blood-brain barrier and does not meaningfully address the cognitive decline of severe Hurler.

For severe Hurler infants, the standard of care globally is hematopoietic stem cell transplantation, HSCT, ideally before age 2 to 2.5, because donor bone marrow produces enzyme that crosses into the CNS. Aldurazyme is used as a bridge to HSCT and often as an adjunct afterwards. For Hurler-Scheie and Scheie patients, ERT alone is typically the long-term answer.

The workup that decides eligibility and shapes the plan

Sidra Medicine's paediatric genetics service runs the diagnostic and pre-treatment workup in-house. The standard components are urinary GAG screen (elevated dermatan sulfate and heparan sulfate as the cheap first screen), alpha-L-iduronidase enzyme activity assay in leukocytes or fibroblasts (definitive enzymatic confirmation), IDUA gene sequencing (specific pathogenic variant identification, severity classification informed by genotype), and baseline organ assessments (echocardiogram, FVC, sleep study, ophthalmology for corneal clouding, ENT, hepatomegaly assessment, joint range of motion, 6-minute walk test).

Severity classification by the paediatric metabolic specialist (severe Hurler, Hurler-Scheie, or Scheie) is the pivotal decision because it shapes the HSCT-versus-ERT-alone conversation. A clinical rationale letter from the metabolic specialist documents the diagnosis, the severity classification, the recommended treatment plan, and the long-term monitoring schedule.

For adult MPS I patients (mostly attenuated Scheie) Hamad Medical Corporation is the operationally appropriate setting; Sidra is paediatric-only.

The Qatar regulatory and infusion pathway in 2026

Qatar Ministry of Public Health (MOPH), Department of Pharmacy and Drug Control, is the registration and import authority. [VERIFY: current MOPH registration status of Aldurazyme 2026]. Where formal registration is in place, standard prescription applies; where the product moves through the named-patient mechanism, the dispensing facility's import pharmacy files. Aldurazyme is treated as a benchmark rare-disease ERT and the MOPH framework is workable for it.

The realistic Qatar infrastructure for MPS I: - **Sidra Medicine, Doha.** Paediatric-only specialty centre. Genetics service, paediatric metabolic team, multidisciplinary surveillance (cardiology, pulmonology, ophthalmology, ENT, orthopaedics), infusion suite with anaphylaxis-management capability. The natural infusion home for paediatric MPS I patients in Qatar. Sidra is a member of Qatar Foundation. - **Hamad Medical Corporation (HMC), Doha.** The public-sector backbone. Paediatric and adult metabolic services. For adult MPS I patients (attenuated Scheie diagnosed in adulthood), HMC is the operationally appropriate centre. - **Al Ahli Hospital** and the **Doha Clinic Hospital / Naseem Healthcare network** provide private-sector alternatives.

Typical regulatory and procurement timing on a complete file is 4 to 8 weeks.

The cost conversation, in the form a Qatari family needs

Aldurazyme is one of the most expensive enzyme replacement therapies on the market, and because it is administered weekly for life, the lifetime cost is what matters most.

The 2026 indicative annual list price is roughly USD 200,000 to USD 500,000 per year, or approximately QAR 728,000 to QAR 1.82 million per year, depending on your child's weight (0.58 mg/kg weekly). Over a multi-decade course for an attenuated Scheie patient, the cumulative drug cost can sit between USD 5 million and USD 15 million, before supportive-care costs.

When we issue a quote at intake, we separate every line: drug per infusion, infusion-suite charges, pre-medication, monitoring labs, our coordination fee. Nothing is bundled. We do not put a markup on the manufacturer's drug price.

For Qatari nationals being treated at Sidra Medicine under the public health system funding pathway, much of the cost may be underwritten directly; direct consultation with the Sidra patient navigator is the right path to confirm what is covered and what is not. For expatriate residents, mandatory private insurance handles specialty drug authorisation on a case-by-case prior-authorisation basis. We supply your insurer with the documentation packet at no charge.

The weekly infusion reality

Aldurazyme is a weekly intravenous infusion of approximately 3 to 4 hours including the slow titration period. Pre-medication with an antihistamine (with or without an antipyretic) is given about 60 minutes before each infusion. For long-term patients, a central venous access device is often placed. Infusion-associated reactions are common particularly during the first months; the infusion suite must have anaphylaxis-management capability on site.

For a Qatari family, weekly clinic time becomes a permanent calendar feature. The infusion centre becomes a known place. School, work, summer travel, and Ramadan all plan around the schedule.

Monitoring on therapy

The MPS I surveillance schedule on long-term Aldurazyme: urinary GAG every 3 to 6 months (primary biochemical marker), anti-laronidase antibody titre at intervals, annual 6-minute walk test, FVC, echocardiogram, ECG, ophthalmology, ENT, audiology, sleep study as indicated, orthopaedic and physiotherapy reviews, hepatosplenomegaly assessment. Sidra's multidisciplinary structure runs this in-house for paediatric patients; HMC for adult patients.

When Aldurazyme is not the right answer, or not the only answer

For severe Hurler infants, ERT alone does not address the cognitive trajectory. HSCT is the standard intervention, ideally before age 2 to 2.5; Sidra and HMC coordinate paediatric BMT and can refer for international BMT-centre evaluation where appropriate. Aldurazyme functions as a bridge before transplant and an adjunct afterwards.

For severe Hurler patients diagnosed late, after the cognitive window for HSCT benefit has closed, the honest conversation is about palliating somatic progression with ERT.

For attenuated Scheie adults, the management is closer to chronic-disease management of a multisystemic condition, with cardiac, ophthalmic, and orthopaedic interventions sometimes mattering more day-to-day than the weekly ERT.

Emerging AAV-based gene therapy programmes for MPS I are in clinical trials internationally but are not yet approved.

What Reserve Meds does for a Qatari family

Our scope for a Qatari family pursuing Aldurazyme depends on the pathway.

For Qatari nationals being treated at Sidra Medicine or HMC under public-system funding: our role is closer to a documentation and second-opinion concierge layer. Sidra and HMC handle the operational coordination; we can support with international second opinions, insurance documentation for expatriates, or translation of records.

For expatriate residents and self-pay families: standard Reserve Meds scope. Regulatory documentation, sourcing from manufacturer's authorised distribution under DSCSA chain of custody, cold-chain logistics to the infusion centre (2-8 degrees Celsius, do not freeze), qualified-centre liaison, named case-lead coordination from intake through the establishment of a stable weekly infusion routine.

For families considering cross-border BMT evaluation for severe Hurler: coordination of the BMT-centre referral alongside the ERT bridge therapy in Qatar.

Reserve Meds is not your child's prescriber. We do not practise medicine. We do not manufacture Aldurazyme. We do not own or operate any infusion centre. Clinical decisions stay with your metabolic specialist and the treating centre.

We work cash-pay (where applicable). Our coordination fee is disclosed in writing.

A note for families weighing this

For Muslim families thinking through the religious-ethical dimension, Aldurazyme is recombinant, produced in CHO cell culture, not derived from animal tissue or human plasma. The Islamic bioethics consensus on life- and function-preserving therapies is broadly permissive. Families typically consult with their religious advisors before committing.

For Qatari families with affected relatives or carrier history in the extended family, the carrier-testing conversation for siblings and cousins is a separate but important thread, and Sidra and HMC offer the appropriate genetic-counselling referrals.

What to do if you want to start

The first concrete step is a call with our case-lead so we can confirm the diagnostic stage your child is at and whether the right next move is the workup, the ERT initiation, the HSCT pathway evaluation, or a combination.

If your child has been diagnosed with MPS I but you have not yet made the severity classification decision, reach out anyway: we will help you get the workup completed at Sidra or HMC before the treatment-plan conversation.

Most families reach us first on WhatsApp, which is the medium we hold open during Qatar business hours (Sunday-Thursday) and on weekends for active cases.

Start your child's case on the portal, or open a WhatsApp conversation with the case-lead and we will take it from there.

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.
reservemeds.com · hello@reservemeds.com