Aldurazyme

Abu Dhabi · access guide

Aldurazyme (laronidase) for an Abu Dhabi family: what the pathway looks like in 2026

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

An Abu Dhabi-based family of a child with mucopolysaccharidosis type I, MPS I, has the strongest UAE infrastructure picture in 2026. Aldurazyme has a long track record in the region. The Department of Health Abu Dhabi handles the emirate-level layer alongside the federal Emirates Drug Establishment (EDE). The Abu Dhabi paediatric metabolic infrastructure at Tawam Hospital Al Ain (the longest-established UAE paediatric metabolic service), Sheikh Khalifa Medical City, Sheikh Shakhbout Medical City, Burjeel Medical City, and Cleveland Clinic Abu Dhabi covers the workup, the weekly infusion delivery, the multidisciplinary surveillance, and, for severe Hurler patients, the BMT pathway evaluation.

This page is meant to be the first honest read you get on Aldurazyme in Abu Dhabi, written by the team that would coordinate around your child's case if you decided you wanted operational support on the workup, the DoH-EDE filing, the qualified centre, or the long-term cost picture.

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 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. It does not cross the blood-brain barrier and does not address the cognitive decline of severe Hurler.

For severe Hurler infants, the standard of care is hematopoietic stem cell transplantation, HSCT, ideally before age 2 to 2.5. 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

The workup has five components: urinary GAG screen, alpha-L-iduronidase enzyme activity assay (the definitive enzymatic confirmation), IDUA gene sequencing for severity classification, baseline organ assessments (echocardiogram, FVC, sleep study, ophthalmology, ENT, hepatomegaly, joint range of motion, 6-minute walk test), and severity classification by the metabolic specialist.

In Abu Dhabi, the workup typically routes through: - **Tawam Hospital, Al Ain.** The longest-established UAE paediatric metabolic service. Has managed UAE MPS patients for many years. Strong infrastructure for the diagnostic workup, weekly infusion delivery, and multidisciplinary surveillance. - **Sheikh Khalifa Medical City (SKMC), Abu Dhabi.** Paediatric metabolic and rare-disease infrastructure. Anaphylaxis-management capability for the infusion suite. - **Sheikh Shakhbout Medical City (SSMC), Abu Dhabi.** Rare-disease infrastructure including ERT delivery. Paediatric neurology and metabolic team. - **Cleveland Clinic Abu Dhabi.** Solid-organ transplant, oncology, rare-disease coordination; paediatric services and BMT programmes for severe Hurler HSCT evaluation. - **Burjeel Medical City.** Metabolic clinic.

For confirmatory enzyme assays and IDUA sequencing, samples can be run at the larger Abu Dhabi tertiaries or sent to a regional reference laboratory. The clinical rationale letter from the treating paediatrician documents the diagnosis, the severity classification, the recommended treatment plan, and the long-term monitoring schedule.

The Abu Dhabi regulatory pathway in 2026

The Emirates Drug Establishment took over MoHAP's pharmaceutical regulatory functions in 2026. Abu Dhabi-based families file through DoH with EDE coordination on the federal layer. 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 via ede.gov.ae.

Typical regulatory and procurement timing on a complete file is 3 to 6 weeks.

For severe Hurler patients, the HSCT pathway adds another layer. Cleveland Clinic Abu Dhabi and the BMT programmes operating in the UAE in 2026 handle paediatric BMT. Some families travel to KFSHRC Riyadh or to international BMT centres for the transplant itself, with the ERT bridge managed in Abu Dhabi.

The cost conversation, in the form an Abu Dhabi 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 AED 734,000 to AED 1.84 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.

Daman is the dominant Abu Dhabi-area insurer with the Thiqa government-employee plan; the rare-disease pathway has historically been workable for benchmark ERTs like Aldurazyme. We supply your insurer with the documentation packet at no charge.

For Emirati nationals being treated at Tawam, SKMC, or SSMC under Thiqa coverage, much of the cost may be underwritten through the government health funding pathways. Your treating consultant will confirm whether and how. For expatriate residents, the financial picture is typically a mix of insurance coverage, employer support where applicable, and family-pay.

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 an Abu Dhabi family, weekly clinic time becomes a permanent calendar feature.

Monitoring on therapy

The MPS I surveillance schedule on long-term Aldurazyme: urinary GAG every 3 to 6 months, 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. Tawam, SKMC, SSMC, and Cleveland Clinic Abu Dhabi have the multidisciplinary infrastructure to run this surveillance schedule in-emirate.

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; Cleveland Clinic Abu Dhabi handles paediatric BMT, and some Abu Dhabi families travel to KFSHRC Riyadh or to international BMT centres of excellence. Aldurazyme functions as a bridge before transplant and an adjunct afterwards.

For severe Hurler patients diagnosed late, 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.

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

What Reserve Meds does for an Abu Dhabi family

For Emirati nationals being treated at Tawam, SKMC, or SSMC under Thiqa coverage, our role is closer to a documentation and second-opinion concierge layer. The treating centre handles the operational coordination; we can support with international second opinions, insurance documentation, translation of records, and cross-border BMT-centre referral evaluation for severe Hurler.

For expatriate residents and self-pay families, our scope is the standard regulatory documentation packet, the DoH-EDE filing in collaboration with your treating hospital's import pharmacy, the sourcing logistics from the manufacturer's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the Abu Dhabi infusion centre (2-8 degrees Celsius, do not freeze), and named case-lead coordination.

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

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.

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, the severity classification if it has been made, and whether the right next move is the workup, the ERT initiation, the HSCT pathway evaluation, or a combination.

Most families reach us first on WhatsApp, which is the medium we hold open during UAE business hours 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.

reservemeds.com · hello@reservemeds.com