Aldurazyme

United Arab Emirates · access guide

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

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

A UAE family of a child with mucopolysaccharidosis type I, MPS I, walks into this decision with more than a treatment question. There is a clinical question about which form of MPS I the child carries and what that means for the therapy plan. There is a regulatory question about how Aldurazyme moves into the country. There is a financial question that is multi-decade, not multi-month. And there is a family question, often a heavy one, because in many MENA families MPS I has appeared in a relative before this child. This page is meant to be the first honest read you get on Aldurazyme in the UAE, written by the team that would coordinate around your child's case if you decided you wanted operational support on the workup, the import, the qualified infusion centre, or the long-term cost picture.

We will be specific about what MPS I is, what the workup decides, what the difference between severe Hurler and attenuated Scheie means for the treatment plan, what the regulatory pathway looks like in the UAE in 2026, what it costs in AED and US dollars, where the infusion can be given in the UAE, and what life looks like for a family settling into weekly lifelong infusions.

What MPS I actually is, in plain terms

Mucopolysaccharidosis type I, MPS I, is a lysosomal storage disorder. The IDUA gene normally produces an enzyme called alpha-L-iduronidase, which lives inside the lysosomes (the cellular recycling compartment) and breaks down two long-chain sugars called dermatan sulfate and heparan sulfate. When IDUA is faulty, those sugars accumulate, slowly, in every tissue. The accumulation is what causes the disease.

MPS I presents on a spectrum. Severe Hurler syndrome, the most-severe form, presents in infancy with coarse facial features, an enlarged liver and spleen, recurrent ear and chest infections, progressive heart valve disease, skeletal changes called dysostosis multiplex, and, critically, progressive cognitive decline. Hurler-Scheie syndrome, the intermediate form, presents later with the somatic features but normal or near-normal cognition. Scheie syndrome, the attenuated form, can be diagnosed in adolescence or adulthood, often with corneal clouding, joint stiffness, and milder somatic involvement.

The form your child has matters because Aldurazyme does not cross the blood-brain barrier. The enzyme reaches the heart, the lungs, the liver, the spleen, and the skeletal-somatic tissues, but it 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 years, because donor bone marrow cells produce enzyme that does cross into the CNS through engrafted microglia. Aldurazyme is used as a bridge to HSCT (controlling somatic disease during the months between diagnosis and transplant) and often as an adjunct afterwards. For Hurler-Scheie and Scheie patients, ERT alone is typically the long-term answer.

We mention this first because it is the most important piece of information for your family. Your metabolic specialist will tell you which form of MPS I your child has based on the IDUA mutation profile and the early clinical picture. The treatment plan that follows is built on that classification.

The workup that decides eligibility and shapes the plan

Many UAE families arrive at Reserve Meds at different diagnostic stages. Some have a complete MPS I workup already on file from Tawam Hospital in Al Ain, from SKMC or SSMC, or from a paediatric metabolic service abroad. Others have been managed for individual organ manifestations for years, an enlarged liver here, recurrent ear infections there, joint stiffness, and the unifying MPS I diagnosis has not yet been confirmed. Both starting points are workable.

The diagnostic and pre-treatment workup has five components.

First, urinary glycosaminoglycan screen. Elevated urinary dermatan sulfate and heparan sulfate is the cheap, accessible first screen.

Second, alpha-L-iduronidase enzyme activity assay in leukocytes or fibroblasts. This is the definitive enzymatic confirmation. Deficient or absent activity diagnoses MPS I.

Third, IDUA gene sequencing. Identifies the specific pathogenic variants and helps with severity classification, family carrier testing, and prognostic conversations. Tawam Hospital's Genetics service, SKMC's paediatric metabolic team, and the metabolic genetics service at SSMC have run this workup on UAE soil for many MPS families.

Fourth, baseline organ assessments. Echocardiogram for cardiac valve disease, pulmonary function testing including FVC, sleep study for apnea, ophthalmology for corneal clouding, ENT for upper airway and hearing, hepatomegaly assessment, joint range-of-motion documentation, baseline 6-minute walk test. These become the surveillance schedule for the rest of your child's life on therapy.

Fifth, severity classification by the metabolic specialist. Severe Hurler, Hurler-Scheie, or Scheie. The HSCT-versus-ERT-alone decision flows from this classification.

A clinical rationale letter from your metabolic specialist documents the diagnosis, the severity classification, the recommended treatment plan, and the long-term monitoring schedule.

The UAE regulatory pathway: how it actually works in 2026

The Emirates Drug Establishment, which absorbed 44 of the Ministry of Health and Prevention's regulatory functions by early 2026, is now the federal authority that the treating hospital files through. Aldurazyme has a long track record in MENA and [VERIFY: current EDE registration status 2026]; where formal registration is in place, standard prescription and import procurement applies, and where not, the named-patient mechanism is filed via ede.gov.ae by the hospital's import pharmacy on the treating physician's behalf. The Department of Health Abu Dhabi or the Dubai Health Authority adds the emirate-level layer depending on where the infusion is given.

In our experience coordinating MPS I cases for UAE families, EDE coordination on a complete, well-documented rare-disease file runs three to six weeks from filing to approval. Aldurazyme is treated as a benchmark rare-disease ERT and the regulatory framework is workable for it.

The realistic UAE infrastructure for MPS I: - **Tawam Hospital, Al Ain.** Tawam has a longstanding paediatric metabolic service that has managed UAE MPS patients for years. Strong infrastructure for the diagnostic workup, the weekly infusion schedule, and the multidisciplinary surveillance. - **Sheikh Khalifa Medical City, Abu Dhabi.** Paediatric metabolic and rare-disease infrastructure. Anaphylaxis-management capability for the infusion suite. - **Sheikh Shakhbout Medical City, Abu Dhabi.** Rare-disease infrastructure including ERT delivery. Paediatric neurology and metabolic team. - **Burjeel Medical City, Abu Dhabi.** Metabolic clinic. - **American Hospital Dubai.** Paediatric service capable of weekly ERT delivery for Dubai-side families. - **Mediclinic City Hospital, Dubai.** Paediatric subspecialty depth.

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 the UAE. Reserve Meds can coordinate either pattern.

The cost conversation, in the form a UAE 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 picture is what matters more than the per-infusion price.

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 (dosing is 0.58 mg/kg weekly). A 10 kg toddler runs at the low end; a 70 kg adolescent Scheie patient runs at the upper end. Over a multi-decade therapy course for an attenuated-Scheie patient, the cumulative drug cost can sit between USD 5 million and USD 15 million, before accounting for the surveillance schedule, valve replacements, corneal transplants, orthopaedic interventions, and other supportive care that almost every MPS I patient eventually needs.

When we issue a quote at intake, we separate every line: drug per infusion, infusion-suite charges, pre-medication, monitoring labs (urinary GAG, anti-laronidase antibody titre, organ surveillance), our coordination fee. Nothing is bundled. We do not put a markup on the manufacturer's drug price. We charge a transparent coordination fee for the case-management work, disclosed in writing before any funds move.

Insurance coverage of Aldurazyme in the UAE is uneven. Daman has approved cases through prior authorisation for certain employer plans and Thiqa-covered Emirati nationals; private insurers vary widely. The Daman rare-disease pathway has historically been workable for benchmark ERTs like Aldurazyme. We supply your insurer with the documentation packet at no charge. We do not process the claim or guarantee coverage.

For Emirati nationals being treated at SKMC or Tawam under the public system, 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 cost 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 to make the weekly access manageable.

Infusion-associated reactions are common, particularly during the first months of therapy. The infusion suite must have anaphylaxis-management capability on site, with epinephrine, oxygen, IV access, trained nursing, and physician availability. Over time the reaction frequency typically decreases, but it does not go to zero.

For a UAE family, the practical implication is that weekly clinic time is a permanent feature of the calendar. School schedules, work schedules, extended-family visits, summer travel, and Ramadan are all planned around the infusion schedule. The infusion centre becomes a known place, and the nursing team becomes part of the family network. This is something many MPS families say openly once they have settled into it.

Monitoring on therapy

The surveillance schedule on long-term Aldurazyme is built around the multisystemic nature of MPS I: - Urinary GAG every 3 to 6 months (the primary biochemical efficacy marker). - Anti-laronidase antibody titre at intervals; some patients develop antibodies and the clinical implications are variable. - 6-minute walk test annually (functional efficacy). - FVC annually (pulmonary). - Echocardiogram annually (cardiac valve surveillance). - ECG annually. - Ophthalmology annually (corneal clouding). - ENT and audiology annually. - Sleep study as clinically indicated. - Orthopaedic and physiotherapy reviews on a schedule set by the team. - Hepatosplenomegaly assessment.

Tawam, SKMC, and SSMC have the multidisciplinary infrastructure to run this surveillance schedule in-emirate. For Dubai-side families, American Hospital Dubai and Mediclinic City coordinate the multidisciplinary visits within the city.

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

For severe Hurler infants, ERT alone does not address the cognitive trajectory. The conversation your family will have with the metabolic specialist will include HSCT timing, donor matching, the BMT centre choice, and the role of Aldurazyme as bridge therapy before transplant and adjunct therapy after. We will not pretend this is a small decision. We can coordinate the BMT centre referral alongside the ERT pathway.

For severe Hurler patients who are diagnosed late, after the cognitive window for HSCT benefit has closed, the honest conversation is about palliating somatic progression with ERT while accepting that the CNS trajectory is on a different track. Your metabolic specialist will frame this candidly. Reserve Meds will support whatever the family and the treating team decide.

For patients with attenuated Scheie syndrome diagnosed in adulthood, the management picture is often closer to chronic-disease management of a multisystemic condition, with the cardiac, ophthalmic, and orthopaedic interventions sometimes mattering more day-to-day than the weekly ERT itself.

For families pursuing emerging therapies, the AAV-based gene therapy programmes for MPS I are in clinical trials internationally but are not yet approved. We can talk through trial eligibility and access where it applies, but we will not present trial therapy as routinely available.

What Reserve Meds does, and what we do not do

Reserve Meds is a US-based concierge coordinator for cross-border and complex specialty medicine. For a UAE family pursuing Aldurazyme, our scope is the regulatory documentation packet, the 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 qualified UAE centre (2-8 degrees Celsius, do not freeze), and named case-lead coordination from intake through the establishment of a stable weekly infusion routine. We continue to coordinate refills, documentation, and any cross-border travel for second opinions or for HSCT centre evaluation where it applies.

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. We are not your insurer. Clinical decisions stay with your metabolic specialist and the infusion centre team; we are the operational layer that turns those decisions into a coordinated case.

We work cash-pay (where applicable). Our coordination fee is disclosed in writing. We will not start work without a signed engagement.

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, and the recombinant production removes some of the dietary-law concerns that arise with some plasma-derived products. Families typically consult with their religious advisors before committing; we will not pressure that conversation.

For families who already have an affected child or relative, the carrier-testing conversation for siblings and for the extended family is a separate but important thread, and your metabolic specialist will offer the appropriate 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, 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.

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 in-emirate before the treatment-plan conversation.

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.

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