Aldurazyme

Saudi Arabia · access guide

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

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

A Saudi 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 reaches the dispensing hospital. There is a financial question that is multi-decade for an attenuated patient. And there is a family question, often heavy, because MPS I, like many autosomal recessive lysosomal storage disorders, has a meaningful prevalence pattern in populations with historical consanguineous marriage and many Saudi families presenting with a Hurler child have already lost an older sibling or have known affected relatives. This page is meant to be the first honest read you get on Aldurazyme in the Kingdom of Saudi Arabia, written by the team that would coordinate around your child's case if you decided you wanted operational support on the workup, the SFDA filing, the qualified 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, what the regulatory and procurement pathway looks like in the kingdom in 2026, what it costs in SAR and US dollars, where the infusion can be given, and what life looks like for a family settling into weekly lifelong therapy.

What MPS I actually is, in plain terms

Mucopolysaccharidosis type 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.

MPS I presents on a spectrum. Severe Hurler syndrome presents in infancy with coarse facial features, an enlarged liver and spleen, recurrent ear and chest infections, progressive heart valve disease, skeletal dysostosis multiplex, and progressive cognitive decline. Hurler-Scheie syndrome, the intermediate form, presents later with somatic features but normal or near-normal cognition. Scheie syndrome, the attenuated form, is sometimes diagnosed in adolescence or adulthood.

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 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.

We mention this first because it is the most important 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 flows from that classification.

The workup that decides eligibility and shapes the plan

Saudi families arrive at Reserve Meds at different diagnostic stages. KFSHRC's genetics service in Riyadh has run substantial portions of the Saudi MPS I clinical and research base, including the natural-history work on Saudi MPS cohorts. KAMC under National Guard Health Affairs has comparable infrastructure. King Fahad Medical City Riyadh and the Saudi MPS network of tertiary centres have managed MPS patients for many years.

The 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. KFSHRC has historically been the Saudi reference centre for IDUA mutation work. The Saudi mutation spectrum for MPS I has been characterised in the published literature; certain pathogenic variants are over-represented in Saudi cohorts due to founder effects, which can shorten the diagnostic pathway when the molecular team recognises the patterns.

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.

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 Saudi regulatory and procurement pathway in 2026

The Saudi Food and Drug Authority (SFDA) is the registration and import authority for medicines in the kingdom. Aldurazyme has been available in the region for many years. [VERIFY: current SFDA registration status of Aldurazyme 2026]; where formal registration is in place, standard prescription and import procurement applies, and where the product moves through the named-patient mechanism, the application is filed through SFDA's drug.sfda.gov.sa portal by the dispensing hospital's licensed pharmacist on the consultant's behalf.

NUPCO, the National Unified Procurement Company, sits in the procurement loop for public-sector hospitals. For Saudi-national families being treated at KFSHRC, KAMC, or King Fahad Medical City, the procurement layer may run through NUPCO with public-system funding. For private-sector cases or for cross-border named-patient arrangements, NUPCO is typically not in the loop.

Typical regulatory and procurement timing on a complete file is 4 to 8 weeks for a rare-disease ERT. Aldurazyme is treated as a benchmark ERT and the framework is workable.

The realistic Saudi infrastructure for MPS I: - **King Faisal Specialist Hospital and Research Centre (KFSHRC) Riyadh.** The historical centre of gravity for Saudi rare-disease and metabolic work. Paediatric metabolic service with multi-decade experience managing MPS patients. BMT programme support for the severe Hurler HSCT pathway. Infusion suite with anaphylaxis-management capability. - **King Abdulaziz Medical City (KAMC, Riyadh).** National Guard Health Affairs flagship. Paediatric metabolic and genetics service. ERT delivery infrastructure. - **King Fahad Medical City, Riyadh.** Paediatric specialty depth, metabolic clinic, ERT delivery infrastructure. - **KFSHRC Jeddah.** Sister facility for the western region, same clinical standards. - **Dr Sulaiman Al-Habib Medical Group.** Private-sector tertiary network with paediatric subspecialty depth for families preferring private-sector care.

For severe Hurler patients, KFSHRC Riyadh and KAMC operate paediatric BMT programmes capable of taking the HSCT pathway in-country. For some families, cross-border BMT (to international centres of excellence) is preferred; Reserve Meds can coordinate either pattern.

The cost conversation, in the form a Saudi 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 SAR 750,000 to SAR 1.88 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.

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. We charge a transparent coordination fee for the case-management work, disclosed in writing before any funds move.

Insurance coverage of Aldurazyme in Saudi Arabia varies. For public-sector cases at KFSHRC, KAMC, or King Fahad Medical City, much of the cost can be underwritten through the public health system. Vision 2030 rare-disease frameworks have supported high-cost specialty therapies for Saudi-national families at flagship hospitals; your treating consultant will know if any current framework applies. Private insurers (Bupa Arabia, Tawuniya, MedGulf, Walaa) handle rare-disease ERTs on a case-by-case prior-authorisation basis. We supply the prior-authorisation documentation packet to your insurer at no charge.

For Saudi-national families being treated at a tertiary public-sector centre, the financial framing is often very different from cash-pay; for expatriate residents and for cases routing through private-sector centres or international referral, the cash-pay-with-documentation pattern applies.

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 of therapy. The infusion suite must have anaphylaxis-management capability on site. Over time the reaction frequency typically decreases, but it does not go to zero.

For a Saudi family, the practical implication is that weekly clinic time is a permanent feature of the calendar. The infusion centre becomes a known place. School schedules, work schedules, extended-family visits, summer travel, and Ramadan are all planned around the infusion schedule.

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. - Anti-laronidase antibody titre at intervals. - 6-minute walk test annually. - FVC annually. - Echocardiogram annually. - ECG annually. - Ophthalmology annually. - ENT and audiology annually. - Sleep study as clinically indicated. - Orthopaedic and physiotherapy reviews on a schedule set by the team. - Hepatosplenomegaly assessment.

KFSHRC, KAMC, and King Fahad Medical City have the multidisciplinary infrastructure to run this surveillance schedule in-house.

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 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. KFSHRC Riyadh and KAMC operate paediatric BMT programmes; some Saudi families travel to international BMT centres of excellence.

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 while accepting that the CNS trajectory is on a different track.

For Scheie patients diagnosed in adulthood, the management picture is closer to chronic-disease management of a multisystemic condition.

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.

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 Saudi family pursuing Aldurazyme, our scope is the regulatory documentation packet, the SFDA filing in collaboration with your consultant and the dispensing hospital's pharmacist, the sourcing logistics from the manufacturer's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the qualified Saudi centre (2-8 degrees Celsius, do not freeze), and named case-lead coordination from intake through the establishment of a stable weekly infusion routine.

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; we are the operational layer.

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, 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.

For Saudi families with affected relatives or known carrier history in the extended family, the carrier-testing conversation for siblings and cousins is a separate but important thread, and your metabolic specialist will 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, 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 the severity classification has not yet been finalised, reach out anyway: we will help you get the workup completed at KFSHRC or KAMC before the treatment-plan conversation.

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

reservemeds.com · hello@reservemeds.com