# Elaprase access in Qatar

How families in Qatar pursue idursulfase, approximately manufactured by Takeda (acquired from Shire), as long-term enzyme replacement therapy for Hunter syndrome (mucopolysaccharidosis type II), via the Ministry of Public Health's named-patient pathway.

*Last reviewed 2026-05-12 by Reserve Meds clinical & regulatory team. This page combines the Qatar country research module with the Elaprase drug module to describe the path families actually walk.*

## Quick orientation

Elaprase (idursulfase) is a recombinant form of human iduronate-2-sulfatase, the lysosomal enzyme that is deficient in Hunter syndrome (mucopolysaccharidosis type II, MPS II), an X-linked lysosomal storage disorder that affects primarily males. The US Food and Drug Administration approved Elaprase in July 2006 for the treatment of patients with Hunter syndrome to improve walking capacity. Elaprase is approximately manufactured and marketed globally by Takeda, which acquired the original developer Shire in 2019. The therapy is delivered as a weekly intravenous infusion at a typical dose of 0.5 mg per kilogram of body weight, administered over approximately three hours. For a Qatar family with a child diagnosed with Hunter syndrome, the practical question is rarely the science. It is the access path and the weekly infusion logistics. Reserved for you.

## Why this drug is hard to source in Qatar

Hunter syndrome is rare, with an estimated incidence of approximately one in 100,000 to one in 170,000 male births globally. Consanguineous marriage patterns in the Gulf region can shift the regional prevalence picture for genetic disorders compared with global baselines, and Qatar's national genomics work has been generating clearer epidemiological signal. Even so, the prevalent MPS II patient population in Qatar is small. Elaprase carries an established marketing authorisation in many countries and historically has been accessible to Qatari MPS II patients through specialty hospital channels, but whether it is held in routine inventory varies by hospital and by the year. In any month where a Qatar family receives a Hunter syndrome diagnosis, the treating pediatric metabolic specialist will typically initiate the named-patient pathway in parallel with the family's first conversation about enzyme replacement.

The condition itself amplifies the access urgency. Hunter syndrome progresses in early childhood with somatic features (coarse facial features, joint stiffness, hepatosplenomegaly,

hearing loss, cardiac valve disease, airway disease) and, in the severe (early-onset, neuronopathic) form, central nervous system involvement. Idursulfase does not cross the blood-brain barrier in clinically meaningful quantities, so its primary clinical effect is on somatic disease progression. The therapeutic window where weekly enzyme replacement can preserve walking distance, joint range, and cardiopulmonary function is real, and every month without infusion is a month of accumulating glycosaminoglycan storage.

## The MOPH-PDCD named-patient pathway

The federal pathway for a Qatar-licensed physician to obtain a medicine that is not registered or not stocked locally is the named-patient import permit, administered by the Pharmacy and Drug Control Department (PDCD) within the Ministry of Public Health. The framework allows hospitals and licensed pharmaceutical establishments to import a specific medicine for a specific patient when the medicine is approved by a recognised reference authority such as the US FDA or the European Medicines Agency and a clinically equivalent locally registered alternative is not suitable. For a rare lysosomal storage disorder like Hunter syndrome, the regulatory analysis is straightforward because alternative therapies do not exist for the somatic disease in MPS II.

A complete PDCD application for an Elaprase case typically includes the clinical justification letter from the treating pediatric metabolic specialist (IDS gene variant confirmation, documented enzymatic confirmation showing deficient iduronate-2-sulfatase activity in leukocytes or fibroblasts, urinary glycosaminoglycan profile, somatic disease burden, prior therapy attempts where applicable), the treating physician's Qatar Council for Healthcare Practitioners (QCHP) license verification, an anonymised patient identifier or Qatar ID where the PDCD submission allows, full product details for idursulfase (brand name Elaprase, 6 mg per 3 mL single-dose vial, quantity requested, intended weekly treatment), the destination dispensing facility name with MOPH pharmacy license number, and a cold-chain plan documenting temperature handling at 2 to 8 degrees Celsius from US release through to the Qatar infusion suite. Approval timelines for routine pediatric metabolic cases through Hamad Medical Corporation (HMC) or Sidra Medicine are typically 2 to 4 weeks for first applications; subsequent monthly or quarterly re-supply runs on the standing permit framework.

## Real costs in QAR and USD

Elaprase is one of the most expensive medicines globally. US list pricing for idursulfase falls broadly in the USD 300,000 to USD 600,000 per patient per year range depending on body weight, with body weight as the dominant cost driver because dosing is weight-based at 0.5 mg per kilogram weekly. In QAR at the 3.64 peg, that is approximately QAR 1.1 million to QAR

2.2 million per patient per year. For a growing child, the annualised cost scales upward each year as weight increases. Therapy is long-term, in many cases lifelong.

International logistics for cold-chain biologic shipments typically runs USD 800 to 2,500 per shipment depending on quantity, urgency, and packaging certification, or approximately QAR 3,000 to QAR 9,100. Reserve Meds quotes the actual logistics line on every firm quote. Qatar customs and PDCD permit fees are nominal relative to the drug cost. For Qatari nationals receiving care at HMC, public-sector subsidy is the dominant financial mechanism and the patient's out-of-pocket exposure is typically limited. For expatriate families, employer-sponsored insurance through carriers such as Qatar Insurance Company, Allianz Care, Cigna, AXA, Bupa Global, or MetLife handles rare-disease enzyme replacement therapy case by case. We supply the documentation set that lets your insurer assess the case. We do not promise coverage from any insurer.

## Timing — what to expect

For a Qatar family initiating Elaprase, the timing question has two distinct windows. The PDCD permit window for a first application through HMC's pediatric metabolic service or Sidra Medicine typically processes in 2 to 4 weeks. Cold-chain international shipping from the US, including chain-of-custody documentation, validated cold-chain packaging, and customs clearance into Doha, runs approximately 5 to 10 business days. The treating physician's clinical workup, including IDS gene variant confirmation, enzymatic assay confirmation, baseline cardiac and pulmonary assessment, ENT evaluation, and the family's informed-consent conversation including discussion of infusion-related reactions and antibody monitoring, occurs in parallel. Reserve Meds frames the working assumption as a 4 to 8 week first-infusion window from intake to dispense, with weekly re-supply cycles thereafter running shorter because the documentation history is on file.

## What your physician needs

The clinical justification letter for an Elaprase PDCD submission addresses the patient's diagnosis (Hunter syndrome confirmed by deficient iduronate-2-sulfatase enzyme activity in leukocytes or fibroblasts, combined with IDS gene variant confirmation), the documented somatic disease burden (cardiac involvement on echocardiogram, hepatosplenomegaly, joint stiffness, walking distance on six-minute walk test, respiratory function, hearing assessment, dysmorphic features), and the rationale for enzyme replacement therapy at this point in the patient's disease course. The dose calculation (0.5 mg per kilogram per week given as a three-hour intravenous infusion, with infusion rate titration starting at approximately 8 mL per hour and increasing every 15 minutes as tolerated) is included, along with the proposed infusion-suite plan and the monitoring framework for infusion-associated reactions, anti-drug antibodies, and somatic outcome measures over time.

The treating physician's QCHP license must be in active standing. For pediatric Hunter syndrome cases in Qatar, the natural treating physicians are pediatric metabolic specialists at Sidra Medicine, where the Qatar Foundation-affiliated pediatric academic medical center holds genetics, biochemical genetics, and lysosomal storage disease expertise, or at Hamad Medical Corporation's pediatric services. Adult MPS II patients (those with the attenuated form who reach adulthood) typically transition to adult metabolic or genetic medicine services within the HMC network. The QCHP license number, the institutional pharmaceutical-establishment license of the dispensing pharmacy, and the family's informed-consent record sit alongside the clinical letter in the PDCD submission.

## Hamad Medical Corporation and Sidra Medicine specialty dispensing

Elaprase dispensing in Qatar is overwhelmingly pediatric and concentrated at Sidra Medicine and HMC pediatric services. Sidra Medicine's genetics, metabolic, and biochemical genetics services hold the deepest local expertise in lysosomal storage disorders, and Sidra operates its own JCI-accredited pharmacy services with cold-chain biologic infrastructure. Sidra typically files PDCD named-patient applications directly. Hamad General Hospital's pediatric and metabolic services within the HMC network also handle Hunter syndrome cases. The infusion suite must hold cold-chain storage for the vials, an infusion pump suitable for the slow-rate paediatric infusion, immediate-availability emergency drugs in the event of an infusion-related reaction, and the staffed capacity for a three-hour outpatient infusion every week.

Re-supply for chronic Elaprase maintenance is built into the PDCD permit framework at the application stage. Reserve Meds typically structures shipments to land monthly so that the infusion suite holds approximately four weeks of vials at any given time, balancing inventory cost against re-supply risk.

## Pharmacovigilance and cold-chain

Elaprase is a cold-chain biologic. Idursulfase vials are stored at 2 to 8 degrees Celsius and protected from freezing. The chain-of-custody documentation tracks lot, expiry, and temperature exposure across every handoff from the US specialty wholesaler through international transit to the Qatar importer's bonded warehouse to the hospital pharmacy and onward to the infusion suite. A documented temperature excursion can trigger a quarantine and replacement cycle. PDCD pharmacovigilance reporting obligations remain with the treating physician and the dispensing facility. Infusion-related reactions, anaphylaxis, hypotension, urticaria, pyrexia, and any serious unexpected event are reportable to PDCD's Pharmacovigilance Center within 15 calendar days. Anti-idursulfase antibodies are monitored periodically as part of the long-term clinical course; antibody-

positive status is documented but does not on its own trigger therapy discontinuation in the standard MPS II protocol.

Reserve Meds supplies the US-side release documentation, the validated cold-chain packaging, the chain-of-custody packet, and the shipping temperature trace to the Qatar importer and to the hospital pharmacy on receipt. We do not file adverse-event reports on the physician's behalf; that obligation sits with the treating physician and the dispensing facility under the PDCD framework.

## Common questions about Elaprase in Qatar

**Will my Qatar national insurance or employer plan cover Elaprase?** For Qatari nationals receiving care at Hamad Medical Corporation or Sidra Medicine, public-sector funding is the dominant mechanism for ultra-rare lysosomal storage disorder enzyme replacement therapy. Out-of-pocket exposure is typically limited. For expatriates, employer-sponsored plans through Qatar Insurance Company, Allianz Care, Cigna, AXA, Bupa Global, or MetLife handle ultra-rare orphan therapies case by case, and pre-authorisation is the norm. We do not promise coverage from any insurer.

**Is hematopoietic stem cell transplant an alternative to Elaprase?** Hematopoietic stem cell transplant has been explored in MPS II with mixed outcomes for the somatic disease and uncertain benefit for CNS disease in the severe phenotype. The decision rests with the treating pediatric metabolic specialist in coordination with hematology. Reserve Meds does not steer the clinical decision; we coordinate access to the regimen the treating team chooses.

**Is Elaprase a controlled substance?** No. Idursulfase is not a DEA scheduled substance. The PDCD pharmacovigilance, chain-of-custody, and cold-chain requirements apply.

**What about anti-idursulfase antibodies?** Most patients develop anti-idursulfase IgG antibodies during long-term therapy. Antibody-positive status alone does not trigger discontinuation under standard MPS II protocols, although the treating physician monitors clinical response over time.

## Where Reserve Meds fits in Elaprase cases

Reserve Meds is a US-based concierge coordinator. We do not replace the treating pediatric metabolic specialist, PDCD, the dispensing pharmacy, or the QCHP-licensed institution. For an Elaprase case specifically, our work is the documentation kit assembly, the US-side DSCSA-compliant specialty wholesaler sourcing, the validated cold-chain shipment plan, the customs and import-permit coordination with the Qatar importer, and one named coordinator through the case. We hold the same coordinator across the year-on-year re-

supply cycles so that the family does not re-explain the case at every shipment. Reserved for you.

## Next step

If a treating pediatric metabolic specialist in Qatar is weighing Elaprase for a child with Hunter syndrome, the waitlist is the first step. We respond within 24 to 48 hours with an eligibility confirmation and a documentation kit for the physician.

*Reserved for you.*

## Related

- Elaprase clinical resource
- Elaprase in the UAE
- Elaprase in Saudi Arabia
- Qatar country page

## Sources


1. FDA approval, Elaprase (idursulfase), approximately Takeda (acquired from Shire), July 2006, for Hunter syndrome (mucopolysaccharidosis type II).
2. Qatar Ministry of Public Health, Pharmacy and Drug Control Department (PDCD), published guidance on named-patient and unregistered-medicine import permits.
3. Qatar Council for Healthcare Practitioners (QCHP), licensing framework and physician registration requirements.