Elevidys

Qatar · access guide

Elevidys (delandistrogene moxeparvovec) 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 Elevidys for a son with Duchenne muscular dystrophy are in a different position from families in most of the rest of the region. The therapy is approved by the Qatar Ministry of Public Health for ambulatory pediatric patients aged 4 and older, and Sidra Medicine in Doha is the first hospital outside of the United States, the fifth globally, to administer Elevidys. As of 2026, Sidra has treated ten DMD patients through its dedicated paediatric gene therapy program. The real-world safety and efficacy data from that cohort have been published in the peer-reviewed gene therapy literature. There is no GCC country, and very few non-US countries anywhere, with deeper documented capability for this specific therapy in this specific patient population.

This page is meant to be the first honest read you get on Elevidys in Qatar, written by the team that would coordinate around your son's case if you decided you wanted an outside perspective on the workup, the international second opinion, or, for some families, a different infusion centre entirely. We assume your paediatric neurologist has either raised it with you or you have raised it with them.

We will be specific about what changed in 2025 about who Elevidys is currently approved for, what the workup decides, what it costs in QAR and US dollars, how the Sidra Medicine pathway actually works, and where Reserve Meds genuinely adds value for a Qatari family (and where it does not).

What changed in 2025, and what it means for your son

In June 2025, after two fatal acute liver failure events in non-ambulatory patients who had been treated with Elevidys, Sarepta voluntarily paused distribution for non-ambulatory boys. In July 2025, the FDA placed Elevidys on a brief clinical hold following a third death from acute liver failure; the hold was lifted on 28 July 2025 with a new boxed warning, the strongest warning the FDA issues, and a narrowed approved indication. As of 2026, both the FDA-approved indication and the Qatar MOPH-approved indication for Elevidys are ambulatory boys aged 4 and older with a genetically confirmed DMD mutation. Non-ambulatory patients are not currently treated at Sidra Medicine outside of specific trial settings.

If your son is still walking, even imperfectly, you are inside the current indication. If your son has lost ambulation, Elevidys is not currently the answer at Sidra or anywhere else, but there are other paths that may be, and we'd be glad to talk those through with you. Exon-skipping therapies for eligible mutations (Exondys 51 for exon 51, Vyondys 53 and Viltepso for exon 53, Amondys 45 for exon 45, depending on the specific mutation), supportive-care optimisation, and emerging therapies in late-stage development each have a place for different patient subgroups. Reach out and we will walk through your son's specific picture before drawing any conclusions.

The under-4 group is also outside the current approved indication. Sidra's published cohort included patients aged 4-5 and 9-11, with the older group reflecting the original FDA expanded-age cohort and the younger group reflecting the early-age subset. Families often ask about waiting until age 4. The answer your paediatric neurologist will give is that the window is age- and stage-sensitive. The benefit of the therapy is highest when there is still dystrophin-producing muscle mass to preserve.

What Elevidys actually is, in plain terms

Elevidys is a single intravenous infusion. The active ingredient is an adeno-associated virus, type rh74, engineered to carry a shortened version of the dystrophin gene called micro-dystrophin. Once infused, the virus delivers that gene to muscle cells, which begin producing a shorter, partially functional version of dystrophin protein. The native DMD gene is too large to package into the virus, which is why the therapy uses a shortened construct designed by Sarepta in collaboration with the Nationwide Children's Hospital team that originated this approach.

What Elevidys is not is a cure. The clinical data describe a disease-modifying therapy: a slowing of functional decline against the natural history of DMD, with variability across patients. Your neurologist will walk you through the most recent functional data, including the EMBARK study, the Sidra Qatar real-world cohort, and the long-term international follow-up. We do not put numbers in marketing form on this page because the honest comparison for your family is your son's current North Star Ambulatory Assessment score against the patient subgroups in the published data, not an averaged headline number.

The workup that decides eligibility

Before any of the rest of the pathway opens, three results need to land.

First, genetic confirmation of a DMD-causing mutation. If your son has already been genetically tested, your neurologist will pull the report; if not, this is the first appointment. Sidra Medicine's genetics service runs the full workup in-house, and most paediatric neuromuscular cases referred to the Gene Therapy Center for Pediatric Rare Diseases at Sidra will move through that service.

Second, anti-AAVrh74 antibody serology. A meaningful fraction of children have pre-existing antibodies to the AAVrh74 viral vector from prior environmental exposure. A positive titre is a contraindication. Sidra runs the test in-country with reference-lab turnaround.

Third, baseline hepatic and cardiac function. The 2025 boxed warning makes this non-negotiable. Active hepatitis, elevated transaminases, prior liver injury, and concurrent hepatotoxic medications all need to be assessed and, where present, addressed before the infusion is scheduled. Sidra's multidisciplinary DMD gene therapy program includes hepatology and cardiology baseline workups as part of standard pre-infusion preparation.

A clinical rationale letter from your paediatric neurologist documents all three findings, the North Star score and other functional baselines, the rehabilitation plan, and the requested treatment.

The Sidra Medicine pathway, in practice

Sidra Medicine, a member of Qatar Foundation, has been administering Elevidys since 2024 and reported its 10th paediatric DMD case in early 2026. The Gene Therapy Center for Pediatric Rare Diseases, established in 2025, is the formal home of the programme. Workflow looks like this:

- **Referral.** Your paediatric neurologist refers the case to Sidra's paediatric neuromuscular team. Self-referral is also possible for families who want to start with Sidra directly. - **Workup at Sidra.** Genetic confirmation, antibody screen, hepatic and cardiac baselines, North Star score, rehabilitation review, and a peri-infusion plan are completed in-house, typically over two to four weeks. - **MOPH coordination.** Because Elevidys is on the MOPH approved list, the regulatory layer is procurement and import authorisation rather than a named-patient application. Sidra's import pharmacy handles this with MOPH's Department of Pharmacy and Drug Control. - **Infusion.** The infusion is administered at Sidra under the multidisciplinary peri-infusion protocol. Hospital admission for the immunomodulation start and overnight monitoring is standard. - **Six-month follow-up.** Sidra runs the post-infusion monitoring schedule in-house: weekly liver function panels months 1-3, biweekly through month 6, cardiac surveillance, and functional reassessment.

Published Qatar real-world data: of the ten patients treated, no severe adverse events were reported through follow-up, with corticosteroid-induced weight gain as the most common manageable issue. That is a meaningful signal for families considering this therapy.

The cost conversation, in the form a Qatari family needs

The Elevidys drug price in 2026 sits in an indicative range of roughly USD 3.0 to 3.5 million, or approximately QAR 10.9 to 12.7 million, for the one-time infusion product itself. That is the manufacturer's price for the gene therapy. The full cost of care includes the pre-infusion workup, the infusion-day admission, the peri-infusion immunomodulation protocol, the intensive monitoring schedule for the first six months, and any travel costs if you are not Doha-based.

For Qatari nationals being treated at Sidra Medicine under Qatar's public health system, the financial structure is often very different from international cash-pay coordination. Qatar Foundation and the Ministry of Public Health have at times underwritten gene-therapy cases for Qatari nationals, particularly within the Sidra programme. Direct consultation with the Sidra patient navigator is the right path to confirm what is currently available for your son's case. We will not speculate about the public-system financial structure on a public page.

For expatriate residents and for cases routed through international referral, Reserve Meds operates the standard cash-pay-with-documentation pattern. We separate every line in the quote: drug, qualified-centre admission, immunomodulation drugs, monitoring labs, our coordination fee. We do not put a markup on the manufacturer's drug price. Our coordination fee is disclosed in writing before any funds move.

Private insurance coverage in Qatar (Qatar Insurance Company, AXA Gulf, Daman, MetLife) for one-time gene therapies is handled on a case-by-case prior-authorisation basis. We supply the documentation packet at no charge.

The six months after the infusion

The peri-infusion immunomodulation protocol is intensive. Your son will be on oral corticosteroids in addition to his existing DMD steroid regimen for roughly the first eight weeks. Weekly liver function panels (AST, ALT, GGT, bilirubin) for the first three months and biweekly through month six are the published monitoring standard. Cardiac surveillance for myocarditis includes troponin checks and echocardiography per the centre's protocol. Hospitalisation for steroid-responsive hepatitis is uncommon but not rare; admission for acute liver injury, which is what the 2025 boxed warning addresses, is the safety event that the monitoring schedule is designed to catch early. Sidra's published cohort has not reported severe adverse events to date.

A practical implication for the family: your son's school attendance, sports participation, and social activity will be partially restricted for several weeks while the immune response is being managed. Sidra's rehabilitation service is part of the post-infusion follow-up.

What Reserve Meds does for a Qatari family, honestly

Because Sidra Medicine is the natural in-country infusion centre and runs one of the most established Elevidys programmes globally, our role for a Qatari family is genuinely different from our role for a UAE, Saudi, or expatriate-MENA family.

For Qatari nationals being treated at Sidra: we are most useful as a documentation and second-opinion concierge layer. We can help with international second-opinion clinical reviews from US qualified-centre paediatric neurologists, assist with prior-authorisation documentation for private insurance, coordinate translation of medical records for international referrals or family review, and provide independent regulatory documentation review. We are honest that Sidra's in-house programme covers the operational coordination; we are not the right firm to insert ourselves into that workflow if your son's care is already routed there.

For expatriate residents in Qatar choosing international treatment: we coordinate cross-border specialty medicine through DSCSA-compliant US distribution, qualified-centre liaison in the US or Europe, cold-chain logistics, and named case-lead management. This is the standard Reserve Meds scope.

For Qatari families considering treatment at Sidra Medicine versus at a US or European centre: we can provide an honest comparison brief — Sidra's experience and outcomes, qualified-centre US options, travel and accommodation cost profile, post-infusion follow-up logistics if treated abroad — so the family can make an informed choice.

Reserve Meds is not your son's prescriber. We do not practise medicine. We do not manufacture Elevidys. We do not own or operate any infusion centre. We are not your insurer. Clinical decisions stay with your paediatric neurologist and the treating centre. We are an operational and documentation layer, not the clinical decision-maker.

A note for families weighing this

For Muslim families thinking through the religious-ethical dimension, the Islamic bioethics consensus on disease-modifying therapies that preserve life and function is broadly permissive, and families typically consult with their religious advisors before committing. We will not pressure that conversation. Families typically take between two and six weeks from first call to readiness, with that time used for extended-family consultation, financial preparation, and religious counsel. Given Sidra's in-country presence and the deep regional experience, the clinical-window stress is often less acute for Qatari families than for families coordinating international referrals.

What to do if you want to start

If you are a Qatari national or resident interested in proceeding at Sidra Medicine, the right first step is direct contact with Sidra's paediatric neurology or Gene Therapy Center referral team. We can support documentation review or international second-opinion logistics around that pathway.

If you are an expatriate resident in Qatar or a family considering international treatment, reach out and we will walk through the cross-border options for your son.

If your son is non-ambulatory, or under 4, or in a situation where Elevidys is not the answer, reach out anyway: we will walk through what other options exist for his specific picture.

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 son'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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