Amondys 45

Abu Dhabi · access guide

Amondys 45 (casimersen) 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 family of a son with Duchenne muscular dystrophy walks into this decision in a clinically well-positioned emirate. Abu Dhabi is the UAE's documented hub for advanced paediatric neuromuscular care, with Sheikh Khalifa Medical City, Sheikh Shakhbout Medical City, and Tawam Hospital paediatric neurology providing depth that few other GCC locations match. This page is meant to be the first honest read you get on Amondys 45 in Abu Dhabi.

The genetic gate: this drug is for a specific subset of DMD families

Amondys 45 is an exon-skipping therapy. It only helps families whose son's DMD mutation is **amenable to exon 45 skipping**. Approximately 8 percent of DMD patients fall into this group. That is the first conversation.

In Abu Dhabi, paediatric DMD genetic workup typically routes through Tawam Hospital paediatric genetics in Al Ain (long-standing rare-disease and neuromuscular service), SKMC paediatric neurology in Abu Dhabi city, or SSMC paediatric services. Whole-gene sequencing or MLPA is sent to regional reference labs and results return in 4 to 8 weeks. Your paediatric neurologist will read out the deletion boundary on the genetic report and tell you whether exon 45 skipping is the right mechanism.

If exon 45 skipping is not the right mechanism, this page is the wrong page. Exondys 51 (eteplirsen) is for exon 51 skipping. Vyondys 53 (golodirsen) and Viltepso (viltolarsen) are for exon 53 skipping. Elevidys is a one-time gene-therapy option for ambulatory boys aged 4 and older. SKMC administered the UAE's first DMD gene transfer therapy on 19 March 2024 under DoH coordination. Reach out and we will talk through your son's specific picture.

What Amondys 45 actually is, in plain terms

Amondys 45 is a weekly intravenous infusion. The active ingredient is casimersen, a phosphorodiamidate morpholino oligomer (PMO) antisense oligonucleotide. PMO is a synthetic chemistry. The molecule binds to a specific stretch of the pre-mRNA of the DMD gene and tells the splicing machinery to skip exon 45, restoring the reading frame and producing a partially functional truncated dystrophin protein.

Amondys 45 is not a cure. The clinical data describe a slowing of functional decline. FDA accelerated approval was granted in February 2021. The European Medicines Agency reviewed Amondys 45 in 2024 and issued a negative CHMP opinion, citing insufficient evidence of clinical benefit. Your neurologist can put the FDA-EMA divergence in clinical context.

The schedule is lifelong. No taper, no stop point.

The rest of the workup

Three more results need to land.

Baseline renal function. Casimersen has a post-marketing warning for renal toxicity. Serum creatinine and urinalysis; quarterly monitoring continues for the life of therapy.

Baseline cardiac function. Echocardiography is the standard baseline.

Baseline functional assessments. The 6-minute walk test, the North Star Ambulatory Assessment, and FVC. Many paediatric patients have a port placed once the decision is committed.

A clinical rationale letter from your paediatric neurologist documents the genetic confirmation, the renal and cardiac baselines, the functional baselines, the rehabilitation plan, the steroid regimen, and the requested treatment.

The Abu Dhabi pathway: how it actually works in 2026

The Emirates Drug Establishment is the federal authority you and your treating hospital file through. `[VERIFY: EDE Amondys 45 registration status in 2026]`. In the absence of EDE registration, the named-patient mechanism is the route. The Department of Health Abu Dhabi adds the emirate-level layer.

In our experience coordinating named-patient paediatric neuromuscular cases in the UAE, EDE approval on a complete, well-documented file takes three to six weeks from filing. Renewal cycles for continuous weekly supply require advance planning. Reserve Meds maintains the renewal calendar.

In Abu Dhabi, the paediatric neurology hubs that can administer Amondys 45 include Tawam Hospital paediatric neurology in Al Ain (Sarepta-comparable family of products and DMD experience), Sheikh Khalifa Medical City paediatric neurology in Abu Dhabi (administered the UAE's first DMD gene transfer therapy in March 2024; Dr Omar Ismayl leads paediatric neurology), Sheikh Shakhbout Medical City paediatric services, Cleveland Clinic Abu Dhabi paediatric services, and Burjeel Medical City. For Emirati nationals, Thiqa and Daman pre-authorisation pathways may apply.

The cost conversation, in the form an Abu Dhabi family needs

The Amondys 45 annual drug price in 2026 sits in an indicative range of roughly USD 700,000 to 1,200,000 per year, depending on body weight, or approximately AED 2.6 to 4.4 million per year. For a typical paediatric patient, cumulative drug cost over a lifetime can reach USD 30 to 50 million plus.

The full cost of care includes pre-treatment workup, port placement if chosen, weekly infusion-centre fees, quarterly monitoring, and our coordination fee. We separate every line. We do not put a markup on the manufacturer's drug price. The coordination fee is disclosed in writing.

Insurance coverage in Abu Dhabi is uneven. Daman and Thiqa have reimbursed exon-skipping therapies for Emirati nationals and certain employer plans through prior authorisation; private insurers vary widely. We supply your insurer with the documentation packet at no charge.

A direct point: families weighing Amondys 45 against Elevidys for an exon-45-skip-amenable son are weighing a roughly AED 11 to 13 million one-time cost (Elevidys) against an indefinite AED 2.6 to 4.4 million annual cost (Amondys 45). That arithmetic has implications beyond the first year.

Life on weekly infusion

The peri-infusion protocol is light compared with gene therapy. Hypersensitivity reactions can occur and the first several infusions are observed at the qualified centre. Once stable, patients move to weekly infusions of 35 to 60 minutes each.

The practical implication is that the infusion fits into the weekly rhythm of the household. Many Abu Dhabi families schedule the infusion for a Saturday morning before the school week. Home infusion programmes are an option for stable patients. A port reduces the friction of weekly peripheral cannulation.

Quarterly visits cover renal function, motor function, pulmonary function, and at scheduled intervals echocardiography.

Religious and ethical considerations

Casimersen is a synthetic antisense oligonucleotide with no animal-source material. Halal status is not in question. 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 to a lifelong therapy.

When Amondys 45 is not the right option

If your son's mutation is not amenable to exon 45 skipping, Amondys 45 is the wrong drug. Depending on the deletion boundary, the right drug may be Exondys 51, Vyondys 53, Viltepso, or for ambulatory boys aged 4 and older, Elevidys gene therapy with SKMC Abu Dhabi as the UAE qualified centre. If your son has clinically significant renal impairment, the therapy is on hold. If your son has been diagnosed with a non-DMD muscular dystrophy, Amondys 45 does not apply.

In all of these situations, reach out anyway.

What Reserve Meds does for an Abu Dhabi family

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. Our scope is the regulatory documentation packet, the EDE named-patient filing in collaboration with the dispensing hospital pharmacy, the sourcing logistics from the manufacturer's authorised US distribution through DSCSA chain of custody, cold-chain shipment of the weekly supply to the Abu Dhabi qualified centre or home-infusion programme, the renewal-cycle calendar, and named case-lead coordination.

Reserve Meds is not your son's prescriber. We do not practise medicine. We do not manufacture Amondys 45. Clinical decisions stay with your paediatric neurologist and the qualified centre.

We work cash-pay. Our coordination fee is disclosed in writing.

What to do if you want to start

The first concrete step is a call with our case-lead so we can confirm whether Amondys 45 is the right consideration for your son. If genetic confirmation is already in hand, we move directly into documentation work. If not, we route through to Tawam, SKMC, or SSMC paediatric genetics first.

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 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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