Amondys 45

Saudi Arabia · access guide

Amondys 45 (casimersen) 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 son with Duchenne muscular dystrophy walks into this decision with more than a treatment question. There is a clinical question, a genetic question, a regulatory question, a financial one, and a family one, and they all need answers in roughly the same week. This page is meant to be the first honest read you get on Amondys 45 in the Kingdom of Saudi Arabia, written by the team that would coordinate it for your son if you decided to go forward. We assume your paediatric neurologist has either raised it with you or you have raised it with them.

We will be specific about the genetic-testing gate that decides whether Amondys 45 is even relevant for your son, what the rest of the workup decides, how the SFDA named-patient pathway works for this drug, what it costs in SAR and US dollars per year for a lifelong weekly infusion, where the infusion can be given in the kingdom, and what life looks like once weekly therapy is in place.

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

Amondys 45 is an exon-skipping therapy. It works by forcing the cell's splicing machinery to skip exon 45 during dystrophin mRNA processing, restoring the reading frame across the patient's deletion and producing an internally truncated but partially functional dystrophin protein. This mechanism only helps families whose son's DMD mutation is **amenable to exon 45 skipping**. Approximately 8 percent of DMD patients fall into this group. Most do not. That is the first conversation, and it has to happen before anything else.

KFSHRC's molecular genetics service in Riyadh has run the Saudi reference cohort for DMD mutations and is the natural setting for whole-gene sequencing or MLPA testing if your son has not yet been tested. KAMC Riyadh's rare-disease genetics service runs the same workup. KFSHRC Jeddah covers the western region with the same clinical standards. Results from whole-gene sequencing typically return in 4 to 8 weeks; MLPA is faster but covers only common deletion-duplication patterns. Your paediatric neurologist will read out the deletion boundary on the genetic report and tell you whether exon 45 skipping is the right mechanism for your son.

If exon 45 skipping is not the right mechanism, this page is the wrong page. We will be direct about what the right page is. 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, independent of which exon is the boundary. Reach out and we will talk through your son's specific picture. We coordinate cases for any of these drugs, not just Amondys 45.

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, not derived from human or animal sources. The molecule binds to a specific stretch of the pre-mRNA of the DMD gene and tells the splicing machinery to leave exon 45 out of the final mRNA. With exon 45 removed, the reading frame across the deletion is restored, and the muscle cell can produce a shortened but partially functional dystrophin protein. The level of dystrophin produced is modest in measured biopsy data; what the clinical data describe is a slowing of functional decline against the natural history of DMD, not restoration of normal function.

What Amondys 45 is not is a cure. The benefit is real but partial, and it requires continuous weekly dosing. There is no taper, no stop point, no course completed. The weekly schedule is lifelong.

The pivotal ESSENCE trial documented a statistically significant increase in dystrophin protein expression by western blot. Long-term functional follow-up against natural-history cohorts is the basis for the FDA accelerated approval granted in February 2021. Your neurologist will walk you through the most recent functional data including the 6-minute walk test, North Star Ambulatory Assessment, and forced vital capacity trajectories in the ESSENCE long-term follow-up cohort.

We will also be honest that the European Medicines Agency reviewed Amondys 45 in 2024 and issued a negative CHMP opinion. The EMA concluded that the available evidence was not sufficient to support marketing authorisation in the European Union as of that date. We mention this because Saudi families occasionally consult European clinicians and the divergence between FDA and EMA decisions deserves a straight answer. Your neurologist can put the FDA-EMA divergence in clinical context against your son's specific situation.

The rest of the workup

Beyond the genetic confirmation that exon 45 skipping is the right mechanism, three more results need to land.

First, baseline renal function. Casimersen has a post-marketing warning for renal toxicity. Serum creatinine, urinalysis, and where available cystatin C are the baseline labs. Active renal impairment requires clinical workup before infusion can be scheduled, and quarterly renal monitoring continues for the life of therapy.

Second, baseline cardiac function. DMD cardiomyopathy presents later in the disease course; echocardiography is the standard baseline, with intervals set per centre protocol.

Third, baseline functional assessments and vascular access planning. The 6-minute walk test (for ambulatory patients), the North Star Ambulatory Assessment, and forced vital capacity provide the functional baseline. Because the infusion is weekly and lifelong, many paediatric patients have a port placed once the decision is committed; weekly peripheral IV access is operationally hard over years.

A clinical rationale letter from your consultant documents the genetic confirmation, the renal and cardiac baselines, the functional baselines, the rehabilitation plan, the steroid regimen (deflazacort or prednisone, which continues alongside Amondys 45), and the requested treatment.

The Saudi regulatory pathway: how it actually works in 2026

The Saudi Food and Drug Authority published its Gene Therapy Products Registration Guidelines in 2023 and has built operational depth in advanced-therapy regulatory review since. `[VERIFY: SFDA registration status of Amondys 45 in 2026]`. As of 2026 the named-patient mechanism is the working assumption for most Saudi paediatric neurology services seeking Amondys 45 access. The application is filed through SFDA's drug.sfda.gov.sa portal by the dispensing hospital's licensed pharmacist on the consultant's behalf, with the clinical rationale letter, genetic report, renal and cardiac baselines, qualified-centre plan, and requested supply quantity attached.

NUPCO sits in the procurement loop for public-sector hospitals. For named-patient cases at NUPCO-contracted public hospitals, NUPCO coordinates the procurement once SFDA approval is in hand. For private-sector cases, the dispensing hospital's pharmacy handles procurement directly through the manufacturer's authorised export channel.

Typical SFDA approval timing on a complete, well-documented file is four to eight weeks. Renewal cycles thereafter (because Amondys 45 supply is continuous and weekly) are typically simpler than the initial approval but require advance planning so that weekly supply continuity is never at risk. Reserve Meds maintains the renewal calendar as part of case management.

In the kingdom, the paediatric neurology hubs that can administer Amondys 45 are clustered in the tertiary research hospitals. King Faisal Specialist Hospital and Research Centre, Riyadh, has the deepest demonstrated DMD clinical and research base in the country, with a paediatric neuromuscular service that has authored Saudi DMD management consensus statements. King Abdulaziz Medical City under National Guard Health Affairs has comparable infrastructure and runs an active rare-disease genetics service. King Fahad Medical City paediatric neurology covers complex paediatric neuromuscular cases. KFSHRC Jeddah serves the western region with the same standards. Dr Sulaiman Al-Habib Medical Group's flagship Riyadh facilities can coordinate Amondys 45 cases in conjunction with a Saudi consultant who holds privileges there.

The cost conversation, in the form a Saudi family needs

This is the conversation that distinguishes Amondys 45 from a one-time gene therapy. Amondys 45 is lifelong, weekly, and dosed by weight. As your son grows, the dose grows, and the annual cost grows.

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 SAR 2.6 to 4.5 million per year. For a typical paediatric patient starting therapy at age 6 to 8, cumulative drug cost over a lifetime can reach USD 30 to 50 million plus at current pricing. That is the manufacturer's price for the drug only. The full cost of care includes the pre-treatment workup, vascular access placement if a port is chosen, the weekly infusion-centre fees, quarterly renal and motor monitoring, periodic cardiac and pulmonary assessment, and our coordination fee.

When we issue a quote at intake, we separate every line: drug per quarter, qualified-centre fees, vascular access procedure if applicable, monitoring labs, our coordination fee. Nothing is bundled. We do not put a markup on the manufacturer's drug price. The coordination fee is disclosed in writing before any funds move.

Insurance coverage of Amondys 45 in Saudi Arabia is uneven. Because Amondys 45 is unlikely to be on the CCHI list, reimbursement under standard CCHI-regulated plans is not currently expected. Private insurers (Bupa Arabia, Tawuniya, MedGulf, Walaa) handle DMD exon-skipping therapies on case-by-case prior-authorisation basis, with approval uncommon outside flagship-hospital Vision 2030 pilot frameworks. We supply the prior-authorisation documentation packet to your insurer at no charge. We do not process claims directly. Most Saudi DMD exon-skipping cases to date have proceeded as cash-pay arrangements with partial reimbursement where available.

For Saudi-national families being treated at KFSHRC or KAMC, your consultant will know whether any current MoH or Vision 2030 pilot framework could underwrite the case in full or in part. Worth asking explicitly.

A direct point: families weighing Amondys 45 against Elevidys for an exon-45-skip-amenable son are weighing a roughly SAR 11.3 to 13.1 million one-time cost (Elevidys) against an indefinite SAR 2.6 to 4.5 million annual cost (Amondys 45). That arithmetic has implications beyond the first year. Your paediatric neurologist will frame the clinical comparison; we will frame the financial-planning comparison honestly.

Life on weekly infusion

The peri-infusion protocol is light compared with gene therapy. Amondys 45 is not a one-time event with months of post-infusion monitoring; it is a routine. Hypersensitivity reactions can occur and the first several infusions are typically observed at the qualified centre, but once stable, many patients move to a routine of weekly infusions of 35 to 60 minutes each.

The practical implication for the family is that the infusion fits into the weekly rhythm of the household. Many Saudi families schedule the infusion for a Saturday morning before the school week, so school attendance is preserved. Vascular access through a port reduces the friction of weekly peripheral cannulation, which is a real consideration for a child who will be on this therapy for decades.

Quarterly visits to the paediatric neurology team cover renal function, motor function, pulmonary function, and at scheduled intervals echocardiography. These are not Amondys 45 specific; they are the standard surveillance schedule for DMD management with Amondys 45 layered in.

Religious and ethical considerations

Casimersen is a synthetic antisense oligonucleotide. It contains no animal-source material. Halal status is not in question. 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 across the schools, and families typically consult with their religious advisors before committing to a lifelong therapy. The "lifelong commitment" aspect of Amondys 45 sometimes invites deeper religious consultation than a one-time therapy would; families who proceed have usually taken between two and six weeks from first call to engagement.

When Amondys 45 is not the right option

We will name the situations where Amondys 45 is not the right page directly.

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, or Viltepso. If your son is ambulatory and 4 or older with a confirmed DMD mutation of any exon position, Elevidys gene therapy may be an option (with the FDA boxed-warning considerations we discuss on the Elevidys page).

If your son has clinically significant renal impairment, the renal toxicity warning on Amondys 45 puts the therapy on hold until the renal picture is workable.

If your son has been diagnosed with a non-DMD muscular dystrophy, Amondys 45 does not apply. The exon-skipping mechanism is specific to DMD-gene splicing.

In all of these situations, reach out anyway.

What Reserve Meds does for a Saudi family

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. For a Saudi family pursuing Amondys 45, our scope is the regulatory documentation packet, the SFDA named-patient filing in collaboration with your consultant and the dispensing hospital's pharmacist, the sourcing logistics from the manufacturer's authorised US distribution through DSCSA-compliant chain of custody, cold-chain shipment of the weekly supply to the qualified Saudi centre, the renewal-cycle calendar so weekly supply continuity is never at risk, and named case-lead coordination from intake forward.

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

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

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 of an exon-45-skip-amenable mutation is already in hand, we move directly into documentation work. If not, we route through to the right paediatric genetics service at KFSHRC or KAMC first.

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 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.

reserved for you.

Composite case examples. This document is for general information only and does not constitute medical advice. Please consult your treating physician.
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