Exondys 51

United Arab Emirates · access guide

How to access Exondys 51 from the UAE, the named-patient import pathway, 2026

By Reserve Meds · Clinical & regulatory team · Last reviewed 2026-04-23

A the UAE family of a boy with Duchenne muscular dystrophy (DMD) whose genotyping confirms an exon-51-skip-amenable mutation may receive a prescription for Exondys 51 (eteplirsen) from their treating paediatric neurologist. Exondys 51 is FDA-approved, manufactured by Sarepta Therapeutics, and is an antisense oligonucleotide that restores partial dystrophin production in patients whose DMD mutation is amenable to exon 51 skipping, roughly 13% of the DMD population. In the UAE, Exondys 51 is not locally registered for routine dispensing, which is why your paediatric neurologist is likely guiding you toward the UAE Ministry of Health and Prevention (MoHAP) named-patient import pathway.

This guide explains the legal pathway, what documentation your physician needs, typical timing and cost bands, and where Reserve Meds fits in.

The clinical situation

Exondys 51 is a phosphorodiamidate morpholino oligomer (PMO) administered by weekly intravenous infusion, indefinitely. Eligibility is strictly genotype-gated: your child's DMD gene mutation must be confirmed by accredited genetic testing as amenable to exon 51 skipping. Treatment requires a paediatric neurologist with DMD experience, a day-infusion facility, and ongoing monitoring including renal function, cardiac assessment, and motor-function scales (NSAA, 6MWT). Dosing is weight-based and is given in perpetuity; this is a long-term commitment rather than a finite course.

Is Exondys 51 legally importable into the UAE?

Yes, through the MoHAP named-patient import framework, administered in coordination with the Ministry of Health for patients treated in public tertiary centres.

The named-patient mechanism allows a the UAE-licensed physician to request import of a medicine not locally registered when: (a) the medicine is approved by a recognised reference authority such as the US FDA, (b) no clinically equivalent locally registered alternative is available, (c) the physician takes clinical responsibility, and (d) the importing party documents the chain of custody end to end. For exon-51-amenable DMD in the UAE, there is no locally registered exon-skipping alternative, making the clinical rationale straightforward once genotype is confirmed.

How the pathway works, step by step

1. **Consultation with your paediatric neurologist.** Confirmation of DMD diagnosis and exon-51-skip-amenable genotype, with baseline NSAA, 6MWT, cardiac, and renal assessments documented.
2. **Treatment-centre identification.** A tertiary hospital with paediatric neurology and infusion-day-unit capacity is confirmed as the administering site.
3. **MoHAP named-patient application.** Your physician or the hospital pharmacy files the application with clinical rationale, genotyping report, patient reference, and chain-of-custody plan.
4. **US-side sourcing.** Reserve Meds coordinates with our US-licensed specialty wholesale partner to secure Exondys 51 from Sarepta's authorised distribution channel.
5. **Shipment.** The product ships under chain-of-custody and manufacturer handling conditions to the administering hospital pharmacy.
6. **Arrival and first infusion.** The treating hospital administers the weekly infusion and establishes the ongoing schedule; Reserve Meds coordinates rolling refills.

What documentation your physician needs

Your physician will typically need to provide:

- A clinical rationale letter confirming DMD diagnosis, exon-51-skip-amenable mutation, baseline motor and cardiac status, and Exondys 51 as the indicated treatment
- Verification of their the UAE medical licence (SCFHS / MOH)
- A copy of the accredited DMD genetic-testing report
- Patient identifier (anonymised reference where possible)
- An administration and monitoring plan including weekly infusion scheduling and long-term follow-up

Reserve Meds provides a physician documentation kit with the templates MoHAP reviewers expect to see for rare-paediatric-neurology named-patient imports, including the exon-skipping genotype gate that is central to Exondys 51 eligibility.

Costs and timing

Exondys 51 is weight-dependent in dosing, so annual cost scales with the child's body weight. Indicative 2026 US cash-pay annual cost for Exondys 51 sits in a broad range of roughly USD 300,000 to over USD 1,000,000 depending on weight, with adolescents and older children at the upper end. International logistics, MoHAP documentation handling, and concierge coordination add incremental cost. Reserve Meds issues a full transparent quote at the start of intake.

Indicative timing for the first infusion after cohort intake opens is 7-14 days from the moment a complete MoHAP application is submitted. Subsequent weekly supply runs on a rolling refill schedule once the pathway is established.

Fulfilment availability is limited to our first cohort, and all timelines published on this site are indicative. If your clinical situation is time-sensitive, tell us at intake. We triage accordingly.

A culturally-aware note: DMD clusters in families with shared ancestry, and in the UAE consanguineous marriage patterns mean multiple affected boys in one extended family are not uncommon. Our concierge coordination is designed around the extended-family caregiving pattern, an uncle, aunt, or grandparent can be a designated case contact, and we coordinate refill logistics around school schedules and family travel between the Kingdom, the Gulf, and Europe.

Reserve Meds's role

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. For Exondys 51 specifically, we provide:

- **Sourcing.** Through our US-licensed specialty wholesale partner, operating under DSCSA chain-of-custody.
- **Documentation.** Regulatory package for your physician and for MoHAP review, tailored to exon-skipping eligibility.
- **Logistics.** Chain-of-custody shipment coordination to the administering hospital pharmacy on a weekly-refill cadence.
- **Concierge case lead.** A named point of contact for the family, managing long-term refill logistics and weight-based dose adjustments as the child grows.

What we do not do: we are not the prescriber, we do not practise medicine, and we are not the dispensing pharmacy. All clinical decisions remain with your treating paediatric neurologist.

Frequently asked

Is this legal in the UAE? Yes, when executed through the MoHAP named-patient framework with appropriate documentation. Cross-border named-patient import for rare-paediatric-disease therapies is a recognised mechanism. See our trust and compliance page.

Is Exondys 51 a cure? No. Exondys 51 is a disease-modifying therapy that restores partial dystrophin expression in exon-51-amenable DMD patients. Your paediatric neurologist will discuss realistic outcome expectations and long-term monitoring.

What if my son's mutation is not exon-51-amenable? Exondys 51 is genotype-specific. Other exon-skipping therapies target exons 45, 53, and others; if your son's mutation is amenable to a different exon skip, that product would be the relevant candidate. Your neurologist's genotype report guides the choice.

Does the infusion have to happen in a hospital? Weekly IV infusion requires an infusion-day unit with paediatric expertise. Home infusion is not currently standard for Exondys 51 in the UAE.

Will insurance or MoH coverage apply? Cash-pay is the default. Some the UAE patients may receive partial MoH coverage or private-insurance consideration on a case-by-case basis; we supply documentation for submission but do not process public-payer or insurance claims directly.

Reserve Meds's role

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Reserve Meds

reserved for you.

Composite case examples. This document is for general information only and does not constitute medical advice. Please consult your treating physician.
Reserve Meds is in pre-launch. Published timelines and cost ranges are indicative, not guarantees.
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