

## Vyondys 53

Qatar · access guide

# How to access Vyondys 53 from Qatar, the named-patient import pathway, 2026

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

An Qatarn family of a boy with Duchenne muscular dystrophy (DMD) whose genotyping confirms an exon-53-skip-amenable mutation may receive a prescription for Vyondys 53 (golodirsen) from their treating paediatric neurologist. Vyondys 53 is FDA-approved, manufactured by Sarepta Therapeutics, and is an antisense oligonucleotide designed to restore partial dystrophin production in DMD patients amenable to exon 53 skipping, roughly 8% of the DMD population. In Qatar, Vyondys 53 is not locally registered, which is why your paediatric neurologist is likely guiding you toward the Qatar Ministry of Public Health (MOPH) named-patient / personal-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

Vyondys 53 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 53 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 continues long-term; this is a multi-year commitment.

## Is Vyondys 53 legally importable into Qatar?

Yes, through the MOPH named-patient import framework and the personal-use import allowance under the Drugs and Cosmetics Rules. The pathway permits 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) a treating physician takes clinical responsibility, and (d) the importing party documents chain of custody end to end.

For exon-53-amenable DMD in Qatar, there is no locally registered exon-skipping alternative. Named-patient personal import for rare-disease therapies has been a recognised pathway used by Qatarn tertiary centres for many years.

## How the pathway works, step by step

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1. **Consultation with your paediatric neurologist.** Confirmation of DMD diagnosis and exon-53-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. **MOPH named-patient / personal-import 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 Vyondys 53 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

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Your physician will typically need to provide:

- A clinical rationale letter confirming DMD diagnosis, exon-53-skip-amenable mutation, baseline motor and cardiac status, and Vyondys 53 as the indicated treatment
- Verification of their Qatarn medical registration (NMC / state council)
- 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 MOPH reviewers and hospital pharmacies expect to see for rare-paediatric-neurology personal-import applications, including the exon-skipping genotype gate that is central to Vyondys 53 eligibility.

## Costs and timing

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Vyondys 53 is weight-dependent in dosing, so annual cost scales with the child's body weight. Indicative 2026 US cash-pay annual cost 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, MOPH 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 MOPH 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: Qatar's DMD community is large, well-organised through disease-specific family networks, and often navigates treatment across multiple cities, genetic diagnosis in one metro, infusion access in another, rehabilitation support at home. Our concierge coordination is designed around that multi-city caregiving reality: a single case lead manages logistics across family members and treating centres, with documentation copies sent to all relevant parties the family designates.

## Reserve Meds's role

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Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. For Vyondys 53 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 MOPH 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

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**Is this legal in Qatar?** Yes, when executed through the MOPH named-patient / personal-import framework with appropriate documentation. Personal import of rare-disease therapies not registered in Qatar has long been a recognised mechanism. See our trust and compliance page.

**Is Vyondys 53 a cure?** No. Vyondys 53 is a disease-modifying therapy that restores partial dystrophin expression in exon-53-amenable DMD patients. Your paediatric neurologist will discuss realistic outcome expectations and long-term monitoring.

**What if my son's mutation is exon-45 or exon-51 amenable?** Vyondys 53 is exon-53-specific. Other exon-skipping therapies target exons 45 and 51; if your son's mutation is amenable to a different exon skip, that product would be the candidate. Your neurologist's genotype report guides the choice.

**Can the infusion happen at home?** Weekly IV infusion requires a day-infusion unit with paediatric expertise in the current Qatari standard of care. Your hospital will confirm the administration setting.

**Will insurance cover this?** Cash-pay is the default. Some private health insurers in Qatar consider rare-disease imports on a case-by-case basis; we supply documentation for your submission but do not process insurance claims directly.

## ***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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### **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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