Exondys 51 access in the Kingdom of Saudi Arabia

How families in the Kingdom pursue Exondys 51 (eteplirsen), Sarepta's phosphorodiamidate morpholino oligomer (PMO) exon-skipping therapy, through the SFDA Personal Importation Program.

Last reviewed 2026-05-12 by Reserve Meds clinical & regulatory team. This page combines the Saudi Arabia country research module with the Exondys 51 drug module to describe the path families actually walk.

Quick orientation

Exondys 51 (eteplirsen) is Sarepta's exon 51-skipping antisense oligonucleotide for Duchenne muscular dystrophy. It received FDA accelerated approval in 2016 for the approximately 13 percent of DMD patients whose dystrophin gene mutation is amenable to exon 51 skipping. For Saudi Arabia families with a child who carries a confirmed exon 51-amenable DMD mutation, this page describes the SFDA Personal Importation Program pathway.

Why this drug is hard to source in Saudi Arabia

Duchenne muscular dystrophy is a rare pediatric neuromuscular disease, and the exon 51-amenable subpopulation is roughly 13 percent of the total. The KSA patient population is correspondingly small. Exondys 51 has not had broad SFDA registration uptake. Even at KFSH&RC and the major academic centers, the local pharmacy is unlikely to stock eteplirsen routinely. Pediatric patients identified through the expanding KSA genomics and DMD diagnostic capacity often need patient-specific import. The PIP route is the lawful path for the treating pediatric neurologist to bring Exondys 51 in for a named patient.

The SFDA Patient Import Permit (PIP) pathway applied to Exondys 51

The Saudi Food and Drug Authority's Personal Importation Program is the federal pathway that allows an SCFHS-licensed physician to import a specific medicine for a specific named patient when the medicine is approved by a recognized reference authority (typically the US FDA, EMA, MHRA, PMDA Japan, or Health Canada) and a clinically equivalent locally

registered alternative is not suitable. Exondys 51 (eteplirsen) holds FDA approval since 2016 for Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation amenable to exon 51 skipping, which places it squarely within the framework's scope.

The application is filed through the dispensing institution's import pharmacy (or, where the institution does not hold internal import-pharmacy capability, through an SFDA-licensed specialty importer in Riyadh or Jeddah). The standard package contains the clinical justification letter from the treating physician, the SCFHS license verification, the anonymized patient identifier, the full product details for Exondys 51 including intravenous infusion, weekly weight-based dosing (30 mg/kg per week), with cold-chain handling at 2 to 8 degrees Celsius documented in the file, the destination dispensing facility license, and the chain-of-custody plan from the US point of release through international transit to the receiving Saudi pharmacy. The SFDA portal at sfda.gov.sa handles regulatory transactions, and named-patient activity increasingly routes through the agency's Ghad digital platform.

Where Exondys 51 gets dispensed in the Kingdom

The major Saudi institutions that handle named-patient imports as established workflow include King Faisal Specialist Hospital and Research Centre (KFSH&RC) with operations in Riyadh, Jeddah, and Madinah; King Abdulaziz Medical City (KAMC) and the Ministry of National Guard Health Affairs network; King Saud University Medical City and the academic medical centers; King Khalid University Hospital; Dr. Sulaiman Al Habib Medical Group (HMG) across multiple Riyadh, Jeddah, and Eastern Province facilities; the Saudi German Hospital network; Dr. Soliman Fakeeh Hospital in Jeddah; and Dallah Hospital in Riyadh. Smaller hospitals typically route their named-patient cases through one of these centers or through an SFDA-licensed specialty importer. For a Exondys 51 case, the dispensing facility is selected on the basis of where the treating physician practices and where the patient receives ongoing care; Reserve Meds does not select the dispensing facility on the family's behalf.

Real costs in SAR and USD

The US wholesale acquisition cost for Exondys 51 is weight-based at 30 mg/kg weekly. For a typical pediatric patient weighing approximately 25 kg, annual drug cost is approximately USD 750,000 to USD 1,000,000, translating to roughly SAR 2,800,000 to SAR 3,750,000 per year. Cold-chain logistics for a refrigerated biologic add approximately SAR 3,000 to SAR 5,600 per shipment, and a weekly dosing schedule means weekly or multi-week consolidated shipments. The cost structure means most KSA families pursuing eteplirsen are working through a combination of cash-pay, named-patient charity engagement with Sarepta where available, and case-by-case insurer engagement.

Reserve Meds quotes an indicative range based on the initial intake and then a transparent firm quote with each line item shown separately. The Reserve Meds concierge fee is published on a tiered schedule and is shown as a separate line. Nothing is bundled. Nothing is hidden.

Timing, what to expect

The SFDA Personal Importation Program processes routine cases (recognized reference-authority drug, well-documented indication, established institution) in approximately 10 to 20 business days. Complex cases (novel mechanism, off-label use within the FDA label scope, ultra-rare patient population, first-time importer) can extend to 6 to 8 weeks. SFDA does not publish guaranteed turnaround times, so case-by-case planning is the norm. In parallel with the SFDA review, Reserve Meds aligns the US-side sourcing, the packaging and cold-chain validation, and the shipment plan, so the drug is ready to move on the day approval comes through. The patient experience runs through ten well-defined steps from initial physician decision through reorder coordination; the full ten-step sequence is documented in the Saudi Arabia country module and in our patient-facing operations brief.

What your physician needs to provide

The treating pediatric neurologist's clinical justification letter typically documents the confirmed DMD diagnosis (genetic test report attached showing the exon 51-amenable mutation, since the indication is genotype-specific), the patient's age and baseline functional status (North Star Ambulatory Assessment, six-minute walk test where applicable), prior therapy including corticosteroids, the proposed weekly weight-based dose, and the planned multi-year monitoring schedule. The SCFHS registration in pediatric neurology accompanies the letter.

The dispensing facility's SFDA-licensed pharmacy completes the submission and accepts the chain-of-custody documentation. The institutional license is what authorizes the dispensing pharmacy to receive the imported drug, so the physician's individual SCFHS license is necessary but not sufficient on its own. Post-import pharmacovigilance commitment to report adverse events through the SFDA National Pharmacovigilance Center is part of the application and runs through the full course of therapy, not just the initial dose.

Vision 2030 and the specialty-access environment

Saudi Vision 2030's Health Sector Transformation Program (HSTP) is the operating frame for healthcare reform in the Kingdom. HSTP is restructuring the Ministry of Health from a provider-and-regulator into a regulator and strategist, with clinical delivery devolving into

regional Health Clusters and Centers of Excellence. The program names tertiary cancer care, rare-disease care, organ transplantation, genomics, and digital health as priority verticals, all of which are heavy users of specialty drugs not registered locally. The practical effect on the PIP framework is twofold. HSTP is expanding the universe of specialty drugs that get formal SFDA registration, which closes some access gaps. At the same time, HSTP is increasing diagnostic capacity in rare disease and oncology genomics, which surfaces new patients who need drugs that are FDA-approved but not yet registered in the Kingdom. The named-patient framework remains essential for the foreseeable future. Saudization (the Nitaqat workforce-nationalization program) does not change the PIP framework, but confirming the prescriber's SCFHS license status before filing is good practice in any case where the treating physician is in a renewal window.

Pharmacovigilance and cold-chain considerations

Exondys 51 has a comparatively benign safety profile relative to other DMD therapies, with the most common adverse events being balance disorder and vomiting. Pharmacovigilance commitments include the standard SFDA adverse event reporting and the routine renal monitoring per the US label. Cold-chain at 2 to 8 degrees Celsius is required.

Reserve Meds' physician documentation kit includes the SFDA adverse-event reporting reference so the treating physician has the framework on hand from day one. Reserve Meds does not file adverse-event reports; that responsibility sits with the SCFHS-licensed treating physician. The dispensing facility carries the chain-of-custody and storage obligations through the dispensing event, and off-label transfer of the imported supply to another patient is not permitted under the PIP framework.

Common questions about Exondys 51 in the Kingdom

Will Bupa Arabia, Tawuniya, or MedGulf cover this? Each insurer handles named-patient imports case by case under the Council of Cooperative Health Insurance (CCHI) framework. Some plans reimburse fully when the medicine appears on the insurer's formulary even where the local hospital pharmacy does not stock it. Others reimburse a percentage. Many require pre-authorization with the clinical justification letter attached. Reserve Meds supplies the documentation that lets the insurer assess the case; the claim is yours or your hospital's to file. Cash-pay is the default operating posture for cross-border access, with reimbursement sought after delivery where your plan permits.

Will my Ministry of Health-employed physician's letter be sufficient if SFDA flags the case? Yes. KSA-licensed physicians at Ministry of Health hospitals, KFSH&RC, KAMC, MNGHA, KSUMC, and other public-sector institutions have full signing authority on PIP applications under their SCFHS license. The clinical justification letter is the cornerstone of

the package. Private-sector physicians at HMG, Saudi German, Fakeeh, Dallah, and similar institutions also have signing authority under their institutional license.

Can I receive the drug at home, or do I need a hospital? The dispensing facility must be a locally licensed pharmacy. For oral medicines, a hospital outpatient pharmacy or specialized SFDA-licensed import pharmacy dispenses to the patient. For infusion or injection products, the medicine ships to the infusion center where you will receive it. Direct-to-home delivery without a licensed dispensing facility in the chain is not the operating model.

What about pediatric patients? The PIP framework applies to pediatric patients the same way it applies to adults. The clinical justification letter typically includes weight-based dosing, pediatric-specific monitoring, and where relevant the involvement of the pediatric specialty center. KFSH&RC, KAMC, and the major HMG facilities have established pediatric specialty programs that handle named-patient imports routinely.

How does Saudization (Nitaqat) affect my case? Saudization is the workforce-nationalization program that shapes hospital staffing composition. It does not change the PIP framework. It can occasionally affect timing if a non-Saudi treating physician's license is in renewal at the moment the PIP file is being prepared. Confirming the prescriber's SCFHS license status before filing is good practice.

Is Exondys 51 a controlled substance? No. Exondys 51 is not a US DEA scheduled substance. The Saudi narcotics-section approvals do not apply. The chain-of-custody documentation, the dispensing facility's pharmaceutical establishment license, and the SFDA pharmacovigilance commitment do apply.

Where Reserve Meds fits in Exondys 51 cases

Reserve Meds is a US-based concierge coordinator. We do not replace your treating physician, the SFDA, the dispensing pharmacy, or the institutional import-pharmacy team. For a Exondys 51 case in the Kingdom, our work is to orchestrate the US-side sourcing, prepare the regulatory documentation kit your physician needs, coordinate international logistics with cold-chain validation where required, and assign a single named coordinator who stays with the case through reorders. The clinical decisions remain with the treating physician. The regulatory authority remains SFDA. The dispensing remains with the licensed Saudi pharmacy. Reserve Meds is the connective tissue between the US supply side and those three Saudi pillars. Reserved for you.

Next step

If a treating physician in the Kingdom is weighing Exondys 51 for a patient, the waitlist is the first step. We respond within 24 to 48 hours with an eligibility confirmation and a documentation kit for the physician.

Reserved for you.

Related

- Exondys 51 clinical resource
- Exondys 51 in the United Arab Emirates
- Exondys 51 in Qatar
- Exondys 51 in Kuwait
- Kingdom of Saudi Arabia country page

Sources

1. FDA approval, Exondys 51 (eteplirsen), Sarepta Therapeutics, initial FDA approval 2016.
2. Saudi Food and Drug Authority (SFDA), Personal Importation Program framework, https://www.sfda.gov.sa/en, and the Ghad digital regulatory platform at https://ghad.sfda.gov.sa/.
3. Saudi Vision 2030, Health Sector Transformation Program, https://www.vision2030.gov.sa/en/explore/programs/health-sector-transformation-program.
4. Saudi Commission for Health Specialties (SCFHS), https://scfhs.org.sa/en, for treating-physician licensing and the institutional pharmacy framework.

Review and oversight. Content on this page is reviewed by the Reserve Meds clinical and regulatory team. A US-licensed pharmacist reviews every prescription before dispensing. Regulatory posture is informational, not legal advice; case-specific questions route to retained outside counsel. Review methodology ›
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