

## Vyondys 53

Pakistan · access guide

# Vyondys 53 access in Pakistan: the DRAP named-patient pathway

Last reviewed 2026-05-16 by Reserve Meds clinical and regulatory team.

## Quick orientation

Vyondys 53 (golodirsén) is phosphorodiamidate morpholino oligomer (PMO) antisense exon-skipping therapy approved by the US FDA in December 2019 (accelerated approval) for Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation in the DMD gene amenable to exon 53 skipping (approximately 8 percent of DMD patients). The drug is manufactured by Sarepta Therapeutics. Pakistan patients use the Drug Regulatory Authority of Pakistan named-patient pathway when the locally registered indication, the stocked presentation, or the available payer coverage does not match what the prescribing physician has written. Reserve Meds coordinates the US-side sourcing through a DSCSA-compliant specialty channel, builds the documentation packet your physician needs to file, and orchestrates the logistics into Pakistan with a single named coordinator carrying the case end-to-end.

## Why Pakistani patients need Vyondys 53 through the named-patient pathway

Pakistan operates a developed pharmaceutical regulatory environment, and Vyondys 53 may be on the local register, may be in commercial review, or may be entirely absent depending on the stage of Sarepta Therapeutics's regional rollout. Several patterns drive cross-border requests. First, indication lag: newer indications, particularly the December 2019 (accelerated approval) FDA approval timeline, often reach local registration 12 to 36 months later. Second, biomarker-defined eligibility: genetic confirmation of a DMD gene mutation amenable to exon 53 skipping (typically MLPA followed by sequencing through a clinical neuromuscular genetics laboratory) can be the diagnostic gate, and where the relevant testing infrastructure is still maturing locally, families coordinate the workup before or in parallel with sourcing. Third, payer coverage: Adamjee Insurance, EFU Health, Jubilee, IGI, State Life, TPL, Salaam Takaful, and the Sehat Sahulat Program for eligible beneficiaries each assess specialty therapies case by case, and step-therapy criteria can fail even where the drug is registered. Fourth, stocking gaps: the local agent may not carry every presentation or dose strength reliably, and named-patient import is the operational mechanism that bridges to the exact label the prescriber has written. In each pattern, the named-patient pathway is the legal mechanism that connects a Pakistani-licensed physician's clinical decision with US-sourced, FDA-labelled product for a specific identified patient.

## **The DRAP named-patient pathway for Vyondys 53**

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The Drug Regulatory Authority of Pakistan (DRAP) administers personal-use imports under the DRAP Act 2012 read with the Drugs Act 1976. The Special Permission or NOC for Personal Use Import is filed via the DRAP OIES portal at dra.gov.pk and routes through the DRAP Division of Pharmacy Services. The framework permits hospitals and licensed pharmaceutical establishments to import a specific medicine for a specific patient when the medicine is approved by a recognised reference authority (typically the US FDA, EMA, MHRA, PMDA Japan, or Health Canada) and either a clinically equivalent locally registered alternative is not suitable, or the patient's clinical profile does not match the locally approved label.

A complete application for Vyondys 53 typically includes a clinical justification letter from the treating physician documenting the patient's diagnosis (Duchenne muscular dystrophy amenable to exon 53 skipping), severity assessment, prior systemic therapy history, any relevant biomarker results (genetic confirmation of a DMD gene mutation amenable to exon 53 skipping (typically MLPA followed by sequencing through a clinical neuromuscular genetics laboratory)), and a clinical rationale for selecting Vyondys 53 over locally available alternatives. The Pakistani physician's licensure with the Pakistan Medical Commission (PMC) and the relevant provincial healthcare regulators (Sindh Healthcare Commission, Punjab Healthcare Commission) is verified through the application. The packet also specifies the dispensing facility name and license number, the pharmacy in charge of the facility, full product details (brand name, generic name, manufacturer, strength, dosage form, pack size, quantity, intended treatment duration), and a chain-of-custody plan describing how the medicine will move from the US manufacturer through the importer to the dispensing pharmacy.

For Vyondys 53 specifically, the clinical justification typically frames the case around the exon-skipping eligibility is mutation-specific; clinical neuromuscular genetics referral with MLPA plus sequencing is the diagnostic anchor, and approximately 8 percent of DMD patients are exon 53 amenable. Approval timelines are typically 15 to 30 business days for routine cases; FX timing and bank documentation often govern wire scheduling more than DRAP itself. The DRAP retains discretion on timing, and we do not promise specific durations.

## **Where Vyondys 53 gets dispensed in Pakistan**

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A focused group of Pakistan institutions handle named-patient specialty-medicine imports as established workflow, with in-house import pharmacy capabilities and physicians experienced with the application set. For Vyondys 53 specifically, the dispensing facility must accommodate the administration profile: tertiary paediatric neurology or neuromuscular clinic with infusion capacity; weekly IV access (often a port over time) is part of the practical care plan. Tertiary centres that meet this profile include Shaukat Khanum Memorial Cancer Hospital and Research Centre (SKMCH&RC) in Lahore and Peshawar, Aga Khan University Hospital (AKUH) in Karachi, Liaquat National Hospital in Karachi, Indus Hospital in Karachi, Doctors Hospital Lahore, Hameed Latif Hospital, Shifa International Hospital in Islamabad, and Quaid-e-Azam International Hospital.

For physicians at smaller hospitals without internal import infrastructure, the common pattern is to route through a licensed pharmaceutical establishment that holds the import licence and files the DRAP application on the prescribing physician's behalf. The medicine then moves under chain-of-custody documentation into the prescribing hospital's outpatient pharmacy for administration.

## **Real cost picture for Vyondys 53 in Pakistan**

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US WAC for Vyondys 53 is approximately USD 1,200 per 100 mg dose, which translates to an annual WAC in the range of USD 300,000 to USD 700,000 per year (weight-banded, increases with patient growth) for the standard regimen at the labelled dose. The Pakistani rupee has been volatile against the US dollar; 1 USD is approximately 280 PKR as of May 2026 (flagged as indicative; we quote USD primary, and the State Bank of Pakistan outward remittance allowance for medical purposes is a practical sequencing constraint families plan around). On that basis, the drug cost alone is materially significant before logistics, the DRAP permit fees (which are nominal relative to drug cost), the destination dispensing hospital's administration fees, and Reserve Meds' concierge fee (which is itemised separately on every firm quote).

International cold-chain or ambient logistics into Pakistan typically runs in the low to mid four-figure USD range depending on origin, urgency, and packaging requirements. On the insurance side, Adamjee Insurance, EFU Health, Jubilee, IGI, State Life, TPL, Salaam Takaful, and the Sehat Sahulat Program for eligible beneficiaries each assess named-patient imports case by case. Some reimburse fully when the medicine is on their formulary even if not stocked, some reimburse a percentage subject to copay, and many require pre-authorisation with documented step-therapy failure. We do not promise coverage from any payer. US manufacturer patient assistance programmes do not extend internationally; cross-border patients pay cash or rely on local payer coverage where available.

## **Clinical evidence and where Vyondys 53 sits in the treatment landscape**

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the phase 1/2 ESSENCE / 4053-101 study and confirmatory ongoing trials demonstrated dystrophin production increases on muscle biopsy with a favourable safety profile. The drug acts as phosphorodiamidate morpholino oligomer (PMO) antisense exon-skipping therapy, and the dosing schedule is 30 mg/kg IV infusion once weekly through a central or peripheral venous access; infusion runs 35-60 minutes.

Within the treatment landscape, Vyondys 53 sits alongside eteplirsen for exon 51 skipping, casimersen for exon 45 skipping, viltolarsen as an alternative exon 53 agent (NS Pharma), corticosteroids (deflazacort, prednisone) as standard DMD background, and elevidys gene therapy in DMD amenable to AAVrh74 micro-dystrophin. The choice between targeted therapies in this space depends on the patient's full clinical profile, prior therapy exposure, biomarker status, comorbidities, and the prescriber's judgment. Reserve Meds coordinates whichever therapy the physician has selected; we do not steer prescribing.

Safety surveillance for Vyondys 53 centres on hypersensitivity reactions including bronchospasm and rash, renal toxicity with regular monitoring of cystatin C-based eGFR, and headache. The dispensing facility and the prescribing physician retain clinical responsibility for monitoring and adverse-event management; Reserve Meds does not provide medical care.

## Typical timeline for Vyondys 53 in Pakistan

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DRAP routine processing is typically 15 to 30 business days for routine cases; FX timing and bank documentation often govern wire scheduling more than DRAP itself from a complete filing. End-to-end, most cases complete within 4 to 8 weeks from first complete documentation, with first-of-kind cases and complex biomarker-dependent workups potentially extending further. Where the administration setting is tertiary paediatric neurology or neuromuscular clinic with infusion capacity, hospital scheduling and infusion-chair availability are additional sequencing factors that families plan around. We do not promise specific durations; the DRAP retains discretion on timing, and shipping windows depend on lane and packaging.

## What your Pakistani physician needs to provide

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For a Pakistani-licensed specialist prescribing Vyondys 53 through the DRAP pathway, the clinical justification letter is the cornerstone of the application. The letter typically documents the patient's diagnosis (Duchenne muscular dystrophy amenable to exon 53 skipping), the relevant biomarker work (genetic confirmation of a DMD gene mutation amenable to exon 53 skipping (typically MLPA followed by sequencing through a clinical neuromuscular genetics laboratory)), prior systemic therapy history, the FDA-approved indication being invoked, and the clinical rationale for Vyondys 53 as the appropriate next step.

The letter also specifies the exact dosing plan per the FDA-approved label: 30 mg/kg IV infusion once weekly through a central or peripheral venous access; infusion runs 35-60 minutes. The monitoring plan references hypersensitivity reactions including bronchospasm and rash, renal toxicity with regular monitoring of cystatin C-based eGFR, and headache. The treating physician's licence number with the Pakistan Medical Commission (PMC) and the relevant provincial healthcare regulators (Sindh Healthcare Commission, Punjab Healthcare Commission), the dispensing facility license number, and the pharmacy in charge of dispensing complete the package. Where biomarker testing requires reference-lab coordination, the physician documents the assay used and the report; Reserve Meds can route this through a US-side reference laboratory where the regional pathway is unavailable.

## Common questions about Vyondys 53 in Pakistan

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**Will Adamjee Insurance or other major Pakistani insurers cover Vyondys 53?** Each insurer assesses named-patient imports case by case. Some reimburse fully when Vyondys 53 is on their formulary even if not currently stocked; others assess based on step-therapy criteria and biomarker documentation. We supply the documentation set that allows your insurer to assess the case; the claim itself sits with you, your physician, or your hospital. We do not promise coverage from any payer.

**Is Vyondys 53 registered locally in Pakistan?** Local registration status changes as Sarepta Therapeutics pursues regional rollout; even where the drug is registered, the specific indication, presentation, or dosing strength your prescriber has written may not align with what is currently stocked. The DRAP named-patient pathway exists precisely to bridge these gaps for individually identified patients.

**What about competitor therapies?** The treatment landscape includes eteplirsen for exon 51 skipping, casimersen for exon 45 skipping, viltolarsen as an alternative exon 53 agent (NS Pharma), corticosteroids (deflazacort, prednisone) as standard DMD background, and elevidys gene therapy in DMD amenable to AAVrh74 micro-dystrophin. The choice depends on the patient's full clinical profile and prescriber judgment. Reserve Meds coordinates whichever medicine the physician has prescribed; we do not steer prescribing decisions and we do not have a financial relationship with any specific manufacturer.

**How is the cold chain or storage managed?** Vyondys 53 ships in validated packaging with continuous temperature logging through the lane where cold-chain handling applies. The handoff ends at the dispensing pharmacy; home storage instructions, where the patient takes the medicine home for self-administration, are part of the patient onboarding kit.

**Do US manufacturer patient assistance programmes (such as Sarepta Therapeutics co-pay or PAP programmes) extend to Pakistan patients?** No. US-resident patient assistance programmes are limited to US-resident patients with US prescription coverage by programme design. Cross-border patients pay cash for the drug and the coordination fee, with local payer reimbursement assessed separately.

**Can the case be resupplied year over year if the patient responds?** Yes. Reserve Meds maintains the case file and re-files DRAP permits at the relevant intervals (or coordinates with the dispensing hospital's pharmacy if they hold the permit). Patients on long-term therapy typically settle into a quarterly or biannual resupply cadence after the first cycle.

**What is the administration setting?** Tertiary paediatric neurology or neuromuscular clinic with infusion capacity; weekly iv access (often a port over time) is part of the practical care plan.

**My physician is at a smaller hospital without an internal import pharmacy. Can the case still proceed?** Yes. The common pattern is to route through a Dubai, Riyadh, Mumbai, Cairo, Karachi, or other regional licensed pharmaceutical establishment that holds the import licence and files the DRAP application on the prescribing physician's behalf. The medicine moves into the prescribing hospital's outpatient pharmacy under chain-of-custody documentation.

## Where Reserve Meds fits in Vyondys 53 cases

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Reserve Meds is a US-based concierge coordinator. We do not replace your Pakistani specialist, we do not replace the DRAP, and we do not replace your dispensing pharmacy. For Vyondys 53 specifically, we orchestrate the US-side sourcing through a DSCSA-compliant specialty channel, build the documentation packet your physician submits, coordinate logistics into Pakistan, and assign a single named coordinator through the case. The pharmacist-of-record review, prescription validation, biomarker confirmation, and physician sign-off are the recurring operational fundamentals for this drug.

### *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.

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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