Tegsedi access in Kuwait: the MOH-KDFC named-patient pathway

How Kuwait families pursue inotersen, an FDA-approved antisense oligonucleotide for hereditary transthyretin-mediated amyloidosis polyneuropathy, when the local supply chain does not reach the rare-disease patient.

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

Quick orientation

Tegsedi (inotersen) is a once-weekly subcutaneous antisense oligonucleotide developed approximately by Akcea Therapeutics in collaboration with Ionis Pharmaceuticals and now held by Sobi (Swedish Orphan Biovitrum), with US FDA approval in October 2018 for the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis with polyneuropathy) in adults. Tegsedi works by hybridising to transthyretin mRNA and reducing hepatic synthesis of the disease-causing transthyretin protein. It is one of three TTR-lowering agents available globally for hATTR polyneuropathy (alongside patisiran and the newer vutrisiran, both RNA interference therapeutics), and it operates under a REMS program in the US given the boxed-warning risk of severe thrombocytopenia and glomerulonephritis. For a Kuwait family weighing this option, the practical question is rarely whether TTR-lowering therapy is the right move once the diagnosis is confirmed. The treating neurologist or cardiologist has typically already decided. The practical question is how to access inotersen specifically when the consultant has chosen the antisense oligonucleotide pathway over the RNA interference pathway, and how to maintain the safety-monitoring schedule that the drug requires. Reserved for you.

Why Tegsedi is hard to source in Kuwait

Hereditary transthyretin amyloidosis is a rare disease globally and in Kuwait. The clinical presentation typically involves progressive sensorimotor polyneuropathy, autonomic neuropathy, and frequently a concurrent cardiomyopathy, with onset most commonly in mid-adult life and faster progression in patients with early-onset variants. Diagnosis requires the combination of clinical presentation, electrodiagnostic confirmation of polyneuropathy, transthyretin gene sequencing, and frequently a cardiac evaluation including pyrophosphate scintigraphy or cardiac MRI for amyloid burden. In Kuwait,

neurology centers at Ibn Sina Hospital and Mubarak Al-Kabeer Hospital manage the small known cohort, and the genetic testing infrastructure for TTR variant confirmation is in place.

Three structural realities follow. First, hATTR polyneuropathy is rare enough in absolute Kuwait numbers that hospital pharmacy formularies do not stock TTR-lowering therapies as a routine matter; each case runs through named-patient procurement. Second, Tegsedi may be registered through the GCC central pathway or in-country at any given review date, but registration does not equal stocking. Third, inotersen carries a boxed warning for severe thrombocytopenia and glomerulonephritis and operates under a REMS program in the US with mandatory weekly platelet count monitoring through the early treatment phase and biweekly monitoring thereafter, plus monthly serum creatinine and urinalysis. The mandatory monitoring infrastructure is part of the access decision in any jurisdiction. The named-patient pathway exists to close exactly that supply gap for the individual patient whose consultant has decided inotersen is the right move.

The Kuwait MOH-KDFC named-patient pathway applied to Tegsedi

The pathway for a Kuwait-licensed consultant neurologist or cardiologist to obtain an unregistered or unstocked rare-disease therapeutic for a specific patient is the unregistered-medicine personal-import permit administered by the Kuwait Drug & Food Control Administration (KDFC) under the Ministry of Health. For Tegsedi, the standard application set applies, with one specific addition for an antisense oligonucleotide with a REMS program. The clinical justification letter from the treating consultant documents the diagnosis (hereditary transthyretin amyloidosis with the TTR variant identified by gene sequencing, the clinical phenotype with polyneuropathy stage, electrodiagnostic findings, autonomic involvement, and any concurrent cardiomyopathy with the cardiac biomarker and imaging baseline), the prior therapy sequence (which is typically supportive care or, in some cases, a prior TTR-lowering agent), and the rationale for inotersen at this point (the antisense oligonucleotide pathway selection over the RNA interference alternatives).

A complete KDFC application for a Tegsedi case typically includes the clinical justification letter, the treating consultant's Kuwait Medical Council registration verification, an anonymised patient identifier (or Civil ID for nationals and residents), product details for Tegsedi (inotersen, Sobi, 284 mg single-dose prefilled syringe, the planned dosing at 284 mg subcutaneously once weekly, and the requested treatment duration, typically 90 days for an initial pull with refill cycles to follow), the destination dispensing facility name with license number and pharmacy in charge, the cold-chain plan from the US manufacturer through the Kuwait importer to the dispensing pharmacy, and an explicit safety-monitoring plan documenting how the patient will receive weekly platelet count monitoring through the

early treatment phase, biweekly platelet count monitoring thereafter, and monthly serum creatinine and urinalysis.

Real costs in KWD and USD

The US wholesale acquisition cost for Tegsedi is approximately USD 8,500 to 9,500 per 284 mg prefilled syringe. At the once-weekly dosing schedule, the monthly drug cost runs approximately USD 37,000 to 41,000. At the indicative exchange of 1 KWD to 3.25 USD, the monthly drug cost translates to approximately KWD 11,400 to 12,600. The Kuwaiti dinar is the highest-valued currency unit in the world by exchange rate, so the cost looks smaller in KWD than in USD, but the underlying USD cost is what drives the manufacturer release price and shipping economics. Reserve Meds quotes always render both currencies on the firm quote.

Drug cost is not the entire cost. Cold-chain international logistics for a 2-8 degrees Celsius injectable biologic to Kuwait International Airport, customs clearance, KDFC permit fee, and Reserve Meds' concierge fee are itemised separately. Total all-in for a one-month Tegsedi supply delivered to a Kuwait dispensing pharmacy typically lands in the USD 38,500 to 43,000 range (approximately KWD 11,800 to 13,200), with the drug cost dominating. The mandatory monitoring lab work (platelet counts, serum creatinine, urinalysis) is the patient's clinic-side cost and is not part of the Reserve Meds quote. Insurance in Kuwait handles named-patient rare-disease imports case by case. The MOH public-system specialty pharmacy may cover rare-disease therapeutics for Kuwaiti nationals when the consultant documents the genetic confirmation and clinical phenotype through the standard KDFC named-patient channel, but coverage is not promised. For expatriate patients on Afya or private employer plans, pre-authorisation is the norm. We supply the documentation set that lets your insurer assess the case. We do not promise coverage from any insurer.

Timing: what to expect

The KDFC permit itself is not the long pole for most cases. Routine submissions process in 7 to 21 business days. Rare-disease submissions with first-of-kind clinical justifications can extend to 4 to 6 weeks given the substantive review. The patient-experience timeline runs from the consultant's prescription decision through documentation assembly (Reserve Meds returns a documentation kit to the physician within 24 to 48 hours of waitlist intake), permit filing, US-side sourcing alignment, manufacturer release, cold-chain air freight to Kuwait International Airport, customs clearance with priority cold-chain handling, and dispensing-pharmacy intake. A typical first-cycle window for cold-chain antisense oligonucleotide therapeutics to Kuwait is 4 to 6 weeks from waitlist intake to first dose, dependent on consultant documentation turnaround and KDFC processing speed.

One Tegsedi-specific timing note. The once-weekly subcutaneous dosing schedule means continuous supply is non-negotiable. A missed dose in the early treatment phase, before TTR knockdown is established, has clinical consequence. Reserve Meds defaults to a 90-day initial pull (covering approximately 12 weekly doses) and continues monthly or quarterly as the consultant directs.

What your physician needs

The clinical justification letter for a Tegsedi KDFC submission typically addresses the diagnosis (hereditary transthyretin amyloidosis with the TTR variant identified by gene sequencing, the affected family pedigree where available, the polyneuropathy stage with electrodiagnostic findings and FAP staging or similar, the autonomic involvement status, any concurrent cardiomyopathy with NT-proBNP, troponin, and cardiac imaging baseline), the documented prior therapy (typically supportive care; in some cases a prior TTR-lowering agent with reason for discontinuation), the rationale for inotersen at this point (the antisense oligonucleotide pathway selection over the RNA interference alternatives patisiran and vutrisiran, with the specific clinical case for the patient), the planned dosing (284 mg subcutaneously once weekly), the planned safety monitoring (weekly platelet count monitoring through the early treatment phase, biweekly platelet count monitoring thereafter, monthly serum creatinine and urinalysis, with explicit clinic responsibility for the monitoring schedule), and an acknowledgment of the boxed warning content (severe thrombocytopenia and glomerulonephritis) with the dose-modification and discontinuation criteria documented.

Three documents sit alongside the letter. The treating consultant's Kuwait Medical Council registration verification is part of the submission. The patient and family informed consent for a Kuwait dispensing facility's named-patient import is documented before the KDFC submission goes in. The vitamin A supplementation plan (recommended for patients on TTR-lowering therapies given TTR's role as a vitamin A transport protein) is part of the standard care package. For a Kuwait public-system case, the dispensing facility is typically the neurology service at Ibn Sina Hospital (national referral center for neurology and neurosurgery), Mubarak Al-Kabeer Hospital, Sheikh Jaber Al-Ahmad Al-Sabah Hospital, or the cardiology service at Sheikh Jaber Al-Ahmad Al-Sabah Center for Cardiac Diseases for cases with predominant cardiac involvement; for private cases, Dar Al Shifa or New Mowasat are common dispensing sites depending on the consultant's primary affiliation.

KCCC and the Kuwait specialty-dispensing network

For a Tegsedi (neurology and cardiology) rare-disease case, the dispensing network is the Kuwait neurology and cardiology footprint rather than the oncology footprint anchored by KCCC. Ibn Sina Hospital at the Sabah Health Region is the national referral center for

neurology and neurosurgery and is the natural dispensing center for hATTR polyneuropathy cases with predominant peripheral neuropathy. Mubarak Al-Kabeer Hospital in Jabriya carries adult neurology and is a primary teaching site. Sheikh Jaber Al-Ahmad Al-Sabah Hospital in Jaber Al-Ahmad City carries broad adult neurology capacity. Sheikh Jaber Al-Ahmad Al-Sabah Center for Cardiac Diseases is the natural dispensing center for hATTR cases with predominant cardiomyopathy. Al-Sabah Hospital, on the Sabah campus, anchors central Kuwait public neurology and internal medicine.

On the private side, Dar Al Shifa Hospital in Hawalli carries adult neurology and cardiology service lines. New Mowasat Hospital in Salmiya, Royale Hayat Hospital in Jabriya, and Al Salam International Hospital in Bneid Al-Gar each carry specialty service lines that work with Kuwait-licensed specialty importers on named-patient cases. Reserve Meds does not select the dispensing facility on the patient's behalf. We work with the dispensing facility the consultant has named.

Pharmacovigilance and cold-chain

Tegsedi is a 2-8 degrees Celsius cold-chain injectable antisense oligonucleotide supplied in prefilled syringes for once-weekly subcutaneous self-administration after appropriate patient and caregiver training. Cold-chain integrity is the dominant risk in the inbound logistics window. The Kuwait climate (peak summer ambient temperatures regularly exceed 45 degrees Celsius) makes the airport-to-hospital leg the highest-risk transit. Reserve Meds defaults to validated insulated shippers with phase-change cold packs sized for 96-hour stability for 2-8 degrees Celsius products, with continuous data loggers on every shipment to document compliance with the labeled storage range from origin through to the dispensing pharmacy refrigerator and to the patient's home refrigerator.

Pharmacovigilance reporting for Tegsedi in Kuwait runs through the KDFC Drug Safety Department, working with the GCC Centre for Pharmacovigilance based in Riyadh. The treating consultant and the dispensing facility share a duty to report adverse drug reactions. Serious adverse reactions (severe thrombocytopenia with bleeding events, glomerulonephritis with significant creatinine rise or proteinuria, severe injection-site reactions, hypersensitivity reactions, vitamin A deficiency symptoms despite supplementation, hepatotoxicity) typically require reporting within 15 calendar days. Reserve Meds does not file adverse-event reports on the consultant's behalf; the obligation sits with the prescriber and the dispensing facility, and the mandatory monitoring schedule documentation is the cornerstone of safe use.

Common questions about Tegsedi in Kuwait

Why inotersen and not patisiran or vutrisiran? Reserve Meds does not steer the clinical decision. The choice among TTR-lowering agents (inotersen antisense oligonucleotide,

patisiran lipid nanoparticle siRNA, vutrisiran subcutaneous siRNA, the older non-TTR-lowering tafamidis stabilizer for cardiomyopathy) rests with the treating neurology and cardiology team and reflects the specific clinical phenotype, prior therapy, comorbidities, and consultant preference. The mandatory monitoring profile differs across the agents. We source whichever TTR-lowering agent the consultant has named.

What about Wainua (eplontersen)? Wainua, also an antisense oligonucleotide targeting TTR, was FDA-approved in December 2023 as a once-monthly subcutaneous alternative to Tegsedi and is positioned as a successor in the Akcea/Ionis antisense pipeline. The clinical choice between Tegsedi and Wainua rests with the treating consultant. We source whichever the consultant has named.

Is Tegsedi a controlled substance? No. Tegsedi is not a DEA-scheduled substance. The MOH Narcotic and Psychotropic Drugs Control framework does not apply. Standard KDFC named-patient permit documentation is sufficient, with explicit attention to the safety-monitoring infrastructure requirement.

What about the REMS program? The US Tegsedi REMS program requires mandatory weekly platelet count monitoring through the early treatment phase and biweekly thereafter, plus monthly serum creatinine and urinalysis. The Kuwait dispensing pathway adopts the equivalent monitoring schedule under the treating consultant's direction. The lab infrastructure for these tests is well-established in Kuwait public-system hospitals.

What about vitamin A supplementation? Patients on TTR-lowering therapy require vitamin A supplementation because TTR is a vitamin A transport protein. The treating consultant's plan documents the daily supplementation regimen typically aligned with the recommended daily allowance and the periodic vitamin A level monitoring.

Where Reserve Meds fits in Tegsedi cases

Reserve Meds is a US-based concierge coordinator. We do not replace your treating neurologist, cardiologist, the KDFC, the dispensing pharmacy, or your Kuwait consultant. For a Tegsedi case specifically, our work is the documentation kit, the US-side sourcing of the manufacturer pack, the cold-chain shipment to Kuwait International Airport, the chain-of-custody handoff to your Kuwait importer or hospital pharmacy, and the named-coordinator continuity through refill cycles. Rare-disease cases with mandatory monitoring schedules run on continuity and discipline. Reserve Meds is built for that continuity. Reserved for you.

Next step

If a treating neurologist or cardiologist in Kuwait is weighing Tegsedi for a patient with hereditary transthyretin amyloidosis polyneuropathy, the waitlist is the first step. We respond within 24 to 48 hours with an eligibility confirmation and a documentation kit for the consultant.

Reserved for you.

Related

- Tegsedi clinical resource
- Tegsedi in Saudi Arabia
- Tegsedi in the UAE
- Kuwait country page

Sources

1. FDA approval, Tegsedi (inotersen), Akcea Therapeutics in collaboration with Ionis Pharmaceuticals; now held by Sobi (Swedish Orphan Biovitrum); approval October 2018 for the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults.
2. Kuwait Ministry of Health, Drug & Food Control Administration; KDFC permit framework for unregistered medicines under the Pharmacy and Practice of Pharmacy Profession Law.
3. Manufacturer label and prescribing information for Tegsedi; 284 mg once-weekly subcutaneous dosing, boxed warning for thrombocytopenia and glomerulonephritis, REMS program, mandatory weekly and biweekly platelet count monitoring, monthly serum creatinine and urinalysis, vitamin A supplementation guidance.

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 ›
Last medically reviewed: 2026-05-12.