Amvuttra

Abu Dhabi · access guide

How to access Amvuttra for hereditary TTR amyloidosis from Abu Dhabi: 2026 pathway via Abu Dhabi neurology, cardiology, and pharmacy supply

By Reserve Meds clinical & regulatory team. Last reviewed 2026-05-20.

Abu Dhabi carries the deepest in-region cardiology amyloid programme in the UAE. Cleveland Clinic Abu Dhabi runs a substantial multidisciplinary amyloid clinic with 99m-technetium pyrophosphate (PYP) scintigraphy, cardiac MRI, advanced echocardiographic strain imaging, in-house TTR sequencing, and a co-located heart-failure and electrophysiology service. Sheikh Shakhbout Medical City (SSMC), under the MD Anderson Cancer Center clinical affiliation, carries advanced cardiology and neurology and a developing rare-disease pathway. Sheikh Khalifa Medical City (SKMC), Burjeel Medical City, Yas Clinic Hospital, and the NMC tertiary network add depth across the emirate, and Tawam Hospital in Al Ain carries long-standing internal medicine and neurology. For UAE patients across the federation, including many Dubai-based patients seeking the deepest cardiac amyloid imaging available, Abu Dhabi is the in-emirate referral hub.

Amvuttra (vutrisiran) is Alnylam Pharmaceuticals' GalNAc-conjugated small interfering RNA (siRNA) therapy for hereditary TTR amyloidosis with polyneuropathy (hATTR-PN) and, since the March 2025 label expansion, for ATTR cardiomyopathy (ATTR-CM) in both hereditary and wild-type forms. For an Abu Dhabi-resident adult with confirmed TTR amyloidosis, the operational question is which agent fits, where the prescribing amyloid clinic conversation happens, how the quarterly subcutaneous routine works, how genetic-testing and family-screening dimensions are handled with Emirati family network involvement, and how the Thiqa rare-disease orphan-therapy pathway runs for nationals or commercial pre-authorisation runs for residents. This page explains the 2026 pathway.

Why Amvuttra, and why now

Amvuttra is vutrisiran, a 21-nucleotide double-stranded siRNA conjugated to GalNAc and recognised by the hepatic asialoglycoprotein receptor. RISC-mediated cleavage of TTR mRNA reduces serum transthyretin by more than 80 percent, which over time slows or partially reverses peripheral nerve and cardiac amyloid deposition.

FDA approved June 2022 for hATTR-PN (HELIOS-A); ATTR-CM expansion March 2025 (HELIOS-B). For Abu Dhabi the relevant UAE-side question is `[VERIFY: DoH/EDE registration status for ATTR-CM indication]`.

For an Abu Dhabi patient with progressive sensorimotor polyneuropathy attributed to a confirmed TTR mutation, or with ATTR cardiomyopathy confirmed by PYP scintigraphy and AL exclusion at Cleveland Clinic Abu Dhabi or SSMC, Amvuttra is the operational pathway to a disease-modifying therapy administered four times per year. The quarterly cadence is a meaningful simplification compared with Onpattro (IV every 3 weeks), Tegsedi (SC weekly), and Wainua (SC monthly).

What Amvuttra is, in plain language

Subcutaneous injection every 3 months. No infusion centre, no inpatient stay, no IV access. Dose: 25 mg as a single prefilled syringe. Administration at the prescribing amyloid clinic or, after training, at home.

Injection sites: abdomen, thigh, or upper outer arm; rotate between quarterly doses.

Storage: 2 to 8 degrees Celsius. Bring to room temperature for 30 minutes before injection. Do not freeze; do not shake.

Treatment is indefinite, for as long as Amvuttra provides clinical benefit and is tolerated. Response is assessed by serum TTR reduction (target engagement, typically greater than 80 percent), neurology scoring (mNIS+7 plus Norfolk QoL-DN) for hATTR-PN, and cardiology scoring (NT-proBNP, 6-minute walk, echo strain) for ATTR-CM.

Mandatory vitamin A supplementation: the siRNA mechanism reduces hepatic vitamin A transport (retinol binding protein 4 is made by the liver and falls when TTR is suppressed). All patients on Amvuttra take oral vitamin A at the recommended daily allowance (approximately 2,500 to 3,000 IU/day for adults) for the duration of treatment. This is a mandatory and lifelong companion to the therapy. It is not negotiable.

Eligibility at an Abu Dhabi amyloid clinic

1. **Confirmed TTR amyloidosis**. For polyneuropathy: TTR sequencing plus clinical features of progressive sensorimotor and/or autonomic neuropathy, plus where indicated tissue biopsy with Congo red staining and amyloid typing. For cardiomyopathy: TTR sequencing (hereditary form) or non-biopsy diagnosis via 99m-Tc-PYP scintigraphy with grade 2 or 3 myocardial uptake, plus AL exclusion. Equivocal cases proceed to endomyocardial biopsy. 2. **AL amyloidosis exclusion**. Serum free light chains, serum and urine immunofixation. Required, because Amvuttra targets TTR mRNA only; a patient with AL amyloidosis treated with Amvuttra would continue to deposit amyloid and progress. Haematology consultation if equivocal. 3. **Genetic counselling** for confirmed hereditary forms. First-degree relatives offered TTR sequencing and clinical surveillance. Cleveland Clinic Abu Dhabi and SSMC genetic counselling services are the right home for this conversation. 4. **Baseline neurology assessment** (hATTR-PN): mNIS+7, Norfolk QoL-DN, 10-metre walk test, modified Body Mass Index, autonomic testing where indicated. 5. **Baseline cardiology assessment** (ATTR-CM or hATTR-PN with cardiac involvement): NT-proBNP, troponin, echo with strain, cardiac MRI, 99m-Tc-PYP scintigraphy. Cleveland Clinic Abu Dhabi carries all of these as standard inputs to its multidisciplinary amyloid clinic. 6. **Treatment-naive vs switching status** from Onpattro, Tegsedi, Wainua, tafamidis, or acoramidis. Each switch has specific washout and overlap considerations. 7. **Vitamin A baseline and supplementation plan**. Baseline serum vitamin A (retinol) level, ophthalmology referral if symptoms or risk factors exist. Supplementation started at or before first dose, continued lifelong. 8. **Pregnancy planning** for women of childbearing potential. Effective contraception during treatment. No human data; animal data suggest teratogenicity from vitamin A depletion. 9. **Renal and hepatic function review**. Standard baseline labs.

An Abu Dhabi patient should arrive at the amyloid clinic with available diagnostic documentation: prior neurology or cardiology workup, nerve conduction studies, echo and cardiac imaging, prior amyloid biopsy results, family history (multi-generational sensorimotor neuropathy or unexplained cardiomyopathy is the dominant pattern), and current medications.

The Abu Dhabi prescribing and supply picture, plainly

Amvuttra availability in Abu Dhabi runs through federal EDE registration with DoH operating at the emirate level. The cardiomyopathy indication's current label status should be `[VERIFY: DoH/EDE registration status for ATTR-CM indication]` at intake. Alnylam's MENA commercial footprint runs through specialty distributor partners.

1. **Prescribing neurologist and/or cardiologist with amyloidosis experience**. Amvuttra is a dual-specialty drug. The Abu Dhabi centres with the deepest amyloid programmes are Cleveland Clinic Abu Dhabi (regional referral hub for cardiac amyloid: PYP scintigraphy, cardiac MRI, strain echo, in-house TTR sequencing, multidisciplinary amyloid clinic) and SSMC (advanced cardiology, neurology, rare-disease pathway). SKMC, Burjeel Medical City, Yas Clinic Hospital, and the NMC tertiary network carry supporting workup. Tawam Hospital in Al Ain handles internal medicine and neurology referrals into the emirate hub. Public-sector pathways for Emirati nationals route primarily through Cleveland Clinic Abu Dhabi (Mubadala-affiliated) and SSMC under the SEHA umbrella. 2. **Genetic testing infrastructure**. Cleveland Clinic Abu Dhabi runs an in-house molecular laboratory; SSMC and private tertiaries send samples to regional reference labs or Centogene/Invitae partners. Turnaround typically 4 to 8 weeks. 3. **Cardiac amyloid imaging**. 99m-Tc-PYP scintigraphy at Cleveland Clinic Abu Dhabi and SSMC as standard; cardiac MRI widely available. 4. **Pharmacy dispensing**. Specialty pharmacy at the prescribing tertiary centre, cold-chain refrigeration. Where the indication is not yet registered, a named-patient pathway applies. 5. **Insurance and state-funded coverage**. For Emirati nationals, Thiqa (the dominant national pathway through Daman) covers rare-disease orphan therapies on a case-by-case basis; the Thiqa pre-authorisation runs through the prescribing centre's case-management team in parallel with the clinical workup. For non-Emirati residents, commercial covers (Daman, MetLife, Cigna, Bupa Arabia, Aetna, AXA Gulf) handle pre-authorisation with documented medical necessity. Smaller plans typically lack the rare-disease rider. 6. **Self-injection training**. Single supervised session at the prescribing amyloid clinic, or an Alnylam patient-support nurse educator visit. Many Abu Dhabi patients keep the quarterly injection as a clinic visit; the quarterly clinic visit doubles as a clinical check-in. 7. **Ongoing monitoring**. Amyloid clinic follow-up at 6 and 12 months, then annually. Serum TTR level at intervals to confirm target engagement. Vitamin A serum level and ophthalmology assessment if deficiency symptoms develop.

The 2026 pathway, step by step

Week 0 to 4: Diagnostic confirmation with the treating amyloid clinic (Cleveland Clinic Abu Dhabi or SSMC most commonly). TTR sequencing if not already done, AL exclusion labs, baseline neurology or cardiology scoring, baseline PYP scintigraphy. If TTR sequencing is the gating step, the timeline extends to lab turnaround (4 to 8 weeks).

Week 4 to 8: Thiqa or commercial pre-authorisation in parallel with the diagnostic workup.

Week 8 to 12: First dose dispensing at the prescribing amyloid clinic. Vitamin A supplementation started. Self-injection training if the patient prefers home administration.

Month 3: Second quarterly dose; cold-chain logistics for home administration where applicable.

Month 6 to 12: Response assessment. Serum TTR reduction confirmed. Neurology or cardiology scoring compared to baseline. Vitamin A serum level reviewed.

Month 12 onwards: Maintenance quarterly dosing; annual amyloid clinic review; family-screening conversation continues over time.

Cost expectation in AED

US list price (WAC) for Amvuttra is approximately USD 463,500 per year (USD 116,000 per quarterly dose). Cash-pay retail in the UAE specialty channel commonly sits in the range of USD 350,000 to 480,000 per year, with per-quarterly-dose pricing in the region of AED 426,000.

At 2026 indicative cross rates (1 AED is approximately USD 0.272), the AED-equivalent annual cost band is approximately AED 1,285,000 to 1,765,000 at cash-pay retail. For Emirati nationals with Thiqa coverage, the rare-disease orphan-therapy pathway typically covers Amvuttra on a documented case-by-case basis. The financial pre-authorisation conversation needs to start before the first dispensing, not after. Daman and other large commercial covers vary; the prescribing amyloid clinic's case-management team is the gating step.

For non-Emirati residents whose cover does not extend to rare-disease orphan therapy, the cash-pay exposure is the full annual band. Reserve Meds surfaces this reality early. Cross-border named-patient supply, where applicable, adds modest overhead but does not materially change the underlying drug cost.

What to monitor

The mandatory safety conversation for Amvuttra centres on vitamin A.

Vitamin A deficiency. The siRNA mechanism reduces hepatic retinol binding protein 4 along with TTR, lowering vitamin A transport. All patients take oral vitamin A at the recommended daily allowance (approximately 2,500 to 3,000 IU/day for adults) for the duration of treatment. Without supplementation, deficiency manifests slowly over months to years as night vision difficulty, dry eyes, or in extreme cases corneal changes. Ocular symptoms trigger ophthalmology assessment with serum vitamin A measurement. Supplementation is non-negotiable and lifelong.

Injection-site reactions (redness, swelling, mild pain) are common and resolve with site rotation and standard local care.

Limb pain and arthralgia at modestly higher rates than placebo in the pivotal trials; mostly mild to moderate.

Falls in patients with autonomic involvement; fall prevention counselling is part of the standard follow-up.

Pregnancy: contraindicated; effective contraception required. Discontinuation planning is managed by the treating amyloid clinic given the long half-life of TTR mRNA suppression.

No specific cardiac, hepatic, or renal toxicity signal from the siRNA mechanism itself. Favourable adverse-event profile compared with the antisense oligonucleotide alternatives.

Religious, ethical, and family-logistics framing

Amvuttra is a synthetic chemical: a chemically modified short double-stranded RNA conjugated to a sugar ligand (GalNAc). There is no human or animal source material, no donor element, no foreign cells, no viral vector. The product is halal-compatible and kosher-compatible by general consensus on synthetic RNA therapeutics. Written halal-certification documentation of the specific commercial product can be requested through Alnylam at intake.

The quarterly cadence accommodates travel, work, multi-generational Emirati family commitments, Ramadan, and Hajj or Umrah travel far more easily than a weekly or monthly self-injection. For families spread across the emirate, Al Ain, and the wider UAE, this matters in practice.

The genetic dimension is the more sensitive cultural conversation. Hereditary TTR amyloidosis is autosomal dominant with variable penetrance and age of onset. A confirmed case in an Emirati family carries implications for first-degree relatives, who may be presymptomatic or have symptoms attributed to other causes. The page does not push specific family-disclosure decisions. The Cleveland Clinic Abu Dhabi or SSMC genetic counselling service is the right home for that conversation, and Reserve Meds supports the family in coordinating sibling and adult-child genetic testing where the family chooses to pursue it. Extended-family consultation is the standard pattern in Abu Dhabi; the timing is the family's call.

Vitamin A supplementation deserves a separate practical note. Patients who would not realistically take a daily oral supplement for years should discuss this frankly at initiation. Treatment without it is not the right course.

When Amvuttra is not the right call

For an Abu Dhabi patient whose amyloidosis is AL rather than TTR, Amvuttra has no role. AL amyloidosis is treated under haematology care; the diagnostic distinction is the gating safety step.

For confirmed TTR amyloidosis with milder cardiomyopathy and no progressive polyneuropathy, where the operational simplicity of an oral once-daily therapy outweighs the deeper TTR suppression of an RNAi or ASO mechanism, tafamidis (Vyndaqel/Vyndamax) or acoramidis (Attruby) is the appropriate alternative. The amyloid clinic conversation about Amvuttra versus tafamidis versus acoramidis is the central clinical decision.

For a patient who cannot or will not comply with mandatory vitamin A supplementation, Amvuttra is not appropriate; tafamidis or acoramidis are operationally simpler and do not carry the vitamin A obligation.

For pregnancy or near-term pregnancy planning, Amvuttra is contraindicated.

For a patient on Onpattro, Tegsedi, or Wainua doing well, the switch decision is individualised.

Reserve Meds does not push a default. The page above describes the Amvuttra pathway because Amvuttra is the therapy the patient has asked about.

What Reserve Meds does on this case

We are a US-based concierge coordinator. We are not the prescriber and not the dispensing pharmacy. On an Abu Dhabi Amvuttra case we build the documentation pack with the treating amyloid clinic (most commonly Cleveland Clinic Abu Dhabi or SSMC), confirm DoH and EDE registration status for the specific indication, run the Thiqa or commercial pre-authorisation conversation alongside the clinical workup, coordinate cold-chain supply logistics for ongoing quarterly dispensing, support family-screening genetic-counselling coordination where the family chooses to pursue it, organise self-injection training if the patient prefers home administration, and stay with the case through the first year of dosing with handoff to the local amyloid clinic. Clinical decisions remain with your treating neurologist and cardiologist.

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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