Amvuttra

United Arab Emirates · access guide

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

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

The UAE has built one of the deepest specialty-medicine footprints in the region. Cleveland Clinic Abu Dhabi runs a substantial cardiology amyloid programme with cardiac amyloid imaging (99m-technetium pyrophosphate scintigraphy, cardiac MRI) and a multidisciplinary review pathway. Sheikh Shakhbout Medical City Abu Dhabi, Mediclinic City Hospital Dubai, American Hospital Dubai, Burjeel Medical City Abu Dhabi, and the Dr Sulaiman Al Habib network all carry the neurology and cardiology services needed to diagnose and manage hereditary transthyretin-mediated amyloidosis (hATTR) in adults. Amvuttra (vutrisiran) is Alnylam Pharmaceuticals' GalNAc-conjugated small interfering RNA (siRNA) therapy for hATTR with polyneuropathy and, since the March 2025 label expansion, for ATTR cardiomyopathy in both hereditary and wild-type forms. For a UAE-resident adult with confirmed TTR amyloidosis (whether the presentation is dominantly peripheral neuropathy, dominantly cardiomyopathy, or both), the operational question is no longer whether a TTR-targeted therapy is reachable: the question is which agent fits, where the prescribing amyloid clinic conversation happens, how the quarterly subcutaneous injection routine works, how the genetic-testing and family-screening dimensions are handled, and how the insurance and state-funded coverage conversation runs at the UAE rare-disease price point.

This page explains the 2026 pathway for a UAE-resident patient: who qualifies, where the prescribing neurologist and cardiologist conversation happens, how Amvuttra is dispensed and stored, what the quarterly dosing schedule looks like, what the realistic out-of-pocket exposure band is in AED at the rare-disease price point, the mandatory vitamin A supplementation that goes with the siRNA mechanism, and how the multi-year treatment course fits into a UAE patient's life. It is concierge documentation written for a patient who is already in conversation with a treating amyloid clinic and wants the operational reality laid out plainly.

Why Amvuttra, and why now

Amvuttra is vutrisiran, a 21-nucleotide double-stranded small interfering RNA conjugated to N-acetylgalactosamine (GalNAc). The GalNAc ligand is recognised by the asialoglycoprotein receptor on hepatocytes, which is what gives Amvuttra its hepatic selectivity. Inside the hepatocyte the siRNA is loaded into the RNA-induced silencing complex (RISC) and cleaves TTR mRNA, sustained over months. The result is reduction of circulating serum transthyretin by typically more than 80 percent, which over time slows or partially reverses peripheral nerve and cardiac amyloid deposition.

The FDA approved Amvuttra in June 2022 for hereditary TTR amyloidosis with polyneuropathy (hATTR-PN) in adults, based on the HELIOS-A trial. In March 2025 the FDA approved the cardiomyopathy label expansion (ATTR-CM, both hereditary and wild-type) based on the HELIOS-B trial.

For a UAE patient with progressive sensorimotor polyneuropathy attributed to a confirmed TTR mutation, or with ATTR cardiomyopathy confirmed by PYP scintigraphy and AL exclusion, Amvuttra is the operational pathway to a disease-modifying therapy that is administered four times per year. The quarterly cadence is a meaningful operational simplification compared with Onpattro (patisiran IV every 3 weeks), Tegsedi (inotersen SC weekly), and Wainua (eplontersen SC monthly), and it is the central practical advantage that drives much of the global shift toward Amvuttra in hATTR-PN treatment.

What Amvuttra is, in plain language

Amvuttra is a subcutaneous injection given once every 3 months. There is no infusion centre requirement, no inpatient stay, no IV access needed. The dose is 25 mg delivered as a single prefilled syringe. Administration is at the prescribing amyloid clinic or, after training, at home.

The standard adult dose is 25 mg subcutaneous every 3 months. Injection sites are the abdomen, the thigh, or the upper outer arm. Sites are rotated between quarterly doses to reduce injection-site reactions.

Storage is at 2 to 8 degrees Celsius refrigeration. Before injection the prefilled syringe is brought to room temperature for 30 minutes. The product is not frozen and not shaken.

This is not a short course. Amvuttra is taken indefinitely, for as long as it provides clinical benefit and is tolerated. Response is assessed by serum TTR reduction (target engagement, typically >80 percent reduction within the first months), neurology scoring (modified Neuropathy Impairment Score +7, or mNIS+7, plus Norfolk Quality of Life-Diabetic Neuropathy) for hATTR-PN cases, and cardiology scoring (NT-proBNP, 6-minute walk distance, echocardiographic strain imaging) for ATTR-CM cases.

One non-optional companion to Amvuttra: 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 supplementation 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 a UAE amyloid clinic

For UAE-resident patients, neurology and cardiology amyloid services apply the FDA and EMA criteria with local insurance adaptation:

1. **Confirmed TTR amyloidosis**. For polyneuropathy: TTR gene sequencing confirming a pathogenic variant, plus clinical features of progressive sensorimotor and/or autonomic neuropathy, plus where indicated tissue biopsy with Congo red staining and amyloid typing by immunohistochemistry or mass spectrometry. For cardiomyopathy: TTR sequencing (for hereditary form) or non-biopsy diagnosis using 99m-Tc-PYP scintigraphy with grade 2 or 3 myocardial uptake, plus exclusion of AL amyloidosis. Equivocal cases proceed to endomyocardial biopsy with amyloid typing. 2. **AL amyloidosis exclusion**. Serum free light chains, serum and urine immunofixation electrophoresis. AL exclusion is 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 light-chain results are equivocal. 3. **Genetic counselling** for confirmed hereditary forms. First-degree relatives should be offered TTR sequencing and clinical surveillance. The autosomal dominant inheritance pattern with variable penetrance means that family members may carry the variant without yet having symptoms. 4. **Baseline neurology assessment** (for hATTR-PN cases): mNIS+7 or equivalent, Norfolk QoL-DN, 10-metre walk test, modified Body Mass Index, autonomic testing where indicated. 5. **Baseline cardiology assessment** (for ATTR-CM cases or for hATTR-PN cases with cardiac involvement): NT-proBNP, troponin, echocardiogram with strain imaging, cardiac MRI where available, 99m-Tc-PYP scintigraphy. 6. **Treatment-naive vs switching status**. Patients may be switching from Onpattro (patisiran), Tegsedi (inotersen), Wainua (eplontersen), tafamidis (Vyndaqel/Vyndamax), or acoramidis (Attruby). Each switch has specific washout and overlap considerations that the treating amyloid clinic manages. 7. **Vitamin A baseline and supplementation plan**. Baseline serum vitamin A (retinol) level, ophthalmology referral if symptoms or risk factors for vitamin A deficiency exist. Vitamin A supplementation at recommended daily allowance started at or before first dose, continued lifelong. 8. **Pregnancy planning** for women of childbearing potential. Effective contraception during treatment. No human pregnancy data; animal data suggest teratogenicity from vitamin A depletion. 9. **Renal and hepatic function review**. Standard baseline labs.

A UAE patient should arrive at the amyloid clinic conversation with the available diagnostic documentation: any prior neurology or cardiology workup, prior nerve conduction studies, prior echo and cardiac imaging reports, any prior amyloid biopsy results, family history (multi-generational sensorimotor neuropathy or unexplained cardiomyopathy), and current medications.

The UAE prescribing and supply picture, plainly

Amvuttra availability in the UAE depends on Emirates Drug Establishment (EDE) registration status at the point of prescription and indication. The hATTR-PN indication has had a longer registration runway across MENA than the March 2025 ATTR-CM expansion; the cardiomyopathy indication's current label status in the UAE should be `[VERIFY: ...]` at intake for any ATTR-CM case. Alnylam's MENA commercial footprint runs through specialty distributor partners. The pathway is:

1. **Prescribing neurologist with amyloidosis experience and/or cardiologist with amyloidosis experience**. This is a dual-specialty drug. The UAE centres with established amyloid programmes include Cleveland Clinic Abu Dhabi (deep cardiology amyloid programme with PYP scintigraphy and multidisciplinary amyloid clinic), Sheikh Shakhbout Medical City Abu Dhabi, Mediclinic City Hospital Dubai, American Hospital Dubai, Burjeel Medical City Abu Dhabi, and the Dr Sulaiman Al Habib network. Public-sector amyloid pathways for Emirati nationals run through Cleveland Clinic Abu Dhabi (Mubadala-affiliated) and SSMC, with referral routing through SKMC and DHA hospitals as needed. 2. **Genetic testing infrastructure**. Cleveland Clinic Abu Dhabi, SSMC, and Mediclinic City Hospital run in-house or partnered molecular labs that handle TTR sequencing. Turnaround is typically 4 to 8 weeks. Smaller centres send samples to regional reference labs or to Centogene/Invitae partners. 3. **Cardiac amyloid imaging**. 99m-Tc-PYP scintigraphy is available at Cleveland Clinic Abu Dhabi, SSMC, Mediclinic City Hospital, and at the major nuclear medicine departments across the UAE tertiary network. Cardiac MRI is widely available. 4. **Pharmacy dispensing**. Specialty pharmacy at the prescribing tertiary centre, with cold-chain refrigeration. Quarterly cadence makes stocking straightforward. Where Amvuttra is not yet registered for the specific indication, a named-patient pathway can apply for documented physician-initiated prescriptions referencing FDA or EMA approved indications. 5. **Insurance and state-funded coverage**. For Emirati nationals, Thiqa coverage has extended to rare-disease orphan therapies on a case-by-case basis. Daman and the major commercial insurers (Oman Insurance, AXA Gulf, MetLife, Cigna, Bupa) handle commercial cover with documented medical necessity and prior-authorisation. The rare-disease cost level means the conversation is typically run by the amyloid clinic's pharmacy and case-management team in parallel with the clinical workup. 6. **Self-injection training**. A single supervised session at the prescribing amyloid clinic, or an Alnylam patient-support nurse educator visit. Many UAE patients choose to keep the quarterly injection as a clinic visit rather than self-administer, because the cadence is forgiving and the quarterly clinic visit doubles as a clinical check-in. 7. **Ongoing monitoring**. Amyloid clinic follow-up at 6 months and 12 months for baseline-to-treatment comparison, then annually for stable patients. Serum TTR level at intervals to confirm target engagement. Vitamin A serum level and ophthalmology assessment if symptoms of deficiency develop.

The 2026 pathway, step by step

Week 0 to 4: Diagnostic confirmation. Reserve Meds builds the documentation pack with the treating amyloid clinic. TTR sequencing (if not already done), AL exclusion labs, baseline neurology or cardiology scoring, baseline PYP scintigraphy or other cardiac imaging as appropriate. Family history documentation. If TTR sequencing is the gating step the timeline extends to whatever the lab turnaround requires (typically 4 to 8 weeks).

Week 4 to 8: Insurance and coverage conversation in parallel with the diagnostic workup. For Emirati nationals, Thiqa rare-disease orphan-therapy pathway via the amyloid clinic's case-management team. For non-Emirati residents, commercial pre-authorisation with documented medical necessity, rare-disease coverage rider (where applicable), and the amyloid clinic's clinical recommendation.

Week 8 to 12: First dose dispensing and administration at the prescribing amyloid clinic. Vitamin A supplementation started. Self-injection training if the patient and family prefer home administration for ongoing doses.

Month 3: Second quarterly dose. Reserve Meds coordinates supply logistics for cold-chain delivery if the patient is self-administering at home.

Month 6 to 12: Response assessment at the amyloid clinic. Serum TTR reduction confirmed. Neurology scoring (mNIS+7) or cardiology scoring (NT-proBNP, 6-minute walk) compared to baseline. Vitamin A serum level reviewed.

Month 12 onwards: Maintenance quarterly dosing. Annual amyloid clinic review. Family-screening conversation continues over time as relatives complete or decline TTR sequencing.

Cost expectation in AED

US list price (WAC) for Amvuttra is approximately USD 463,500 per year (USD 116,000 per quarterly dose). MENA pricing varies by country and the rare-disease orphan-therapy framework that applies. Cash-pay retail pricing for Amvuttra in the UAE specialty channel commonly sits in the range of USD 350,000 to 480,000 per year.

At 2026 indicative cross rates, 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 commercial covers vary; the prescribing amyloid clinic's case-management team is the gating step.

For non-Emirati residents whose employer plan or commercial cover does not extend to rare-disease orphan therapy, the cash-pay exposure is the full annual band. Reserve Meds surfaces this reality early in the conversation. 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 production along with TTR, which reduces vitamin A transport in the bloodstream. All patients take oral vitamin A supplementation at the recommended daily allowance (approximately 2,500 to 3,000 IU/day for adults) for the duration of treatment. Without supplementation, vitamin A deficiency manifests slowly over months to years as night vision difficulty, dry eyes, or in extreme cases corneal changes. Patients who report ocular symptoms during treatment are referred for ophthalmology assessment with serum vitamin A measurement.

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

Limb pain and arthralgia have been reported in the pivotal trials at modestly higher rates than placebo. Most cases are mild to moderate and manageable.

Falls have been reported, particularly in patients with autonomic involvement from the underlying polyneuropathy. Fall prevention counselling is part of the standard amyloid clinic follow-up.

Pregnancy. No human data. Animal data suggest teratogenicity from vitamin A depletion in pregnancy. Effective contraception during treatment is required for women of childbearing potential. Discontinuation planning for pregnancy 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. The treatment-related adverse-event profile is favourable compared with the antisense oligonucleotide alternatives (Tegsedi, which has thrombocytopenia and renal toxicity signals).

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. The classical analogy is to other synthetic injectable drugs rather than to vaccines or biologics. If a UAE family requires written halal-certification documentation of the specific commercial product, this can be requested through Alnylam at intake.

The quarterly cadence is a major operational and family-logistics advantage. Travel, work, multi-generational family commitments, Ramadan, and the rhythm of UAE life accommodate a four-times-a-year clinic visit far more easily than a weekly or monthly self-injection. The case-management conversation often hinges on this practical reality.

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 a UAE family carries implications for first-degree relatives, who may be presymptomatic or may have symptoms attributed to other causes. The page does not push specific family-disclosure decisions; the treating amyloid clinic's genetic counselling service is the right home for that conversation, and Reserve Meds supports the patient and family in coordinating sibling and adult-child genetic testing where the family decides to pursue it.

Vitamin A supplementation deserves a separate practical note. Patients and families who would not realistically take a daily oral supplement for years should discuss this frankly with the amyloid clinic at initiation. The supplementation is mandatory and lifelong; treatment without it is not the right course.

When Amvuttra is not the right call

For a UAE patient whose amyloidosis is AL rather than TTR (light-chain amyloidosis from plasma cell dyscrasia), Amvuttra has no role. AL amyloidosis is treated with anti-plasma-cell therapy under haematology care; the diagnostic distinction is the gating safety step before TTR-directed therapy is started.

For a patient with confirmed TTR amyloidosis whose phenotype is milder cardiomyopathy without progressive polyneuropathy, where the operational simplicity of an oral once-daily therapy outweighs the deeper TTR suppression of an RNAi/ASO mechanism, tafamidis (Vyndaqel for hATTR-PN, Vyndamax for ATTR-CM) or acoramidis (Attruby for ATTR-CM) 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 the appropriate choice; tafamidis or acoramidis are operationally simpler and do not carry the vitamin A obligation.

For a pregnant patient or a woman who is planning pregnancy in the near term, Amvuttra is contraindicated until the pregnancy and lactation course is complete; the amyloid clinic manages the discontinuation and re-initiation timing.

For a patient on Onpattro (patisiran), Tegsedi (inotersen), or Wainua (eplontersen) who is doing well, the switch decision is individualised; Amvuttra's quarterly cadence is the operational draw, but the clinical evidence for switching versus staying on the current agent is patient-specific.

Reserve Meds does not push a default. The page above describes the Amvuttra pathway because Amvuttra is the therapy the patient has asked about. If the conversation with the treating amyloid clinic points toward tafamidis, acoramidis, Wainua, Onpattro, or continued symptomatic care, the operational pathway shifts accordingly.

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 a UAE Amvuttra case we build the documentation pack with the treating amyloid clinic, confirm EDE registration status for the specific indication (hATTR-PN or ATTR-CM), run the insurance pre-authorisation conversation alongside the clinical pre-authorisation conversation, coordinate the 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 for ongoing surveillance. 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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