Hemgenix

Abu Dhabi · access guide

How to access Hemgenix from Abu Dhabi, the named-patient coordination pathway, 2026

By Reserve Meds · Clinical & regulatory team · Last reviewed 2026-04-23

A Abu Dhabi adult living with moderately severe to severe Hemophilia B, recurrent bleeds despite routine factor IX prophylaxis, may be evaluated by their treating haematologist for Hemgenix (etranacogene dezaparvovec). Hemgenix is FDA-approved, developed by CSL Behring and uniQure, and is a one-time intravenous adeno-associated virus (AAV5) gene therapy that delivers a functional factor IX transgene to the liver. Because Hemgenix is a single-infusion gene therapy with strict eligibility gating and a multi-year monitoring commitment, access for Abu Dhabi patients involves cross-border referral to a gene-therapy-qualified treatment centre rather than a routine drug import.

This guide explains the legal and operational pathway, the eligibility gating, what your haematologist needs to coordinate, indicative timing and cost bands, and where Reserve Meds fits in.

The clinical situation

Hemgenix is delivered as a single IV infusion over one to two hours at a qualified gene-therapy centre. Eligibility is tightly gated: patients are screened for pre-existing neutralising antibodies against the AAV5 capsid (AAV5-negative titre is typically required), liver health (ALT/AST within bounds, no active HBV or HCV, no advanced fibrosis on elastography), and a factor-IX-inhibitor-free history. Post-infusion, patients are monitored closely for transaminitis and generally receive a tapering oral corticosteroid course if liver enzyme rises occur. Long-term surveillance for factor IX activity, liver function, and AAV-related safety signals continues for at least five years per manufacturer and regulatory guidance.

Is Hemgenix legally accessible for Abu Dhabi patients?

Hemgenix cannot be "imported" as a conventional drug for local infusion, it is a single-vial one-time product requiring administration at a gene-therapy-qualified centre with the infrastructure to manage pre-infusion screening, infusion monitoring, and the multi-year follow-up protocol. Access for Abu Dhabi patients typically follows cross-border referral within the UAE Ministry of Health and Prevention (MoHAP) and Ministry of Health medical-referral framework:

Pattern A, Cross-border referral to an authorised gene-therapy centre. The patient travels to a qualified international centre (in the US, Europe, or a select Middle-East tertiary centre operating under the manufacturer's qualified-treatment-centre model) for eligibility workup, infusion, and early post-infusion monitoring. Long-term surveillance is then handed back to Abu Dhabi haematologist.

Pattern B, Hybrid coordination with a Abu Dhabi tertiary centre. Where a Abu Dhabi centre participates in CSL Behring's qualified-treatment-centre network, portions of screening and long-term follow-up may be localised.

The MoHAP and MoH medical-referral frameworks support both patterns, with documentation covering medical necessity, treatment-centre identification, and the return-to-home surveillance plan.

How the pathway works, step by step

1. **Consultation with your haematologist.** Bleed history, prior factor IX regimen, inhibitor history, and liver status are reviewed.
2. **Eligibility workup.** AAV5 capsid antibody titre, factor IX inhibitor assay, hepatic panel with elastography, and hepatitis serology.
3. **Treatment-centre identification.** Reserve Meds coordinates referral to a qualified gene-therapy centre.
4. **MoHAP / MoH medical-referral dossier.** Filed by your physician with supporting clinical rationale.
5. **Cross-border travel and infusion.** Single IV infusion at the qualified centre; inpatient observation and early outpatient monitoring.
6. **Handover to Abu Dhabi haematology team.** Structured multi-year surveillance plan covering factor IX activity, LFTs, and AAV safety parameters.

What documentation your physician needs

Your physician will typically need to provide:

- Clinical rationale letter with diagnosis, bleed phenotype, prior prophylaxis, inhibitor history, and Hemgenix as the indicated therapy
- Verification of their the DoH medical licence (Abu Dhabi emirate) (SCFHS / MOH)
- AAV5 capsid antibody, factor IX inhibitor, and hepatic workup results
- Identification of the qualified treatment centre and the referral plan
- Long-term surveillance plan for return to the Abu Dhabi

Reserve Meds provides a coordination kit bundling the templates MoHAP reviewers and qualified treatment centres expect to see for cross-border gene-therapy referrals.

Costs and timing

Hemgenix's US list price for the one-time product is indicatively around USD 3.5 million; total cost of care, including pre-infusion workup, infusion-day services, inpatient/outpatient monitoring, and travel for a family caregiver, runs materially higher when delivered at a US qualified centre. Reserve Meds issues a transparent all-in quote at intake. Because this is a one-time therapy rather than a recurring prophylaxis spend, families often work with their haematologist and financial advisor to model the cost against a lifetime of factor IX prophylaxis.

Indicative timing from intake to infusion typically runs 10-20 weeks, driven by eligibility workup, treatment-centre calendar, and travel coordination. Early post-infusion monitoring typically requires several weeks at or near the qualified centre.

Fulfillment availability is limited to our first cohort, and all timelines published on this site are indicative. If your clinical situation is time-sensitive, tell us at intake. We triage accordingly.

A culturally-aware note: gene-therapy journeys typically require a family caregiver throughout the extended stay; our coordination includes caregiver travel, prayer-space orientation, and halal-dining support at partnering centres.

Reserve Meds's role

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine and gene therapy. For Hemgenix specifically, we provide:

- **Treatment-centre referral.** Coordination with authorised Hemgenix qualified-treatment centres.
- **Documentation.** Cross-border referral and MoHAP/MoH named-patient package.
- **Logistics.** Patient and caregiver travel, accommodation, and post-infusion return-home planning.
- **Concierge case lead.** A named point of contact throughout the gene-therapy journey.

What we do not do: we are not the prescriber, we do not practise medicine, we do not manufacture the product, and we are not the treatment centre. All clinical decisions remain with your treating haematologist and the qualified centre.

Frequently asked

Is this legal in Abu Dhabi? Yes, when executed through the MoHAP and MoH medical-referral framework with a qualified treatment centre. See our trust and compliance page.

What if my AAV5 antibody titre is positive? Pre-existing AAV5 neutralising antibodies above the manufacturer's threshold typically exclude a patient from Hemgenix. Your haematologist will review options with you.

Will I still need factor IX prophylaxis afterward? Clinical trial data show many patients experience durable factor IX expression with reduced or discontinued prophylaxis. Your haematologist manages the taper decision based on serial factor IX activity measurements.

What about long-term safety? Multi-year surveillance is a core part of the protocol, including liver function monitoring. Your haematology team will explain the full profile.

Will MoH coverage apply? Some Abu Dhabi patients receive partial coverage for complex international referrals; we supply documentation for submission but do not process public-payer claims directly.

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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