

## Hemgenix

Kuwait · access guide

# How to access Hemgenix from Kuwait, the named-patient coordination pathway, 2026

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

A Kuwait 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 Kuwait 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 Kuwait 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 Kuwait patients typically follows cross-border referral within the Kuwait Ministry of Health (KMOH) 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 Kuwait haematologist.

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

The KMOH 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. **KMOH / 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 Kuwait 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 Kuwait medical licence (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 Kuwait

Reserve Meds provides a coordination kit bundling the templates KMOH 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.

## **Why Hemgenix cases out of Kuwait have a longer pre-administration runway than other specialty therapies**

---

Hemgenix is a single-dose AAV5-based gene therapy for adult Hemophilia B (FIX activity at or below 2%) administered as a 60-minute IV infusion. The drug itself is simple to deliver. The operational gating around it is exceptional. Administration must happen at a center with hepatology integration (transaminase monitoring for a year or more), infectious-disease consultation if HIV, HBV, or HCV coinfection is present, and immediate access to immunosuppressive escalation if liver enzyme elevation triggers the management protocol.

In Kuwait, this concentrates eligible centers to Mubarak Al-Kabeer Hospital, Amiri Hospital, and the Hematology Department at the Kuwait Cancer Control Center. The Kuwait Drug and Food Control (KDFC) at MoH reviews gene-therapy NPP submissions on an extended timeline (8 to 16 weeks) given the single-administration, irreversible nature of the therapy.

Cases that move forward require: confirmed FIX:C at or below 2%, age 18 years or older, no pre-existing anti-AAV5 neutralizing antibodies (testing typically done at the manufacturer's reference lab), no active hepatic disease, and documented prior FIX therapy history. The anti-AAV5 testing in particular cannot be skipped, a positive result disqualifies the patient and there is no alternative AAV serotype currently on label.

Funding is the binding constraint for almost every case. Hemgenix at international list cost requires significant family resources, employer support, or special-case state coverage. We have walked families through the all-in figure and the post-administration monitoring requirements before any application is filed, the worst outcome would be advancing a case where downstream funding evaporates. Cold-chain to Kuwait works through validated specialty couriers with onsite handoff at the administering hospital pharmacy; we coordinate the receipt-to-thaw-to-administration timing closely with the treating haematologist.

### **More questions, specific to this case**

#### **Does the patient need to travel out of Kuwait to receive Hemgenix?**

Not necessarily. If a Kuwait center qualifies operationally, administration is local. Some families do choose to travel for the post-administration monitoring infrastructure available in a specific reference center elsewhere.

#### **What is the post-administration monitoring schedule?**

Weekly liver enzymes for the first 3 months, then less frequent for a total of at least 12 months. FIX activity is monitored regularly to assess durability of expression.

#### **Is a second dose ever given if response is suboptimal?**

No. AAV-based gene therapy generates anti-AAV antibodies that prevent re-dosing. This is part of the pre-treatment counselling.

### ***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.

---

### **Reserve Meds**

*reserved for you.*

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.

reservemeds.com · hello@reservemeds.com