Hemgenix in Saudi Arabia

The SFDA cross-border gene-therapy coordination pathway — indicative 2026

HEMATOLOGY / GENE THERAPY · HEMOPHILIA B

The clinical situation

Hemgenix (etranacogene dezaparvovec) is a one-time intravenous AAV5 gene therapy delivering a functional factor IX transgene to the liver, FDA-approved for adults with moderately severe to severe Hemophilia B. Developed by CSL Behring and uniQure. Eligibility is tightly gated: AAV5-neutralising-antibody-negative titre, factor-IX-inhibitor-free history, liver health (ALT/AST, no active HBV/HCV, no advanced fibrosis on elastography). Post-infusion patients are monitored for transaminitis — typically with a tapering oral corticosteroid course — and long-term surveillance continues for at least five years per manufacturer and regulatory guidance.

The pathway, 6 steps

1. **Consultation with your haematologist.** Bleed history, prior factor IX prophylaxis, inhibitor history, and liver status reviewed.
2. **Eligibility workup.** AAV5 capsid antibody titre, factor IX inhibitor assay, hepatic panel with elastography, hepatitis serology.
3. **Treatment-centre identification.** Reserve Meds coordinates referral to a qualified gene-therapy centre (US, Europe, or partnering ME tertiary centre operating under CSL Behring's QTC model).
4. **SFDA / MoH medical-referral dossier.** Filed by your physician with supporting clinical rationale.
5. **Cross-border travel & single infusion.** One IV infusion over 1–2 hours at the qualified centre; inpatient observation and early outpatient monitoring follow.
6. **Handover to Saudi haematology team.** Structured multi-year surveillance plan for factor IX activity, LFTs, and AAV safety parameters.

Indicative economics

Reference US list price: ~USD 3.5M one-time product; total cost of care (workup, infusion, monitoring, caregiver travel) runs materially higher. Reserve Meds issues a transparent all-in quote at intake.

Indicative first-infusion timing

10–20 weeks from intake to infusion, driven by eligibility workup, treatment-centre calendar, and travel coordination. Indicative — not guaranteed.

Reserve Meds's role

Coordination. Referral to qualified gene-therapy treatment centre aligned with CSL Behring's QTC model. **Documentation.** Coordination kit for your haematologist and SFDA / MoH medical-referral review, keyed to the Hemgenix gene-therapy rationale. **Logistics.** Cross-border travel coordination including caregiver support and accommodation guidance. **Concierge**

case lead. Named point of contact for family and care team across the multi-year surveillance horizon. **We are a coordinator** — not the prescriber, not the treatment centre. All clinical decisions remain with your treating haematologist and the QTC team.

COMPOSITE EXAMPLE · PRE-LAUNCH WAITLIST

Join the Hemgenix × Saudi Arabia first-cohort waitlist. *Our concierge reaches out as we open intake.*

reservemeds.com/access-guides/hemgenix-saudi-arabia.html

SCAN TO JOIN

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