Hemgenix access in UAE: certified-center coordination for hemophilia B gene therapy

How patients in the United Arab Emirates with hemophilia B access Hemgenix (etranacogene dezaparvovec). Hemgenix is a single one-time AAV5 gene therapy administered only at manufacturer-certified treatment centers; for UAE patients the practical path is travel to a qualified center after AAV5 antibody pre-testing.

Last reviewed 2026-05-12 by Reserve Meds clinical and regulatory team. Hemgenix is FDA-approved for adults with hemophilia B (congenital factor IX deficiency) meeting label criteria. Hemgenix is not registered in the UAE and cannot be administered outside a CSL Behring certified treatment center. UAE patients access it through travel-for-treatment, not through a conventional shipment into the country.

Quick orientation for UAE patients

Hemgenix, the brand name for etranacogene dezaparvovec-drlb, is an adeno-associated virus serotype 5 (AAV5) based in vivo gene therapy that delivers a codon-optimized Padua variant of the human factor IX gene to hepatocytes. After a single one-time intravenous infusion, liver cells continuously express factor IX-Padua, a naturally occurring gain-of-function variant of human factor IX, with the goal of eliminating or substantially reducing the need for prophylactic factor IX replacement. The U.S. Food and Drug Administration approved Hemgenix on 22 November 2022. It was the first FDA-approved gene therapy for hemophilia, the first liver-directed AAV vector therapy approved by FDA, and at launch the highest-priced FDA-approved therapy in history. Hemgenix is developed by CSL Behring under a global license from uniQure. The drug is not registered in the UAE, and it cannot be administered outside a CSL Behring certified treatment center. For a UAE-resident patient, the realistic path is travel to a certified hemophilia treatment center in the US, EU, UK, or Canada after AAV5 neutralizing antibody pre-testing confirms candidacy. Reserve Meds coordinates the patient-side travel, the certified-center handoff, and family logistics around a single one-time infusion event with multi-year follow-up. Reserved for you.

Why UAE patients reach for Hemgenix via cross-border coordination

For an adult UAE patient with hemophilia B who is on factor IX prophylaxis, has current or historical life-threatening hemorrhage, or has experienced repeated serious spontaneous bleeding episodes, and whose treating hematologist has identified gene therapy as the next step, the access problem is not the molecule but the infrastructure. Three constraints drive the cross-border path. First, Hemgenix is not registered with the EDE or any GCC national regulator. Second, administration is gated by certified-center capability that does not exist in the UAE today. Third, the USD 3.5 million list price for a one-time infusion sits well outside any UAE

national-payer construct, and even where local registration exists in other jurisdictions, payer coverage of one-time multimillion-dollar therapies is uneven globally.

UAE hematology centers (Sheikh Khalifa Medical City, Cleveland Clinic Abu Dhabi, Tawam Hospital, American Hospital Dubai, Mediclinic City Hospital, King's College Hospital London Dubai) handle the diagnostic confirmation, baseline workup, post-infusion follow-up, and long-term coordination, but none holds CSL Behring certified treatment center status for Hemgenix as of this review. The federal MOHAP and EDE unregistered-medicine framework, administered through the EDE portal at ede.gov.ae from 29 December 2025, is the import-permit mechanism but it does not bridge the certified-center constraint. The bridge is travel to a CSL-certified center that has imported a single dose against the patient's identity, paired with the patient's UAE-based clinical team for pre-screening and long-term follow-up.

AAV5 neutralizing antibody pre-testing: the gate to candidacy

The Hemgenix prescribing information mandates AAV5 neutralizing antibody titer testing before treatment to support patient selection per the current label. AAV5 antibodies, where present, can neutralize the AAV5 vector before it transduces hepatocytes, blunting or eliminating the therapeutic effect. The antibody pre-test is the single most important pre-treatment gate after the diagnosis itself. UAE patients pursuing Hemgenix typically have the AAV5 antibody titer drawn either at their treating UAE hematology center (with the sample shipped to a reference lab capable of the assay) or at the destination certified center as part of the pre-treatment workup. Where the titer is above the candidacy threshold per the current label, Hemgenix is not appropriate and the treating hematologist returns to factor IX prophylaxis or considers an alternative AAV serotype gene therapy such as fidanacogene elaparvovec (Beqvez, Pfizer, FDA-approved April 2024) which uses a different vector. The AAV5 antibody titer is therefore the first concrete data point in a UAE Hemgenix evaluation.

Alongside the AAV5 titer, the prescribing information requires baseline factor IX activity confirmation, liver function tests (ALT, AST, alkaline phosphatase, bilirubin), hepatitis B and hepatitis C screening, and assessment for active liver disease or significant hepatic fibrosis. Patients with active or untreated viral hepatitis, advanced fibrosis, or other significant liver pathology require evaluation before consideration. These workups are typically completed in the UAE under the treating hematologist before the family commits to certified-center travel.

The corticosteroid algorithm for transaminitis

Hemgenix carries a known post-infusion safety pattern. Transaminase rises (typically ALT elevations) are expected in a meaningful fraction of patients in the weeks following infusion. The label specifies a corticosteroid algorithm: a tapering oral corticosteroid course is initiated when ALT rises above protocol thresholds, with monitoring through resolution. This is not an adverse event in the optional sense; it is a planned management algorithm that runs at the certified center under the prescribing hemophilia specialist's authority. Long-term follow-up through 5 years has not identified AAV-related oncogenicity or chronic hepatotoxicity in reported data, but transaminase monitoring is the central post-infusion safety surveillance lever.

For a UAE patient, the practical implication is that the immediate post-infusion window (weeks 1 through approximately 12) is best handled at or in coordination with the certified center because that center holds the corticosteroid algorithm protocol, the laboratory cadence, and the experience of running it. The UAE-side treating hematologist takes over for the longer arc once the transaminitis window has resolved.

The MOHAP and EDE framework versus the Hemgenix reality

The federal unregistered-medicine import perm