Hemgenix access from Pakistan

How patients in Pakistan reach Hemgenix (etranacogene dezaparvovec-drlb) for hemophilia B through travel-to-treatment at certified gene therapy centers abroad.

Quick orientation

Hemgenix is the brand name for etranacogene dezaparvovec-drlb, an adeno-associated virus serotype 5 (AAV5) based gene therapy that delivers a codon-optimized Padua variant of the human factor IX gene to hepatocytes through a single one-time intravenous infusion. The U.S. FDA approved Hemgenix on November 22, 2022 for hemophilia B (congenital factor IX deficiency) in adults who currently use factor IX prophylaxis therapy, who have current or historical life-threatening hemorrhage, or who have experienced repeated, serious spontaneous bleeding episodes. It is the first FDA-approved gene therapy for hemophilia and was launched at approximately USD 3.5 million per patient, making it the highest-priced FDA-approved therapy at the time of launch. Pakistan does not have a certified Hemgenix treatment center. The pre-infusion AAV5 neutralizing antibody titer test (mandatory per the FDA label), the liver workup, the infusion itself, and the structured post-infusion transaminitis monitoring with the label-specified corticosteroid algorithm all require an infrastructure that is not currently operational in Pakistan. Reaching Hemgenix for a Pakistani patient is a travel-to-treatment case to a certified center in the U.S., EU, UK, or Canada. Reserve Meds is the upstream coordinator. The certified-center hemophilia specialist remains the clinical authority. Reserved for you.

Why hemophilia B patients in Pakistan need Hemgenix via travel-to-treatment

Three structural constraints converge to make Hemgenix a travel-to-treatment case rather than a DRAP named-patient import. First, Hemgenix is administered only at CSL Behring certified treatment centers (CTCs) that have completed site qualification, staff training, and infusion-readiness validation; no Pakistani institution currently holds CTC status. Second, the AAV vector is a regulated genetically modified organism (GMO) in many jurisdictions, including Pakistan, which adds a regulatory layer beyond the standard DRAP Special Permission framework that the framework was not designed for. Third, the cost envelope at USD 3.5 million for the medicine alone is prohibitive for any payer-financed model in Pakistan; Sehat Sahulat's Rs. 1,000,000 per family per year ceiling does not begin to approach the figure, and no Pakistani private insurer covers gene therapy at this price tier. The practical path for a Pakistani hemophilia B patient whose treating hematologist has set Hemgenix as the plan is travel to a certified center, with AAV5 antibody screening, liver workup, infusion, and post-infusion monitoring all performed in the destination country.

For Pakistani families, the destination question is shaped by visa accessibility, language, family network, and clinical referral pathway. Families with relatives in the U.S., the UK, Canada, or Germany often route to one of those countries because the family network supports the multi-month commitment. The clinical authority sits with the destination hemophilia specialist at the certified center, not with Reserve Meds and not with the Pakistani referring hematologist. The Pakistan-side care continuum (AKUH, Indus, Shaukat Khanum, the Hemophilia Patients Welfare

Society Pakistan support network) handles the long-term follow-up in coordination with the certified center.

How Hemgenix access actually works from Pakistan

There is no DRAP filing for Hemgenix because there is no Hemgenix import into Pakistan. The medicine is a per-patient dose-pack manufactured against the patient's confirmed candidacy and shipped from CSL Behring's specialty logistics chain directly to a CSL-certified hemophilia treatment center in a country with regulatory authorization. The operational structure is therefore: referral acceptance at a candidate certified center abroad, visa support for the patient and a caregiver, pre-treatment workup at the destination including AAV5 neutralizing antibody titer testing (which is mandatory per the FDA label, and patients with pre-existing AAV5 antibodies above the threshold may not be eligible), baseline factor IX activity confirmation, liver function tests including ALT, AST, alkaline phosphatase, and bilirubin, screening for hepatitis B and hepatitis C, 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; some are ruled out at this stage.

If the patient is eligible after the AAV5 antibody titer and liver workup, the destination certified center coordinates the per-patient dose-pack with CSL Behring, the patient receives the single weight-based intravenous infusion at 2 x 10 to the 13 genome copies per kilogram, and structured post-infusion monitoring begins. Transaminases are checked at defined intervals, with an expected rise in ALT in a meaningful fraction of patients. The label specifies a tapering oral corticosteroid course for ALT elevations above protocol thresholds. Factor IX activity is measured serially to track endogenous expression. The destination certified center handles all of this. Post-infusion the patient returns to Pakistan with a structured handoff to the referring hematologist for long-term monitoring, which extends for years and feeds back to the certified center for durability follow-up.

The HOPE-B phase 3 trial reported a 64 percent reduction in annualized bleeding rate during months seven through eighteen versus the lead-in period, with 96 percent of participants free from continuous prophylaxis at 18 months. These are the patient-counseling reference points; Reserve Meds does not promise specific clinical outcomes.

Where Hemgenix gets administered (destination certified centers)

Hemgenix is administered only at CSL Behring certified treatment centers (CTCs) that have completed site qualification, staff training, and infusion-readiness validation. In the U.S., these are typically major academic hemophilia treatment centers within or associated with NCI-designated cancer centers or large academic medical systems. EMA granted conditional marketing authorization for Hemgenix in the EU in February 2023, the UK MHRA issued separate authorization, and Health Canada has authorized Hemgenix; certified centers exist across multiple EU member states, the UK, and Canada under CSL Behring's country-specific rollout. For the Reserve Meds Pakistani patient archetype, the destination shortlist typically narrows by visa accessibility, language, and family network. Families with U.S. relatives often route to a U.S. CTC. Families with UK or EU relatives often route to a UK or German CTC. Reserve Meds verifies certified-center status with CSL Behring's published or institution-confirmed network at the point of each quote.

Reserve Meds does not certify centers, does not infuse Hemgenix, and does not select destination on clinical grounds. The destination decision is the treating hematologist's, with Reserve Meds

providing the operational orientation around AAV5 antibody pre-test access (which is sometimes performed in Pakistan at a major center, sometimes performed at the destination), visa, travel logistics, and cost envelope at each candidate destination.

Real cost picture for Hemgenix from Pakistan

The U.S. wholesale acquisition cost for Hemgenix is approximately USD 3.5 million per patient for the single one-time infusion, set by CSL Behring at launch in late 2022 and reported by Fierce Pharma, MM and M, Managed Healthcare Executive, Hemophilia News Today, and other industry sources. CSL Behring's pricing rationale references the one-time nature of the therapy, the projected reduction in lifetime prophylaxis cost (which the company cites as exceeding USD 20 million per patient with moderate-to-severe hemophilia B), and the elimination or reduction of bleed-related morbidity. The Institute for Clinical and Economic Review (ICER) placed a fair price range at approximately USD 2.9 million, below the launched WAC. International list prices vary by jurisdiction and are commonly negotiated confidentially with national payers.

For a Pakistani family on a self-pay path, the cost stack has multiple categories. The drug itself dominates at approximately USD 3.5 million. The certified-center clinical charges (pre-infusion workup including AAV5 antibody titer and liver workup, the infusion itself, the structured post-infusion monitoring window, the corticosteroid algorithm management) add a destination-country institutional fee typically in the USD 100,000 to USD 300,000 range depending on country and length of stay. Travel for patient and one caregiver, accommodation for the workup and monitoring window, ground transport, and meals add a destination-country living cost layer. Reserve Meds adds a transparent coordination fee per quote, shown separately rather than embedded in the drug price.

Currency context matters even more in Pakistan than for the lower-priced cells. The PKR is in the 278 to 280 range to the USD as of May 2026, with April 2026 CPI inflation at 10.9 percent. At PKR 280 to the USD, the drug alone translates to roughly PKR 980 million; no Pakistani family operates at this scale in PKR terms. The Pakistani diaspora funding pattern is essentially the only realistic funding model for Hemgenix at the typical cash-pay tier. Pakistan received roughly USD 4.4 billion in remittances from Saudi Arabia, USD 3.1 billion from the UAE, and USD 2.7 billion from the UK in recent reporting periods. Families pursuing Hemgenix typically pool resources across many relatives in the Gulf, the UK, the U.S., and Canada, and the multi-country funding coordination is the single biggest operational lift for the family before the destination institution's deposit can clear. Pakistani health plans do not cover gene therapy at this price tier. Sehat Sahulat does not stretch.

Typical timeline for Hemgenix from Pakistan

The end-to-end timeline from first inquiry to return-home post-infusion typically runs four to nine months for a Pakistani patient. Reserve Meds intake and destination shortlist orientation typically runs three to seven days. AAV5 antibody titer testing (where performed in Pakistan) adds two to four weeks; where performed at the destination, it is part of the pre-infusion workup. Referral acceptance and AAV5-eligibility confirmation at a candidate destination center runs four to eight weeks. Visa coordination runs two to twelve weeks depending on destination. Patient travel and pre-infusion workup at the destination run two to four weeks. Infusion is a single intravenous administration over a defined time interval with patient monitoring throughout. Post-infusion monitoring at the destination runs at least eight to twelve weeks given the corticosteroid algorithm for transaminitis and serial factor IX activity tracking. Total destination-country

residence typically runs three to four months. Return home and long-term durability follow-up at the Pakistani referring center extend over years.

What your physician needs to provide

The Pakistani referring hematologist's role is the referral package for the destination certified center. This typically includes the hemophilia B diagnosis with baseline factor IX activity, the bleeding history (annualized bleeding rate where measured, joint involvement, history of life-threatening or repeated serious spontaneous bleeding episodes), the current prophylaxis regimen including specific factor IX product and dosing, liver health status with available LFT results and any history of hepatitis B or hepatitis C, AAV5 antibody titer if locally performed, and the rationale for gene therapy over continued prophylaxis. The destination certified center conducts its own confirmatory pre-infusion workup including the mandatory AAV5 neutralizing antibody titer, baseline factor IX activity, LFTs, hepatitis screening, and assessment for active liver disease or significant hepatic fibrosis.

The Pakistani referring hematologist's PMDC license is verified by the destination institution as part of the referral acceptance. The Pakistani prescriber is not the infusing physician; the destination certified center's hemophilia specialist is. Post-infusion handoff back to the Pakistani referring hematologist is structured by the certified center, and durability follow-up reports flow in both directions.

Common questions about Hemgenix from Pakistan

Can Hemgenix be administered in Pakistan? Not currently. The CSL Behring certified-center framework, the AAV5 antibody titer infrastructure (which exists at limited Pakistani centers), the post-infusion corticosteroid algorithm management, and the multi-year durability follow-up framework all sit at certified centers abroad. The path is travel-to-treatment.

What if my AAV5 antibody titer is too high? Patients with pre-existing AAV5 neutralizing antibodies above the threshold defined in the FDA label may not be eligible for Hemgenix. The titer test is mandatory and is part of the pre-infusion workup. If the titer rules out Hemgenix, alternatives such as fidanacogene elaparvovec (Beqvez, Pfizer, FDA approved April 2024) or continued lifelong factor IX prophylaxis are clinical conversations for the treating specialist; Reserve Meds does not recommend among them.

Will Adamjee, Jubilee, EFU, or State Life cover this? Pakistani health plans do not cover gene therapy at the USD 3.5 million price tier. Cash-pay through pooled diaspora resources is the practical funding model.

Our family pools funds across the Gulf, the UK, and North America. How does Reserve Meds handle that? For Hemgenix, multi-country funding coordination is the operating norm and is typically the largest single operational lift for the family before the destination institution's deposit can clear. Reserve Meds quotes in USD, accepts wire transfers from any USD-accessible source, and coordinates timing against the destination institution's deposit deadline. The pricing transparency on this page lets the family plan funding before contacting us, because destination acceptance and visa coordination cannot start until the cost envelope is socialized within the family.

What about safety risks? Most common adverse reactions in clinical development include hepatic transaminase elevations, headache, flu-like symptoms, infusion-related reactions, creatine kinase elevation, malaise, and fatigue. Transaminase rises are expected in a meaningful

fraction of patients and are managed with the label-specified corticosteroid algorithm at the destination certified center. Long-term follow-up through five years has not identified AAV-related oncogenicity or chronic hepatotoxicity in reported data. The destination hemophilia specialist handles all clinical aspects.

Is Hemgenix a controlled substance? No. Hemgenix is a gene therapy and is not on any DEA schedule. The travel-to-treatment pathway is the operational reality, not a DRAP Special Permission filing.

Where Reserve Meds fits in Hemgenix cases

Reserve Meds is the upstream coordinator for travel-to-treatment. For a Hemgenix case from Pakistan, Reserve Meds confirms eligibility and case fit within 24 to 48 hours of intake, orients the family on the AAV5 antibody pre-test, the certified-center framework, the multi-month destination residence, and the long-term durability follow-up arc, supports the referral package preparation, coordinates with the destination certified center on acceptance timing, and assigns a single named Concierge Patient Coordinator with Urdu- and English-language support who stays with the family throughout the four- to nine-month travel-to-treatment arc and the years of post-infusion follow-up coordination. Reserve Meds does not administer Hemgenix, does not certify centers, does not perform the AAV5 antibody titer, does not advise on Hemgenix versus Beqvez versus continued factor IX prophylaxis, and does not act as a clinical decision-maker. The clinical authority sits with the destination hemophilia specialist at the certified center. The Pakistani referring hematologist remains the long-term follow-up partner after return home.

Next step

If your treating hematologist has set Hemgenix as the plan, the waitlist is the entry point. Reserve Meds responds within 24 to 48 hours with a destination shortlist orientation and an indicative cost envelope by destination. The destination-specific quote follows after a candidate certified center accepts the referral and the AAV5 antibody titer confirms eligibility.

Reserved for you.