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Hemgenix access in India: travel-for-treatment under the CDSCO framework

How Indian families with hemophilia B orient to Hemgenix (etranacogene dezaparvovec-drlb), the AAV5 gene therapy from CSL Behring, when they have made the clinical decision to pursue a one-time gene therapy over lifelong factor IX prophylaxis, with AAV5 antibody pre-testing and certified-centre administration built into the case plan.

Last reviewed 2026-05-12 by Reserve Meds clinical and regulatory team.

Quick orientation

Hemgenix (etranacogene dezaparvovec-drlb) is an adeno-associated virus serotype 5 (AAV5) based gene therapy from CSL Behring that delivers a codon-optimized Padua variant of the human factor IX gene to hepatocytes, eliminating or reducing the need for prophylactic factor IX replacement after a single one-time intravenous infusion. The US Food and Drug Administration approved Hemgenix on 22 November 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. The EMA granted conditional marketing authorization in February 2023. Hemgenix is not registered with the CDSCO as of this review, and India does not host a CSL Behring-certified Hemgenix infusion centre. For Indian patients with hemophilia B who have made the clinical decision to pursue gene therapy, the realistic access route is cross-border travel-for-treatment to a CSL-certified hemophilia treatment centre abroad, with the CDSCO Rule 36 framework supporting ancillary medicine flows but not the gene therapy administration itself.

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Why patients in India need Hemgenix via cross-border coordination

India has a substantial hemophilia patient population identified through state Hemophilia Society chapters and through comprehensive hemophilia care centres at AIIMS New Delhi, Christian Medical College Vellore (which holds the largest hemophilia comprehensive care registry in South Asia), Tata Memorial Centre Mumbai, Apollo Hospitals, Kokilaben, Fortis, and Manipal. Within that population, hemophilia B (factor IX deficiency) is the less common of the two principal congenital coagulation factor deficiencies but still represents a meaningful cohort of patients on lifelong prophylactic factor IX replacement therapy. The standing alternative to Hemgenix is continued lifelong prophylactic factor IX replacement (recombinant or plasma-derived factor IX, including extended half-life products), which is available locally in India and is the operating standard of care.

The clinical reason families consider gene therapy is the one-time-intervention profile rather than indefinite recurring infusions. The HOPE-B phase 3 trial reported a 64 percent reduction in annualized bleeding rate during months 7 through 18 versus the lead-in period, with 96 percent of participants free from continuous prophylaxis at 18 months. For a patient with high prophylaxis burden, difficult venous access, adherence challenges, or a strong preference for a

one-time intervention over ongoing infusions, the clinical case for gene therapy can be material. The treating hematologist makes the comparator decision. A second hemophilia B gene therapy, fidanacogene elaparvovec (Beqvez), was subsequently FDA-approved in April 2024 by Pfizer; the choice between Hemgenix and Beqvez sits with the treating specialist.

Hemgenix is not on the CDSCO register. Even if it were, the certified-centre administration model and the AAV vector classification as a genetically modified organism (GMO) in many regulatory frameworks mean that Hemgenix cannot be shipped to and administered at a non-certified Indian site under a routine personal-import permit. The realistic path is travel to a CSL-certified centre in the US, EU, or UK. The patient travels with a hemophilia-treatment-centre referral from the treating Indian hematologist, completes the pre-treatment AAV5 neutralizing antibody titre testing (mandatory under the label), undergoes liver function and hepatitis B and C screening, receives the single infusion at the certified centre, completes the immediate post-infusion monitoring including the label-specified corticosteroid algorithm for transaminitis, and returns home for long-term follow-up coordinated between the destination centre and the treating Indian hematologist.

The CDSCO Rule 36 framework for ancillary medicine in Hemgenix cases

The legal foundation for personal import of an unregistered medicine into India is Rule 36 of the Drugs and Cosmetics Rules 1945. Rule 36 permits the import of a small quantity of a drug, whose import would otherwise be prohibited under Section 10 of the Drugs and Cosmetics Act 1940, for the exclusive personal use of a named patient. Form 12A is the application for the permit. Form 12B is the permit itself, issued by the office of the DCGI at FDA Bhawan, Kotla Road, New Delhi, or by designated CDSCO Port Offices. The application is accompanied by a prescription from a Registered Medical Practitioner (RMP) showing the RMP's National Medical Commission (NMC) registration number and the quantity required for treatment.

For Hemgenix specifically, Rule 36 is not the primary access mechanism because the gene therapy is administered abroad and is not shipped to India. The framework matters at the margins: any ancillary medicines that the patient brings home from the destination centre (oral corticosteroid courses for the label-specified transaminitis algorithm if not already locally available in the appropriate formulation; specific hepatic-supportive medicines as needed) may route through Rule 36 if they are unregistered locally and the treating Indian hematologist's NMC-registered prescription supports the import. For institutional Compassionate Use of drugs not approved for marketing in India at all, the parallel pathway is the Compassionate Use application route to the DCGI by a government hospital, a registered medical practitioner, a pharmaceutical company, or the patient, used when the drug is approved by a recognised reference authority (FDA, EMA, MHRA, Health Canada, PMDA) for an unmet medical need. Government institutions including AIIMS New Delhi have established Compassionate Use workflow for rare-disease cases.

The Reserve Meds operational focus in a Hemgenix case is upstream patient and family orientation rather than CDSCO filings: the AAV5 antibody pre-testing requirement (a meaningful fraction of adult hemophilia B patients globally are AAV5-antibody positive and therefore not candidates), the liver workup, the destination-centre referral, the cost envelope at the USD 3.5 million price tier, and the travel and stay logistics around the infusion and the immediate post-infusion monitoring window.

Where Hemgenix gets administered for Indian patients

Hemgenix is shipped only to CSL Behring-certified treatment centres that have completed site qualification, staff training, and infusion-readiness validation. Vials are stored frozen and require defined thaw and handling steps before infusion per the FDA-approved package insert. The product is supplied as a ready-to-dilute solution requiring aseptic preparation in the infusion suite, dilution per label, and immediate use within the post-thaw window. There is no off-the-shelf inventory; each dose is calculated and assembled per patient at a single one-time intravenous dose of 2×10^{13} genome copies per kilogram of body weight, infused intravenously over a defined time interval with patient monitoring throughout.

For Indian patients, this means selecting a destination treatment centre in a jurisdiction where Hemgenix is registered and a CSL-certified hemophilia treatment centre exists. In the US, the CSL Behring certified-centre network includes major academic hemophilia treatment centres. In the EU, the EMA conditional marketing authorization since February 2023 supports a certified-centre network across selected member states with country-by-country payer rollout. The UK MHRA authorization supports a comparable certified-centre framework. India does not have a Hemgenix-certified infusion centre at the time of this review. The treating Indian hematologist (often at AIIMS New Delhi, Christian Medical College Vellore, Tata Memorial Centre Mumbai, Apollo, Kokilaben, or Medanta) coordinates the referral, the pre-treatment clinical workup (factor IX activity baseline, AAV5 neutralizing antibody titre testing, liver function tests including ALT, AST, alkaline phosphatase, bilirubin, hepatitis B and C screening, assessment for active liver disease or significant hepatic fibrosis), and the long-term follow-up after the family returns home.

Patients with active or untreated viral hepatitis, advanced fibrosis, or other significant liver pathology require evaluation before consideration. Patients who are AAV5-antibody positive above the protocol threshold are typically not candidates under the current label. These two screening results, available locally in India through tertiary hematology laboratories, materially shape the go/no-go decision and the destination-centre conversation before any cross-border logistics step begins.

Real cost picture for Hemgenix for Indian patients

Costs sit in Indian rupees with the rupee floating against the US dollar. In May 2026 the USD/INR rate is in the 94 to 95 range. Pricing in this section is expressed in USD for portability; the actual invoice converts at the prevailing rate on the day of the transaction.

The US wholesale acquisition cost for Hemgenix is approximately USD 3.5 million per patient for the single one-time infusion, as set by CSL Behring at launch in late 2022 and reported by Fierce Pharma, Managed Healthcare Executive, and Hemophilia News Today, among others. CSL Behring's pricing rationale references the one-time nature of the therapy, the projected reduction in lifetime prophylaxis cost (the company cites that figure 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) issued a cost-effectiveness assessment placing a fair price range at roughly USD 2.9 million, below the launched WAC. International list prices vary by jurisdiction and are commonly negotiated confidentially with national payers.

For Indian families, the cost envelope must include the drug cost, the destination centre's infusion and pre-infusion workup charges, the AAV5 antibody titre testing where it is repeated abroad, the post-infusion monitoring window including the corticosteroid algorithm for transaminitis if triggered, travel and accommodation for the patient and at least one caregiver,

visa and immigration processing, and the pre-departure clinical workup costs that remain in India. Total time abroad is typically shorter than for an autologous CAR-T (Hemgenix is an in-vivo gene therapy without a manufacturing cycle), but the immediate post-infusion monitoring window is structured and the patient should plan on remaining within proximity of the destination centre for several weeks following infusion at minimum.

None of the major Indian private insurers (Star Health and Allied Insurance, HDFC ERGO, ICICI Lombard, Niva Bupa) reimburse a cross-border gene therapy at this price tier as a standard line item. CGHS and ESIC are not structured for cross-border gene-therapy reimbursement. The NPRD 2021 INR 50 lakh financial-assistance ceiling under the Rashtriya Arogya Nidhi umbrella scheme is structured around one-time treatments for rare diseases, which is conceptually aligned with a one-time gene therapy, but the INR 50 lakh ceiling is materially below the Hemgenix list price even before cross-border logistics, and the operating expectation has not been NPRD coverage for cases at this cost tier. Cash-pay through diaspora-funded or family-pooled financial structures is the working financial posture.

Typical timeline for Hemgenix for Indian patients

The Hemgenix operational arc is shorter than an autologous cell therapy because there is no patient-specific manufacturing cycle. The dose is calculated and the AAV5 vector is supplied as a ready-to-dilute solution from CSL Behring's specialty logistics chain to the destination certified centre against the confirmed candidate's body weight. From the patient's perspective, the cycle runs: pre-treatment clinical workup in India (AAV5 neutralizing antibody titre, factor IX activity, liver function tests, hepatitis screening, assessment for hepatic fibrosis, commonly 2 to 4 weeks); destination-centre referral and acceptance (commonly 2 to 6 weeks); travel and visa preparation (commonly 2 to 8 weeks depending on destination jurisdiction); arrival and confirmatory workup at the destination centre; the single intravenous infusion; the structured post-infusion monitoring window with serial ALT monitoring and the corticosteroid taper triggered by transaminase elevation per the label-specified algorithm; and return travel back to India once the destination centre confirms the patient is stable for the next phase of follow-up.

Long-term durability follow-up extends for years. Transaminases are monitored at defined intervals, with an expected rise in ALT in a meaningful fraction of patients managed with the label-specified corticosteroid algorithm. Factor IX activity is measured serially to track endogenous expression. The treating Indian hematologist coordinates the long-term monitoring with the destination centre's hemophilia-treatment-centre team. Total time abroad commonly runs 4 to 8 weeks depending on the destination centre's protocol and any transaminitis management requirements; some centres permit earlier return home with structured remote follow-up.

What your physician needs to provide

The clinical justification framework for Hemgenix differs from a typical NPP filing because Rule 36 does not gate the gene therapy itself. The treating Indian hematologist's responsibility runs to documentation supporting the destination-centre referral: a confirmed diagnosis of hemophilia B (congenital factor IX deficiency) with factor IX activity level documented; the prophylaxis history with current product, dose, frequency, and adherence; the bleeding history including any current or historical life-threatening hemorrhage or repeated serious spontaneous bleeding episodes that align with the FDA-approved indication; the AAV5 neutralizing antibody titre result from a qualified testing laboratory (mandatory under the label and the gating eligibility test); baseline liver function tests (ALT, AST, alkaline phosphatase, bilirubin); hepatitis B and hepatitis C

screening results; assessment for active liver disease or significant hepatic fibrosis; and a written referral to the destination centre's hemophilia treatment team. The destination centre then runs its own intake assessment, including site-readiness for the infusion and the post-infusion monitoring window.

Any ancillary medicines that the patient brings home for the corticosteroid algorithm or for ongoing hepatic supportive care, where unregistered locally, route through standard Rule 36 with the treating Indian hematologist's NMC-registered prescription. The Pharmacovigilance Programme of India (PvPI) adverse-event reporting obligation through the Indian Pharmacopoeia Commission continues for any imported product, and Reserve Meds includes the PvPI reference in the relevant documentation kit; the reporting obligation itself stays with the prescribing physician.

Common questions about Hemgenix in India

What if the AAV5 antibody titre is positive?

Pre-treatment AAV5 neutralizing antibody titre testing is mandatory under the Hemgenix label and is the gating eligibility test. A meaningful fraction of adult hemophilia B patients globally are AAV5-antibody positive above the protocol threshold and are not candidates under the current label. The test result is typically the first data point Reserve Meds confirms before any further coordination step. Patients who do not meet the antibody threshold remain on their existing factor IX prophylaxis; the treating hematologist may discuss alternative pathways including the second hemophilia B gene therapy (fidanacogene elaparvovec / Beqvez) or continued prophylaxis with extended half-life factor IX products.

Will the NPRD INR 50 lakh ceiling cover Hemgenix?

The NPRD 2021 financial-assistance ceiling under the Rashtriya Arogya Nidhi umbrella scheme is structured around one-time treatments for rare diseases, which is conceptually aligned with a one-time gene therapy. Hemophilia is listed under the NPRD framework. The INR 50 lakh ceiling is materially below the Hemgenix list price even before cross-border logistics, and the operating expectation has not been NPRD coverage for cases at this cost tier. Families confirm eligibility with the NPRD coordinator at the designated Centre of Excellence before assuming any partial-coverage scenario.

What is the safety profile we should be aware of?

The 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 (a tapering oral corticosteroid course initiated when ALT rises above protocol thresholds, monitored through resolution). Long-term follow-up through 5 years has not identified AAV-related oncogenicity or chronic hepatotoxicity in reported data, but the durability follow-up window extends for years.

What about pediatric patients?

The FDA-approved indication is currently restricted to adults. Pediatric and adolescent certification pathways are under development globally but are not the operating clinical indication at the time of this review. Reserve Meds does not coordinate pediatric Hemgenix cases.

Will Star Health, HDFC ERGO, ICICI Lombard, or Niva Bupa reimburse a cross-border Hemgenix case?

None of the major Indian private insurers reimburse a cross-border gene therapy at this price tier as a standard line item. Reserve Meds itemises the patient-side coordination charges separately from destination-centre medical charges so the family can pursue any reimbursement attempt with clean documentation. Cash-pay is the working posture.

Does FCRA affect a diaspora-funded Hemgenix case?

The Foreign Contribution (Regulation) Act 2010 (FCRA), as proposed to be amended by the Foreign Contribution (Regulation) Amendment Bill 2026, regulates foreign donations to Indian organisations and individuals. For a patient family paying for the treatment themselves, including an adult child or sibling overseas paying for an Indian-resident patient's care, FCRA is generally not engaged. Where a foreign foundation or hemophilia-focused diaspora group is funding the treatment, FCRA registration of the recipient organisation and the donation route can become relevant. Reserve Meds does not provide FCRA legal advice; we flag the question so it reaches the right adviser early.

Where Reserve Meds fits in Hemgenix cases

Reserve Meds is a US-based concierge coordinator. We do not administer Hemgenix, do not perform infusions, do not run the AAV5 antibody assay, and do not act as a clinical decision-maker. The coordination value sits in upstream patient and family orientation: confirming the AAV5 antibody screening result as the gating eligibility test before any further coordination step, confirming cash-pay capacity at the reference price tier, supporting the destination-centre referral arranged by the treating Indian hematologist, helping the family understand the cost envelope including hospitalisation and post-infusion monitoring, and coordinating travel and stay logistics across the 4-to-8-week destination-country window. The Reserve Meds Concierge Patient Coordinator carries the case from intake through return-to-India and into the long-term durability follow-up window with the treating Indian hematologist and the destination certified centre. Combinations with other named-patient orders are unusual; Hemgenix coordination is a standalone single-event case profile. No prior Reserve Meds case experience exists for Hemgenix as of this review.

Next step

If an Indian adult patient with hemophilia B has a treating hematologist considering gene therapy and the family has specifically chosen Hemgenix, add the case to the waitlist. We will respond within 24 to 48 hours with an orientation memo on the AAV5 antibody pre-testing requirement, the destination-centre referral framework, and an indicative cost envelope.

Reserved for you.

This guide is informational, not medical or legal advice. The named-patient framework requires a licensed Indian physician's clinical judgment; Reserve Meds is the coordinator, not the prescriber.