

Empaveli access in India

How Indian families with paroxysmal nocturnal hemoglobinuria (PNH) pursue Empaveli (pegcetacoplan), a C3 complement inhibitor delivered by self-administered subcutaneous infusion.

Last reviewed 2026-05-12 by Reserve Meds clinical & regulatory team. This page combines the India country regulatory module with the Empaveli drug module to describe the path families actually walk.

Quick orientation

Empaveli (pegcetacoplan) is a pegylated C3 complement inhibitor approved by the US Food and Drug Administration in May 2021 for adults with paroxysmal nocturnal hemoglobinuria (PNH). The product is marketed approximately by Apellis Pharmaceuticals. Pegcetacoplan targets the central complement protein C3, controlling both intravascular and extravascular hemolysis, and is delivered as a twice-weekly self-administered subcutaneous infusion. Its mechanism distinguishes it from terminal complement inhibitors that target C5. For Indian PNH patients who remain anemic on C5 inhibitor therapy because of residual extravascular hemolysis, pegcetacoplan is a meaningful therapeutic option. Reserved for you.

Why this drug is hard to source in India

PNH is a rare acquired hematologic disorder, with an Indian patient population estimated in the low thousands. Empaveli does not have a current Indian marketing authorisation through CDSCO. The terminal complement inhibitor eculizumab (Soliris) has been available in India through specialty importers, and the next-generation longer-acting C5 inhibitor ravulizumab (Ultomiris) is similarly accessible by import. For pegcetacoplan specifically, the originator manufacturer's launch sequence has prioritised US and select reference markets, and an Indian filing has not been a near-term focus. Indian-made generics or biosimilars of pegcetacoplan do not exist as of this review date. Patients hitting an access wall on Empaveli in India are typically those who have failed to achieve transfusion independence on a C5 inhibitor, and whose treating hematologist judges that a C3-level inhibitor is the appropriate next line.

The CDSCO personal-import pathway under Rule 36

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 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; Form 12B is the permit issued by the office of the Drugs Controller General of India at FDA Bhawan in New Delhi or by designated CDSCO Port Offices. The application is accompanied by a prescription from a Registered Medical Practitioner showing the practitioner's NMC registration number and the quantity required for treatment, capped at one hundred average doses per application.

For Empaveli, the routine application set is sufficient where the treating hematologist documents the PNH diagnosis with flow cytometry confirmation, the prior course on C5 inhibitor therapy if applicable, the rationale for transition to a C3 inhibitor, the meningococcal vaccination status, and the planned monitoring schedule. The Form 12B permit issues on the CDSCO documented priority timeline, typically within one to two business days for routine applications with complete documentation. End-to-end from physician decision to dispensed product is typically two to four weeks, with most of that window in documentation and international logistics rather than the regulator's stamp.

Real costs in INR and USD

The US wholesale acquisition cost for Empaveli is approximately USD 460,000 to 500,000 per year for a typical adult on twice-weekly subcutaneous dosing. In INR at the prevailing 94 to 95 range, that translates to approximately INR 4.3 crore to 4.7 crore per year. Indian-made generics or biosimilars of pegcetacoplan do not exist as of this review date. The molecule is under patent protection and the biologic complexity of a pegylated cyclic peptide does not support a near-term biosimilar pathway in India. Indian Union Budget 2026-27 expanded the customs duty exemption list for rare-disease drugs, and PNH treatments may qualify under the rare-disease exemption depending on the specific HSN classification at filing.

The National Policy for Rare Diseases 2021 framework with its INR 50 lakh per patient ceiling under the Rashtriya Arogya Nidhi umbrella is relevant context but is meaningfully below an annual Empaveli course. Reserve Meds patient cases default to cash-pay, with the documentation kit structured so the patient or hospital can pursue insurance, CGHS Expert Committee, or rare-disease scheme reimbursement after the fact where eligibility allows.

Timing, what to expect

From physician decision to first dispensed dose, a routine Empaveli case runs two to four weeks. Meningococcal vaccination should be completed at least two weeks before the first dose, which sometimes is the practical scheduling constraint rather than the import timeline. Documentation assembly takes three to five business days. The Form 12B permit issues in one to two business days on the priority timeline. US-side sourcing and cold-chain shipment runs one to two weeks. For ongoing supply, Reserve Meds aligns refill cycles to a four to six week reorder rhythm so the patient does not run short between cycles.

What your physician needs

The clinical justification letter for an Empaveli Form 12A submission documents the PNH diagnosis with flow cytometry confirming GPI-anchored protein deficiency, the prior treatment history including any C5 inhibitor course and the response, the rationale for transition to a C3 inhibitor (commonly residual extravascular hemolysis with persistent anemia), the meningococcal vaccination record (meningococcal A/C/W/Y and B), the patient training plan for self-administered subcutaneous infusion, and the planned monitoring schedule. The treating hematologist's NMC registration number appears on the prescription. The dispensing facility's drug licence number is part of the submission package.

Customs clearance and IOR

Customs at the port of entry reviews the Form 12B permit, the commercial documentation, the airway bill, the cold-chain monitoring record, and the importer's drug licence. The Importer of Record is the licensed dispensing facility or specialty importer holding the wholesale drug licence under the Drugs and Cosmetics Rules. Reserve Meds does not act as the IOR. We align with the licensed importer named on the Form 12B permit. Empaveli is stored refrigerated at 2 to 8 degrees Celsius; temperature excursions during transit are documented through the carrier monitor.

Pharmacovigilance

India operates the Pharmacovigilance Programme of India (PvPI) coordinated by the Indian Pharmacopoeia Commission. Treating physicians report adverse events through PvPI for imported products. For Empaveli specifically, the principal post-marketing safety concerns are infection risk including encapsulated bacterial infection (mitigated by meningococcal vaccination), injection-site reactions, and complement-related laboratory monitoring. The prescribing physician's monitoring schedule is documented in the Form 12A clinical

justification letter, and the PvPI reference is included in the Reserve Meds physician documentation kit.

Where Reserve Meds fits

Reserve Meds is a US-based concierge coordinator. We do not replace the treating hematologist, CDSCO, the hospital pharmacy, or the licensed importer. For an Empaveli case, our work is US-side sourcing, documentation orchestration, cold-chain logistics coordination, and a single named coordinator who carries the case from the first cycle through the ongoing refill rhythm.

Next step

If a treating hematologist in India is weighing Empaveli for a patient with PNH, the waitlist is the first step. We respond within 24 to 48 hours with an eligibility confirmation and a documentation kit for the physician.

Reserved for you.

Related

- Empaveli clinical resource
- Ultomiris in India
- India country page
- CDSCO personal-import pathway

Sources

1. FDA approval, Empaveli (pegcetacoplan), Apellis Pharmaceuticals, BLA approval May 2021 for PNH.
2. CDSCO, Procedure for Permission to Import Small Quantities of Drugs for Personal Use (Form 12A / Form 12B under Rule 36, Drugs and Cosmetics Rules 1945).
3. Indian Pharmacopoeia Commission, Pharmacovigilance Programme of India (PvPI) reporting framework.

Common questions Indian families ask

Will Star Health, HDFC ERGO, ICICI Lombard, or Niva Bupa cover this? Each Indian private insurer assesses named-patient imports case by case. None of the major private insurers reimburse a Rule 36 personal import as a standard line item. Some have

reimbursed full or partial drug cost where the underlying medicine is on the formulary and the named-patient route operated as a stocking workaround. Reserve Meds supplies the documentation that lets your insurer evaluate. The claim itself is filed by the patient or the hospital. Cash-pay remains the default posture for Reserve Meds patient cases.

Will my CGHS or ESIC entitlement cover this? CGHS (Central Government Health Scheme) provides for life-saving and anti-cancer medicines not in the standard formulary to be considered case by case by an Expert Committee under the Special DG (DGHS) where the prescribing specialist documents the requirement. Drugs not approved by the DCGI for use in India face a stricter Expert Committee review. ESIC's formulary is narrower. Neither scheme is structured for routine personal-import reimbursement; check eligibility with your CGHS Wellness Centre or the ESIC dispensary before assuming coverage.

Will my AIIMS, Tata Memorial, Apollo, Fortis, Medanta, Kokilaben, MGM, CMC Vellore, or Manipal physician's letter be sufficient? Yes. A Registered Medical Practitioner with a valid National Medical Commission registration number can support a Form 12A application. Physicians at AIIMS, Tata Memorial Centre, government medical colleges, and state-run tertiary hospitals routinely do so. Private-sector specialists at Apollo, Fortis, Medanta, Kokilaben Dhirubhai Ambani Hospital, MGM Healthcare, CMC Vellore, and Manipal Hospitals also have signing authority subject to their institutional drug licence.

What if my treating institution does not have an import pharmacy desk? The practical route is to work with one of the named tertiary centres that handles compassionate and named-patient imports as established workflow, or with a CDSCO-licensed specialty importer in Mumbai, Delhi, or Bangalore that handles the documentation and chain-of-custody on behalf of smaller hospitals or independent specialists. Reserve Meds aligns with the importer named on the Form 12B permit.

Can I receive the drug at home, or do I need a hospital? The dispensing facility must hold a valid drug licence under the Drugs and Cosmetics Rules. For oral medicines, a hospital outpatient pharmacy or a licensed import pharmacy is the dispensing point. For infusion products, the medicine ships to the infusion centre where the patient will receive it. Direct-to-home delivery outside a licensed dispensing facility is not the model.

What about pediatric patients? The Rule 36 framework applies the same way for pediatric patients. The clinical justification letter typically includes weight-adjusted dosing and pediatric-specific monitoring. AIIMS, Tata Memorial, Apollo, Kokilaben, and CMC Vellore handle pediatric named-patient imports routinely. Where the indication is approved in adults only, the off-label use is the physician's clinical judgement and is documented as such in the Form 12A letter.

Does FCRA affect a patient 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 medicine themselves, FCRA is generally not engaged. Where a foreign foundation or diaspora group is funding a treatment, FCRA registration of the recipient organisation and the donation route can become relevant; the structure should be reviewed with counsel before funds move. Reserve Meds does not provide FCRA legal advice; we flag the question so it reaches the right adviser early.

What is the role of the Indian Pharmacopoeia Commission? The Indian Pharmacopoeia Commission coordinates the Pharmacovigilance Programme of India (PvPI) and publishes the Indian Pharmacopoeia, the legal compendium of pharmaceutical standards in India. For imported originator products under Rule 36, the PvPI reporting framework applies to adverse event surveillance, and the prescribing physician is the reporting party. The Indian Pharmacopoeia is the reference standard against which Indian-manufactured products are tested; imported originator products carry their reference-country pharmacopoeial standards (typically USP for FDA-approved products).

How does this compare with access in the UAE or Saudi Arabia? India's Rule 36 framework with the published Form 12B priority timeline (one to two business days for routine documentation) is often faster than the SFDA Personal Importation Program in Saudi Arabia (typically 10 to 21 business days routine) and broadly comparable to the UAE Emirates Drug Establishment pathway. India's offsetting friction is the customs and logistics layer rather than the regulator's stamp. India's tertiary specialty hospital depth substantially exceeds any single peer country in the South Asia and GCC region, which usually offsets the longer end-to-end cycle for complex cases.

What documentation does my family need to assemble before contacting Reserve Meds? The minimum useful package is the treating physician's name and registration number, the patient's diagnosis and current treatment summary, recent relevant investigations (imaging, biopsy, molecular pathology, blood work as applicable), and a contact pathway to the dispensing facility you intend to use. With that package, Reserve Meds can complete eligibility within 24 to 48 hours and route the documentation kit to your physician.

How Indian families coordinate across cities and countries

For Indian families, the coordination problem is often distributed across multiple cities and sometimes multiple countries. A grandmother in Hyderabad, an oncologist at Tata Memorial in Mumbai, an adult child in Bangalore managing logistics, and a son in Dubai or London paying the invoice is a common configuration. The Reserve Meds single named coordinator model is built for exactly this pattern. One coordinator carries the case file, one chain of correspondence captures the decisions and documents, and one set of contact records reaches every family member who needs visibility into the case, regardless of how many

cities the family touches or how many time zones the case spans. The Reserve Meds patient portal at portal.reservemed.com holds the document set and the case timeline; the coordinator handles the email, phone, and WhatsApp follow-through that the case needs at each step.

For smaller cities where the local hospital does not maintain an import pharmacy desk, the practical route is to work with a CDSCO-licensed specialty importer in Mumbai, Delhi, Bangalore, Chennai, or Hyderabad. The importer carries the CDSCO relationship, the customs broker relationship, and the chain-of-custody documentation. Reserve Meds aligns with the importer on US-side sourcing and with the treating physician on clinical documentation. The patient sees one face throughout, which is the named coordinator.

The patient experience, step by step

From the family side, the sequence looks like this. Your physician decides this drug is the right next step. That is a clinical decision and stays with them. Your physician or the hospital pharmacy team reaches out to Reserve Meds, or the patient submits a request through the Reserve Meds portal and Reserve Meds connects with the physician. Reserve Meds confirms eligibility within 24 to 48 hours and sends a documentation kit to your physician, including the Form 12A reference, the clinical justification letter template, and the chain-of-custody plan. Your physician completes the documentation, attaches the prescription with their NMC registration number, and the application goes to CDSCO through the appropriate port office or the DCGI New Delhi office, or via the hospital's licensed importer. The Form 12B permit issues on the documented priority timeline. While the permit issues, Reserve Meds aligns US-side sourcing and the shipment plan with the dispensing pharmacy. The shipment moves cold-chain or ambient as appropriate. Customs at the destination port reviews the permit and clears the consignment. The dispensing pharmacy receives, logs, and stores the medicine according to its drug licence requirements. Your physician initiates therapy. Adverse event reporting through PvPI continues for the duration of therapy.

Review and oversight. Content on this page is reviewed by the Reserve Meds clinical and regulatory team. A US-licensed pharmacist reviews every prescription before dispensing. Regulatory posture is informational, not legal advice; case-specific questions route to retained outside counsel. Review methodology ›

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