Roctavian access in India: the CDSCO Rule 36 named-patient pathway

How adult patients in India with severe hemophilia A access Roctavian, the first AAV5 gene therapy approved in the United States and EU, when the infusion requires travel to a certified treatment centre abroad and the list price is approximately USD 2.9 million.

Last reviewed 2026-05-12 by Reserve Meds clinical and regulatory team. This page describes the personal-import and certified-treatment-centre pathway for Roctavian for adult patients in India with severe hemophilia A who meet the eligibility profile for the AAV5 gene therapy.

Section 1. Quick orientation

Roctavian (valoctocogene roxaparvovec-rvox) is a one-time intravenous gene therapy for severe hemophilia A in adults, manufactured by BioMarin Pharmaceutical Inc. of San Rafael, California. The modality is an adeno-associated virus serotype 5 (AAV5) vector that delivers a B-domain-deleted human coagulation factor VIII (FVIII) transgene to hepatocytes, where the transgene drives endogenous FVIII production and partially corrects the underlying clotting defect. The US Food and Drug Administration approved Roctavian on 29 June 2023 for adults with severe hemophilia A (FVIII activity less than 1 IU/dL) who do not have pre-existing antibodies to AAV5 (detected by an FDA-approved test) and have no history of FVIII inhibitors. The European Commission granted conditional marketing authorisation on 24 August 2022. Roctavian is not registered with the Central Drugs Standard Control Organization (CDSCO) for commercial sale in India. There is no BioMarin-certified treatment centre in India. For Indian adults who meet the eligibility profile, the lawful pathway is the personal-importation framework under Rule 36 of the Drugs and Cosmetics Rules 1945, combined with travel to a certified BioMarin treatment centre in the United States or European Union where the infusion is administered. Reserve Meds coordinates the eligibility verification, the certified-centre booking, the frozen-product logistics, and the long-term monitoring framework. **Reserved for you.**

Section 2. Why India patients need Roctavian through a named-patient pathway

India carries a significant hemophilia A patient population. The Hemophilia Federation (India), known as HFI, is the national advocacy organisation supporting people with bleeding disorders across the country through patient registries, education, and access advocacy; HFI's network of chapters and patient support groups is the most established hemophilia-community infrastructure in South Asia. The standard-of-care reality in India for severe hemophilia A is recombinant or plasma-derived FVIII prophylaxis where supply and reimbursement permit, with emicizumab (Hemlibra) available where the patient or family can fund it or where state-level schemes cover it. Roctavian represents a categorically different option: a one-time infusion intended to provide multi-year freedom from prophylaxis dosing for patients who meet the eligibility profile.

The structural reasons Indian adults pursue Roctavian through a cross-border pathway are concrete. BioMarin has not registered Roctavian with the CDSCO. There is no certified treatment centre in India because BioMarin's commercial footprint in the Asia-Pacific region for this product is limited and the certified-centre training, vector handling certification, infusion readiness sign-off, and outcomes warranty infrastructure have not been built locally. Patients in India who meet the eligibility profile (severe hemophilia A, AAV5-negative, no FVIII inhibitor history, no contraindicating hepatic disease) and who can self-fund or have verified third-party funding must travel to a BioMarin-qualified Hemophilia Treatment Centre in the United States or European Union to receive the infusion. The personal-import permit under Rule 36 facilitates the logistics for the cross-border movement of the product where the patient's home country requires documentation, but the infusion itself is administered at the certified centre abroad, not in India.

Section 3. The CDSCO Rule 36 named-patient pathway and the BioMarin certified-centre pathway for Roctavian

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 small quantities 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 (DCGI) at FDA Bhawan, Kotla Road, New Delhi, or by designated CDSCO Port Offices. For institutional Compassionate Use of drugs not approved for marketing in India at all, the pathway is the Compassionate Use application route to the DCGI by a government hospital, a registered medical practitioner, a pharmaceutical company, or the patient. Government institutions including AIIMS have established workflow for this pathway.

For Roctavian specifically, the dominant pathway is travel-and-infuse abroad rather than import-and-infuse-at-home, because BioMarin does not support infusion outside a qualified Hemophilia Treatment Centre. The Rule 36 framework remains relevant for the patient's pre-infusion AAV5 antibody testing logistics (the FDA-approved companion test may not be available at all Indian hematology labs and sample shipment abroad may require import-export documentation), for the post-infusion corticosteroid prescription if filled in India, and for any pre-infusion factor product the patient brings into India in transit.

The cell-specific clinical-justification framework for Roctavian is the gene-therapy eligibility envelope. BioMarin's RoctavianConnect programme handles the eligibility verification: confirmed severe hemophilia A with FVIII activity less than 1 IU/dL, negative AAV5 neutralising antibody titre on the FDA-approved companion test, no current or historical FVIII inhibitors confirmed through prior medical records (not patient recall alone), baseline liver function tests (ALT, AST, alkaline phosphatase, total bilirubin) within an acceptable range, no significant fibrosis, and no active hepatitis B or C or HIV with uncontrolled viral load. Patients who do not pass any one of these gates are not Roctavian candidates and the conversation routes back to the treating hematologist for alternative therapy planning.

Section 4. Where Roctavian gets infused for Indian patients

Roctavian is supplied only to a limited network of BioMarin-qualified Hemophilia Treatment Centres (HTCs) that have completed product-specific training, vector handling certification, and infusion readiness sign-off. There are no certified centres in India. For Indian adults who meet the eligibility profile, the available infusion locations are HTCs in the United States and in

European Union countries where Roctavian is commercially available. The Indian-side institutional partners that handle pre-travel eligibility work-up, post-travel monitoring, and corticosteroid management are the major tertiary hematology centres.

Christian Medical College (CMC) Vellore is globally recognised for hematology and runs a comprehensive hemophilia programme; CMC's hematology unit is one of the most experienced in India for complex bleeding-disorder management and can support both the pre-travel work-up and the post-travel monitoring framework. All India Institute of Medical Sciences (AIIMS), New Delhi has an established hemophilia programme and the institutional capacity to handle compassionate-use documentation. Tata Memorial Centre, Mumbai handles hematology cases through its medical oncology and hematology divisions. Apollo Hospitals (Chennai flagship, Delhi, Bangalore, Hyderabad, Kolkata), Fortis Memorial Research Institute (Gurgaon) and Fortis network sites, Medanta - The Medicity (Gurgaon), Kokilaben Dhirubhai Ambani Hospital (Mumbai), and Manipal Hospitals (Bangalore) all hold the hematology consultant infrastructure and the institutional drug licences needed for pre- and post-infusion management. For families who are also active in HFI chapters, the chapter network can support patient education and family logistics around the travel-and-infuse arc, alongside the clinical institutions handling the medical case.

The travel-and-infuse logistics are demanding. The patient must travel to the certified centre, undergo final pre-infusion confirmation locally at that centre, receive the one-time intravenous infusion over approximately two hours, and remain near the certified centre for weeks of post-infusion corticosteroid management and FVIII expression monitoring before returning to India. Reserve Meds coordinates the certified-centre booking, the patient and family travel logistics, the visa documentation flagging where needed, and the handoff between the certified centre's hematology team and the Indian-side hematology team for post-travel monitoring.

Section 5. Real cost picture for Roctavian in India

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. Reserve Meds quotes are itemised, not bundled.

- **Drug cost reference.** US wholesale acquisition cost is approximately USD 2.9 million per patient for the single one-time dose, publicly disclosed by BioMarin at the June 2023 launch and corroborated by FiercePharma, BioPharma Dive, and Managed Healthcare Executive. This is a list price subject to confidential payer-specific net pricing and to the outcomes-based warranty rebate structure in the US market, neither of which extends to international self-pay patients. EU pricing is country-specific and confidential at the net level; publicly reported German list reference and negotiated reimbursement amounts indicate an order-of-magnitude similar to US WAC. International self-pay patients should be quoted against the US WAC plus the full coordination, travel, infusion-centre, and post-infusion monitoring cost stack, not against a discounted figure. In INR terms, the drug-only list price corresponds to approximately INR 27 to 28 crore at the prevailing rate.
- **International logistics and travel.** Frozen-product logistics from BioMarin to the certified centre run on validated dry-ice or deep-frozen shippers with continuous temperature monitoring; this cost is largely embedded in BioMarin's supply chain to the certified centre rather than in a separate international shipping line to India. The dominant travel-side line items are patient and immediate-family travel (international airfare,

accommodation near the certified centre for the multi-week post-infusion monitoring window), local ground transport, visa processing where applicable, and incidentals. A realistic envelope is USD 30,000 to 80,000 for the travel-and-stay layer depending on certified-centre location, family configuration, and stay duration.

- **Pre-travel work-up and post-travel monitoring.** AAV5 antibody testing at the FDA-approved companion test, FVIII inhibitor history confirmation, baseline liver function tests, and any imaging required at the Indian-side hematology centre. Post-travel weekly liver function tests during the corticosteroid taper, then tapering frequency over the first year, FVIII activity monitoring at protocol-defined intervals to track expression, hepatocellular carcinoma surveillance per HTC protocol, and bleeding-event tracking. These costs are paid to the Indian-side institution (AIIMS, CMC Vellore, Apollo, Fortis, Medanta, Kokilaben, Manipal) and are typically modest relative to the drug and travel layers.
- **Regulatory and concierge.** CDSCO documentation fees where applicable, Reserve Meds' concierge coordination fee itemised separately on every firm quote.

India's private insurance market does not reimburse a USD 2.9 million one-time gene therapy as a standard line item. The Pradhan Mantri Jan Arogya Yojana (PMJAY) does not, in practice, cover Roctavian at this price point. The Rashtriya Arogya Nidhi umbrella scheme under the National Policy for Rare Diseases (NPRD) 2021 contemplates a one-time financial assistance ceiling currently set at INR 50 lakh per patient for rare diseases requiring one-time treatment; that ceiling is meaningful relief but covers approximately 2 percent of the drug-only list price. CGHS provides for life-saving medicines not in the standard formulary to be considered case-by-case by an Expert Committee under Special DG (DGHS); CAR-T and gene therapy products have been considered through this route in select cases. Cash-pay funded through patient and family resources, sometimes supplemented by diaspora contribution, is the default posture; where a foreign foundation or diaspora group is funding part of the treatment, FCRA (Foreign Contribution Regulation Act 2010, as proposed to be amended by the 2026 Bill) registration of the recipient organisation and the donation route should be reviewed with counsel before funds move.

Section 6. Typical timeline for Roctavian in India

Roctavian timelines extend materially beyond the Rule 36 regulatory clock because the certified-centre pathway is the rate-limiting layer. Pre-travel eligibility work-up at an Indian hematology centre (AAV5 antibody titre, FVIII activity confirmation, FVIII inhibitor history, baseline liver work-up) typically takes 4 to 8 weeks depending on sample-shipment logistics for the AAV5 companion test if performed abroad and local laboratory scheduling. Certified-centre booking and BioMarin RoctavianConnect verification typically takes 4 to 12 weeks depending on the centre's capacity and the patient's documentation completeness. Travel preparation, visa processing where required, and family logistics take 2 to 8 weeks running in parallel. The certified-centre stay itself is several weeks, structured around the infusion day and the subsequent corticosteroid-managed monitoring window. Post-return monitoring in India runs for years. A realistic end-to-end planning horizon from first contact to infusion is 3 to 9 months. These ranges are typical, not promises.

Section 7. What your physician needs to provide

For Roctavian, the documentation work is more extensive than for any other molecule in the Reserve Meds matrix because both the Indian-side hematology team and the BioMarin certified centre require comprehensive medical records. The Indian-side treating hematologist provides the patient identifier, the severe hemophilia A diagnosis with FVIII activity confirmed less than 1 IU/dL, the FVIII inhibitor history confirmed negative through prior medical records (Bethesda titre history where available, not patient recall alone), the bleeding-event history and any prophylaxis regimen the patient is on (recombinant or plasma-derived FVIII, emicizumab), baseline liver function tests (ALT, AST, alkaline phosphatase, total bilirubin), any history of hepatic disease, hepatitis B or C status, HIV status with viral load if positive, and the rationale for selecting Roctavian over continued FVIII prophylaxis or emicizumab.

The AAV5 neutralising antibody titre on the FDA-approved companion test is a hard pre-gate; the result must be in hand and negative before any certified-centre booking is initiated. The Indian hematologist coordinates the sample collection and submission to the laboratory performing the FDA-approved test. The corticosteroid management plan is structured around the BioMarin label, which prescribes a prophylactic or reactive corticosteroid regimen (typically oral prednisone or equivalent) initiated based on ALT trends and tapered over weeks to months, individualised by the certified centre and continued under the Indian hematologist's supervision after return. The Indian hematologist's NMC registration number with state council registration is on every document; the certified centre's hematology team provides parallel documentation under their own institutional credentials. Compassionate Use application by an Indian government institution such as AIIMS may be filed for the Roctavian product where the institutional pathway is appropriate.

Section 8. Common questions about Roctavian in India

Will Star Health, HDFC ERGO, ICICI Lombard, or Niva Bupa cover this? A USD 2.9 million one-time gene therapy is not a standard reimbursable line item for any Indian private insurer. We do not promise coverage from any insurer.

Will PMJAY, NPRD/Rashtriya Arogya Nidhi, or CGHS cover this? PMJAY does not contemplate this price point. The NPRD/Rashtriya Arogya Nidhi rare-disease one-time-treatment ceiling at INR 50 lakh is meaningful but small relative to the drug list price. CGHS Expert Committee review under Special DG (DGHS) for non-formulary life-saving drugs is possible for eligible beneficiaries but a USD 2.9 million approval is not standard practice. Where a foreign foundation or diaspora group is funding part of the treatment, FCRA registration of the recipient organisation should be reviewed with counsel before funds move.

Can I be infused in India? No. BioMarin does not support infusion outside a qualified Hemophilia Treatment Centre, and there are no qualified centres in India. The infusion takes place at a certified centre in the United States or European Union, with travel, accommodation, and weeks of post-infusion monitoring at the centre as a non-negotiable part of the pathway.

Will my CMC Vellore, AIIMS, or Apollo hematologist's letter be sufficient? The Indian hematologist's clinical documentation is essential for the pre-travel work-up and post-travel monitoring, and feeds the certified centre's eligibility verification. The certified centre's own hematology team performs final pre-infusion confirmation and runs the infusion-day care; the case is genuinely co-managed.

What is the safety profile? The most common adverse reactions in the GENEr8-1 phase 3 trial were transient ALT elevation, headache, nausea, vomiting, fatigue, abdominal pain, and infusion-related reactions. The dominant safety signal is hepatic transaminase elevation requiring corticosteroid management. There is a theoretical long-term risk of hepatocellular carcinoma related to AAV vector integration; no causal cases have been reported in the trial programme, and the label requires long-term hepatic surveillance.

What is the durability picture? Roctavian is not pitched as a cure. GENEr8-1 follow-up published in NEJM and subsequent journal updates show that FVIII expression declines over time in many patients. At five years, 80.8 percent of participants remained off regular prophylaxis, and modelled median time-to-return-to-prophylaxis ranged from 6.4 to 16.1 years. The honest framing for Indian patients and families: meaningful but not permanent benefit for most, with a real possibility of returning to factor replacement therapy within a decade.

How does Roctavian compare to emicizumab? Standard of care alternatives are recombinant FVIII prophylaxis and emicizumab (Hemlibra, Roche/Genentech), a bispecific antibody dosed subcutaneously. Hemlibra is a long-running maintenance therapy, broadly available including in jurisdictions where Roctavian is not registered, and suitable for patients with inhibitors. Roctavian offers the prospect of multi-year freedom from prophylaxis dosing for eligible patients. The choice is the treating hematologist's; Reserve Meds does not steer the decision.

Section 9. Where Reserve Meds fits in Roctavian cases

Reserve Meds is a US-based concierge coordinator. We do not replace your hematologist, do not replace BioMarin's RoctavianConnect programme, do not replace the certified treatment centre, and do not replace the CDSCO. For Roctavian specifically, the orchestration we provide is eligibility verification coordination between the Indian-side hematologist and BioMarin's RoctavianConnect, AAV5 antibody testing logistics where the FDA-approved companion test is performed abroad, certified-centre booking and the parallel scheduling of pre-travel and travel logistics, support to the family on the travel-and-stay layer, and the documentation framework that hands off between the certified centre's hematology team and the Indian-side hematology team for post-travel monitoring. The Hemophilia Federation (India) and its chapter network often have existing relationships with families pursuing complex hemophilia therapies, and Reserve Meds works alongside HFI patient support rather than in place of it. Operating notes that bind the coordinator: AAV5 neutralising antibody titre is a hard pre-gate; FVIII inhibitor history is confirmed from records not recall; infusion is at the certified centre, not in India; durability is framed honestly; no off-label use; no pediatric coordination; no coordination for patients with active hepatic disease without explicit treating-centre sign-off. No prior Reserve Meds case experience exists for Roctavian at the date of this page. Standard NPP coordination applies with the gene-therapy-specific operating notes above as binding.

Section 10. Next step

If your hematologist has identified Roctavian as a possible option for severe hemophilia A and you are based in India, the next step is the waitlist. We confirm eligibility within 24 to 48 hours, route the conversation to a structured pre-travel work-up, and align with your hematology centre on the documentation framework. **Reserved for you.**

This guide is informational, not medical or legal advice. The Rule 36 framework and the BioMarin certified-centre pathway both require licensed clinical judgment; Reserve Meds is the coordinator, not the prescriber or the infusion centre.

Review & oversight. Content on this page is reviewed by Reserve Meds's 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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