Roctavian access in Pakistan: the DRAP and travel-to-treatment pathway

How adult patients in Pakistan 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 BioMarin treatment centre 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-travel-to-treatment pathway for Roctavian for adult patients in Pakistan with severe hemophilia A who meet the eligibility profile for the AAV5 gene therapy. We frame durability honestly throughout; Roctavian is not pitched as a cure.

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 Drug Regulatory Authority of Pakistan (DRAP) for commercial sale, and there is no BioMarin-certified Hemophilia Treatment Centre in Pakistan. For Pakistani adults who meet the eligibility profile, the lawful pathway is the DRAP Special Permission framework for the documentation and sample-shipment logistics combined with travel to a certified BioMarin treatment centre in the United States or European Union where the infusion is administered. The Hemophilia Patients Welfare Society Pakistan and the broader patient-advocacy network around the major haematology centres can run alongside the medical and logistics coordination. Reserve Meds coordinates eligibility verification, certified-centre booking, frozen-product logistics, the travel-and-stay framework, and the long-term monitoring handoff. **Reserved for you.**

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

Pakistan has a meaningful hemophilia A patient population concentrated in family registries managed through the major haematology centres and through the Hemophilia Patients Welfare Society Pakistan (HPWS Pakistan), the principal patient-advocacy organisation supporting people with bleeding disorders across the country. The standard-of-care reality in Pakistan for severe hemophilia A is recombinant or plasma-derived FVIII prophylaxis where supply and funding permit, with emicizumab (Hemlibra) accessible through named-patient channels and limited reimbursed pathways for patients with inhibitors or where prophylactic factor coverage is

constrained. Roctavian represents a categorically different option: a single intravenous infusion intended to provide multi-year freedom from prophylaxis dosing for patients who meet the eligibility profile.

The structural reasons Pakistani adults pursue Roctavian through a cross-border pathway are concrete. BioMarin has not registered Roctavian with DRAP. There is no certified Hemophilia Treatment Centre in Pakistan because BioMarin's commercial footprint in South Asia for this product is limited and the certified-centre training, AAV5 vector handling certification, infusion readiness sign-off, and outcomes warranty infrastructure have not been built locally. Patients in Pakistan who meet the eligibility profile (severe hemophilia A with FVIII activity less than 1 IU/dL, AAV5-negative on the FDA-approved companion test, 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 for the infusion. The DRAP Special Permission framework facilitates documentation for the cross-border movement of the AAV5 antibody-test sample (the FDA-approved companion test may not be available at all Pakistani haematology labs and sample shipment abroad may require import-export documentation), for the post-infusion corticosteroid prescription if filled in Pakistan, and for any pre-infusion factor product the patient brings into Pakistan in transit. The infusion itself takes place at the certified centre abroad. Pakistan's diaspora pattern is particularly relevant here: families routinely fund specialty care by pooling resources across overseas relatives in Saudi Arabia, the UAE, the UK, the United States, and Canada, and a Roctavian case stretches that funding model to its outer limit.

Section 3. The DRAP Special Permission pathway and the BioMarin certified-centre pathway for Roctavian

DRAP regulates the import of medicines through the Quality Assurance and Laboratory Testing Division's Import and Export Section, with Drug Registration Board oversight for new product registration matters. For unregistered medicines or therapeutic-goods movements required by a specific patient, DRAP issues a Special Permission, also referred to as the No Objection Certificate (NOC) for Personal Use Import. Applications are filed through DRAP's Online Import and Export System (OIES). Reserve Meds does not file with DRAP and does not act as a Pakistani importer of record.

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 and no such centre exists in Pakistan. The DRAP framework remains relevant for the patient's pre-infusion AAV5 antibody testing logistics (sample-shipment documentation if the FDA-approved companion test is performed abroad), for any pre-infusion factor product the patient brings into Pakistan in transit, and for the post-infusion corticosteroid prescription if filled in Pakistan rather than at the certified centre. Reserve Meds coordinates the AAV5 sample logistics, the certified-centre booking with the BioMarin RoctavianConnect programme, the patient and family travel logistics including visa documentation flagging where needed, and the handoff between the certified centre's haematology team and the Pakistani-side haematology team for post-travel monitoring.

The cell-specific clinical-justification framework for Roctavian is the gene-therapy eligibility envelope. BioMarin's RoctavianConnect handles the eligibility verification through the certified centre: 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 (Bethesda titre history where available, not

patient recall alone), baseline liver function tests (ALT, AST, alkaline phosphatase, total bilirubin) within an acceptable range, no significant fibrosis, no active hepatitis B or C, and HIV with controlled viral load if applicable. Patients who do not pass any one of these gates are not Roctavian candidates and the conversation routes back to the treating haematologist for continued FVIII prophylaxis or emicizumab. Routine DRAP documentation for the parallel logistics elements is typically processed in a four to eight week window once the package is complete; the certified-centre booking is the rate-limiting layer and runs separately on a four to twelve week cadence depending on the centre's capacity.

Section 4. Where Roctavian gets infused for Pakistani 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 Pakistan. For Pakistani 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 Pakistani-side institutional partners that handle pre-travel eligibility work-up, post-travel monitoring, and corticosteroid management are the major tertiary academic and private haematology centres.

Aga Khan University Hospital (AKUH) in Karachi, with its Department of Oncology and haematology services and 24/7 institutional pharmacy network with temperature-controlled storage, is the most experienced private-academic partner for complex bleeding-disorder management in southern Pakistan. The Pakistan Kidney and Liver Institute and Research Centre (PKLI) in Lahore, the public-sector specialty institute with kidney, liver, and bone marrow transplant programmes, provides hepatology and transplant-adjacent infrastructure relevant to the liver-focused pre- and post-Roctavian work-up. Shaukat Khanum Memorial Cancer Hospital and Research Centre in Lahore brings haematology and institutional-pharmacy infrastructure with a long track record of manufacturer relationships. Indus Hospital and Health Network in Karachi handles tertiary haematology cases through its referral network with collection centres in Karachi, Lahore, and Hyderabad. Liaquat National Hospital in Karachi, Shifa International Hospital in Islamabad serving the federal capital region, and the Combined Military Hospitals (CMH) network with tertiary haematology capacity at CMH Rawalpindi and CMH Lahore round out the institutional landscape. The Children's Hospital and Institute of Child Health in Lahore handles paediatric haematology, although Roctavian is approved for adults only and paediatric coordination is not within Reserve Meds' scope. The Hemophilia Patients Welfare Society Pakistan supports family education and logistics planning around the travel-and-infuse arc alongside the clinical institutions.

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 (dose-volume dependent), and remain near the certified centre for weeks of post-infusion corticosteroid management and FVIII expression monitoring before returning to Pakistan. Reserve Meds coordinates the certified-centre booking, the patient and family travel logistics, visa documentation flagging where needed, and the handoff between the certified centre's haematology team and the Pakistani-side haematology team for post-travel monitoring.

Section 5. Real cost picture for Roctavian in Pakistan

Reserve Meds quotes in US dollars and accepts USD wire transfers from any USD-accessible source. The Pakistani Rupee has been volatile across the last several years; as of May 2026 the

USD to PKR rate is in the 278 to 280 range. Quoting in USD insulates the patient and the family-pooled funding source from intra-case currency drift, which for a multi-month travel-and-infuse arc is particularly important.

- **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 PKR terms at the prevailing rate the drug-only list price corresponds to approximately PKR 808 million.
- **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 Pakistan. 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 (US B-2 medical-treatment visa or EU Schengen medical-treatment visa where applicable), and incidentals. A realistic envelope is USD 30,000 to USD 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 Pakistani-side haematology centre (AKUH, PKLI, Shaukat Khanum, Indus, Liaquat National, Shifa International, CMH). Post-travel weekly liver function tests during the corticosteroid taper, then tapering frequency over the first year, FVIII activity monitoring at protocol-defined intervals, hepatocellular carcinoma surveillance per HTC protocol, and bleeding-event tracking. These costs are paid to the Pakistani-side institution and are typically modest relative to the drug and travel layers.
- **Regulatory and concierge.** DRAP documentation fees where applicable, Reserve Meds' concierge coordination fee itemised separately on every firm quote.

Pakistan's private health insurers (State Life, Adamjee, EFU, Jubilee, IGI, Pak-Qatar Family Takaful) do not reimburse a USD 2.9 million one-time gene therapy as a standard line item. Each insurer assesses named-patient claims case by case, but a gene-therapy price point at this level is not a standard reimbursable line for any Pakistani insurer. The Sehat Sahulat Program's Rs. 1,000,000 per family per year ceiling does not contemplate Roctavian at any rollout. Cash-pay funded through patient and family resources, supplemented by diaspora contribution from relatives in Saudi Arabia, the UAE, the UK, the US, and Canada, is the default posture; Pakistan's remittance corridors with around USD 4.4 billion from Saudi Arabia, USD 3.1 billion from the UAE, and USD 2.7 billion from the UK in recent reporting periods make multi-country USD

coordination a practical reality for some families. BioMarin's US outcomes-based warranty and US copay assistance do not extend to international self-pay patients.

Section 6. Typical timeline for Roctavian in Pakistan

Roctavian timelines extend materially beyond the DRAP regulatory clock because the certified-centre pathway is the rate-limiting layer. Pre-travel eligibility work-up at a Pakistani haematology 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 (US B-2 medical-treatment visa or EU Schengen medical-treatment visa) 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 Pakistan runs for years. A realistic end-to-end planning horizon from first contact to infusion is three to nine months. These ranges are typical, not promises.

Section 7. What your physician needs to provide

For Roctavian the documentation work is the most extensive in the Reserve Meds matrix because both the Pakistani-side haematology team and the BioMarin certified centre require comprehensive medical records. The Pakistani-side treating haematologist provides the patient identifier (CNIC), 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 Pakistani haematologist coordinates the sample collection and submission to the laboratory performing the FDA-approved test, with DRAP sample-shipment documentation supporting the cross-border movement where required. 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 Pakistani haematologist's supervision after return. The Pakistani haematologist's PMDC license number is on every document; the certified centre's haematology team provides parallel documentation under their own institutional credentials. Pharmacovigilance reporting through the DRAP Pharmacovigilance Centre for the long-running post-infusion monitoring stays with the treating haematologist in Pakistan.

Section 8. Common questions about Roctavian in Pakistan

Will Adamjee, Jubilee, EFU, or State Life cover this? A USD 2.9 million one-time gene therapy is not a standard reimbursable line item for any Pakistani private insurer. Each insurer

assesses named-patient claims case by case, but coverage of a gene therapy at this price point is not standard practice in the Pakistani market. We do not promise coverage.

Does Sehat Sahulat cover Roctavian? No. The Rs. 1,000,000 per family per year ceiling does not contemplate gene therapy at this price band. The funding path is cash-pay or family-pooled cash-pay, often supplemented by diaspora contribution from relatives in the Gulf, Europe, or North America.

Can I be infused in Pakistan? No. BioMarin does not support infusion outside a qualified Hemophilia Treatment Centre, and there are no qualified centres in Pakistan. 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 AKUH, PKLI, or Shaukat Khanum haematologist's letter be sufficient? The Pakistani haematologist'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 haematology team performs final pre-infusion confirmation and runs the infusion-day care; the case is genuinely co-managed across borders.

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 Pakistani 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 (Hemlibra)? 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 haematologist's; Reserve Meds does not steer the decision.

How does the Hemophilia Patients Welfare Society Pakistan fit in? HPWS Pakistan supports family education, peer-to-peer connection with other Pakistani families navigating advanced bleeding-disorder care, and logistics planning around the travel-and-infuse arc. Reserve Meds works with HPWS Pakistan and the patient-advocacy networks around the major haematology centres rather than in place of them; the clinical case stays with the treating haematologist and the certified centre.

Section 9. Where Reserve Meds fits in Roctavian cases

Reserve Meds is a US-based concierge coordinator. We do not replace your haematologist, do not replace BioMarin's RoctavianConnect programme, do not replace the certified treatment centre, and do not replace DRAP or the Hemophilia Patients Welfare Society Pakistan. For Roctavian

specifically, the orchestration we provide is eligibility verification coordination between the Pakistani-side haematologist 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 including USD coordination across diaspora-based relatives, and the documentation framework that hands off between the certified centre's haematology team and the Pakistani-side haematology team for post-travel monitoring. 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 Pakistan; durability is framed honestly; no off-label use; no paediatric 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 haematologist has identified Roctavian as a possible option for severe hemophilia A and you are based in Pakistan, 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 haematology centre on the documentation framework. **Reserved for you.**

This guide is informational, not medical or legal advice. The DRAP 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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