

Roctavian

Qatar · access guide

How to access Roctavian from Qatar, the named-patient coordination pathway, 2026

By Reserve Meds · Clinical & regulatory team · Last reviewed 2026-04-23

A Qatar adult with severe Hemophilia A, baseline factor VIII activity below 1% and significant bleed or prophylaxis burden, may be evaluated by their treating haematologist for Roctavian (valoctocogene roxaparvovec). Roctavian is FDA-approved, developed by BioMarin, and is a one-time intravenous adeno-associated virus (AAV5) gene therapy that delivers a functional B-domain-deleted factor VIII transgene to the liver. Because Roctavian is a single-infusion gene therapy with tight eligibility gating and multi-year follow-up, Qatar access typically involves cross-border referral to a gene-therapy-qualified centre rather than a routine drug import.

This guide explains the legal and operational pathway, eligibility gating, what your haematologist needs to coordinate, indicative timing and cost bands, and where Reserve Meds fits in.

The clinical situation

Roctavian is delivered as a single IV infusion at a qualified gene-therapy centre. Eligibility is tightly gated: patients must be AAV5-capsid-antibody-negative on a validated assay (pre-existing AAV5 neutralising antibodies above the threshold exclude treatment), have no history of factor VIII inhibitors, and pass a liver-health workup (ALT/AST bounds, no active HBV or HCV, no advanced fibrosis on elastography, no autoimmune hepatitis). Post-infusion, patients are monitored for transaminitis and typically receive a tapering oral corticosteroid course to protect the transgene. Factor VIII activity, liver function, and AAV safety parameters are tracked for at least five years.

Is Roctavian legally accessible for Qatar patients?

Roctavian cannot be "imported" as a conventional drug for local administration, it is a single-vial one-time product requiring infusion at a gene-therapy-qualified centre with infrastructure for pre-infusion screening, infusion-day management, and multi-year surveillance. Access for Qatar patients typically follows cross-border referral within the Qatar Ministry of Public Health (MOPH) medical-referral framework:

Pattern A, Cross-border referral to an authorised gene-therapy centre. The patient travels to a qualified international centre (in the US, Europe, or a partnering Middle-East tertiary centre operating under BioMarin's qualified-treatment-centre model) for eligibility workup, infusion, and early post-infusion monitoring.

Pattern B, Hybrid coordination with a Qatar tertiary centre. Where a Qatar centre participates in the manufacturer's qualified-treatment-centre network, portions of screening and long-term follow-up may be localised.

The MOPH medical-referral framework supports both patterns, with documentation covering medical necessity, treatment-centre identification, and the return-to-home surveillance plan.

How the pathway works, step by step

1. **Consultation with your haematologist.** Bleed history, prior prophylaxis or on-demand regimen, inhibitor history, and liver status.
2. **Eligibility workup.** AAV5 capsid antibody titre, factor VIII inhibitor assay, hepatic panel with elastography, hepatitis serology, autoimmune hepatitis screen.
3. **Treatment-centre identification.** Reserve Meds coordinates referral to a qualified gene-therapy centre.
4. **MOPH medical-referral dossier.** Filed by your physician with supporting clinical rationale.
5. **Cross-border travel and infusion.** Single IV infusion at the qualified centre; early post-infusion monitoring and corticosteroid management.
6. **Handover to Qatar haematology team.** Structured multi-year surveillance plan covering factor VIII activity, LFTs, and AAV safety parameters.

What documentation your physician needs

Your physician will typically need to provide:

- Clinical rationale letter with diagnosis, bleed phenotype, prior prophylaxis, inhibitor history, and Roctavian as the indicated therapy
- Verification of their Qatar medical licence
- AAV5 capsid antibody, factor VIII inhibitor, and hepatic workup results
- Identification of the qualified treatment centre and the referral plan
- Long-term surveillance plan for return to Qatar

Reserve Meds provides a coordination kit bundling the templates MOPH reviewers and qualified treatment centres expect to see for cross-border gene-therapy referrals.

Costs and timing

Roctavian's US list price for the one-time product is indicatively around USD 2.9 million; total cost of care, including pre-infusion workup, infusion-day services, inpatient and outpatient monitoring, and caregiver travel, runs materially higher at a US qualified centre. Reserve Meds issues a transparent all-in quote at intake. Because this is a one-time therapy rather than recurring factor VIII prophylaxis, families often model the cost against a lifetime of factor VIII or factor-mimetic spend with their haematologist and financial advisor.

Indicative timing from intake to infusion typically runs 10-20 weeks, driven by eligibility workup, treatment-centre calendar, and travel coordination. Early post-infusion monitoring typically requires several weeks at or near the qualified centre.

Fulfillment availability is limited to our first cohort, and all timelines published on this site are indicative. If your clinical situation is time-sensitive, tell us at intake. We triage accordingly.

Reserve Meds's role

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine and gene therapy. For Roctavian specifically, we provide:

- **Treatment-centre referral.** Coordination with authorised Roctavian qualified-treatment centres.
- **Documentation.** Cross-border referral and MOPH named-patient package.
- **Logistics.** Patient and caregiver travel, accommodation, and post-infusion return-home planning.
- **Concierge case lead.** A named point of contact throughout the gene-therapy journey.

What we do not do: we are not the prescriber, we do not practise medicine, we do not manufacture the product, and we are not the treatment centre. All clinical decisions remain with your treating haematologist and the qualified centre.

Frequently asked

Is this legal in Qatar? Yes, when executed through the MOPH medical-referral framework with a qualified treatment centre. See our trust and compliance page.

What if my AAV5 antibody titre is positive? Pre-existing AAV5 neutralising antibodies above the manufacturer's threshold typically exclude a patient from Roctavian. Your haematologist will review alternatives such as factor VIII prophylaxis or emicizumab.

Will I still need factor VIII prophylaxis afterward? Clinical trial data show many treated patients experience meaningful factor VIII expression with reduced bleed rates and reduced or discontinued prophylaxis. Durability varies and is tracked by serial factor VIII activity measurements.

What about long-term safety? Multi-year surveillance is a core part of the protocol, including liver function monitoring. Your haematology team will explain the full risk profile.

Will private insurance apply? Some Qatar private insurers reimburse named-patient coordination on a case-by-case basis; we supply documentation for submission but do not process insurance claims directly.

Reserve Meds's role

US-based concierge coordinator for cross-border specialty medicine. We are not the prescriber, not the dispensing pharmacy, and not the manufacturer. All clinical decisions remain with your treating physician.

Reserve Meds

reserved for you.

Composite case examples. This document is for general information only and does not constitute medical advice. Please consult your treating physician.

Reserve Meds is in pre-launch. Published timelines and cost ranges are indicative, not guarantees.

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