Fabhalta access in Pakistan: the DRAP Special Permission pathway

How patients in Pakistan access Fabhalta (iptacopan) for paroxysmal nocturnal hemoglobinuria, IgA nephropathy and C3 glomerulopathy.

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

1. Quick orientation

Fabhalta is the brand name for iptacopan, an oral small molecule taken twice daily that selectively inhibits factor B in the alternative complement pathway, blocking the pathway upstream of the C3 convertase. By acting proximally, iptacopan controls both intravascular and extravascular hemolysis in paroxysmal nocturnal hemoglobinuria (PNH) and reduces complement-mediated tissue injury in complement-driven kidney diseases. The US Food and Drug Administration approved Fabhalta on December 5, 2023 for adult PNH (the first oral monotherapy approved for this indication), on August 8, 2024 for reduction of proteinuria in adults with primary IgA nephropathy at risk of rapid disease progression (accelerated approval), and on March 20, 2025 for C3 glomerulopathy in adults (the first treatment approved for this rare kidney disease). Novartis is the US marketing authorisation holder. There is no DRAP registration for Fabhalta in Pakistan as of this page's review date, and the product is not in routine commercial stocking. Pakistani patients whose treating hematologist or nephrologist has confirmed an indication-matched diagnosis reach Fabhalta through the Drug Regulatory Authority of Pakistan (DRAP) Special Permission / Personal Use Import No Objection Certificate, with mandatory FABHALTA REMS vaccination documentation as the gating step. Reserved for you.

2. Why Pakistan patients need Fabhalta via the named-patient pathway

Three patterns of access gap apply across Pakistan: a drug is on the DRAP register but the patient's hospital pharmacy does not have it on hand; a drug is registered for a different indication; or a drug is FDA-approved but the manufacturer has not yet completed DRAP registration. Fabhalta sits in the third pattern. Outside the US, the EU, and the UK (and Japan for C3 glomerulopathy), Fabhalta has no local marketing authorisation in major MENA, Gulf, South Asian, or East Asian markets. There is no local-agent supply route for Pakistani patients.

Four structural reasons drive Fabhalta to the named-patient channel for Pakistani patients. First, the international registration gap is absolute in the SAARC region. Second, the underlying diseases are ultra-rare or under-served. PNH affects roughly 1 to 2 per million population. C3 glomerulopathy is rarer still. IgA nephropathy is more common but historically had limited disease-modifying options, and Fabhalta is the first complement-targeted therapy approved for it. National payers and commercial entities have limited incentive to expedite local registration for such small patient pools. Third, the patient populations are highly motivated. Pakistani patients facing chronic transfusion dependence in PNH or progressive kidney decline in IgA nephropathy and C3G are well-informed through international advocacy networks (Aplastic Anemia and MDS International Foundation, NephCure, IgA Nephropathy Foundation) and seek the first oral monotherapy across borders, often via family connections in the Gulf or the UK who first identify the option. Fourth, the modality is logistically simple. Oral capsules, room temperature, no cold chain. The complexity is regulatory (REMS vaccination, DRAP filing) rather than physical.

3. The DRAP Special Permission pathway for Fabhalta

DRAP regulates the import of medicines through the Quality Assurance and Laboratory Testing (QA&LT) Division's Import and Export Section, with Drug Registration Board oversight for new product registration matters. For unregistered

medicines required by a specific patient, DRAP issues a Special Permission, also known as the Personal Use Import No Objection Certificate (NOC). The framework covers Personal Use Import by an individual patient on physician prescription and Special Permission for Import of Unregistered Therapeutic Goods by hospitals or institutions, filed through DRAP's Online Import and Export System (OIES) electronic portal.

A complete DRAP application for Fabhalta includes the clinical justification letter from the treating hematologist (PNH cases) or nephrologist (IgA nephropathy and C3G cases); the treating physician's PMDC license verification with specialist registration in hematology or nephrology; the patient identifier (CNIC); full product details (Fabhalta, iptacopan, Novartis Pharmaceuticals Corporation, 200 mg hard gelatin capsules in bottles of 60 capsules per 30-day supply at the approved twice-daily regimen, requested quantity covering the planned initiation and refill cadence for chronic indefinite therapy); the destination dispensing facility license; the manufacturer or authorised distributor letter confirming legitimate supply chain sourcing through Novartis's REMS-certified US specialty pharmacy partners (Biologics by McKesson and Onco360); and a chain-of-custody plan for ambient shipment.

The clinical justification angle for Fabhalta turns on two simultaneous requirements: indication-matched diagnostic confirmation and FABHALTA REMS vaccination documentation. The treating Pakistani specialist documents the diagnosis with explicit reference to indication-specific evidence (flow cytometry confirming GPI-deficient cell population for PNH; kidney biopsy with mesangial IgA deposition and proteinuria threshold documentation for IgA nephropathy at risk of rapid disease progression; kidney biopsy with C3-dominant deposition pattern for C3G). The letter then documents the patient's vaccination history against encapsulated organisms required by the FABHALTA REMS: meningococcal (ACWY and B), pneumococcal, and Haemophilus influenzae type b vaccines per the most current ACIP recommendations for patients receiving complement inhibitors, with completion at least two weeks before initiating therapy or appropriate antibiotic prophylaxis if the risk of delaying therapy outweighs the infection risk. Routine DRAP personal-use cases typically clear in four to eight weeks; complex first-import complement-inhibitor cases with REMS vaccination coordination can extend to ten to sixteen weeks.

4. Where Fabhalta gets dispensed in Pakistan

Fabhalta is an oral hard gelatin capsule supplied in 200 mg strength in bottles of 60 capsules (a 30-day supply at the approved twice-daily regimen), stored at controlled room temperature (20 to 25 degrees Celsius) with permitted excursions to 15 to 30 degrees Celsius. Shipping does not require cold-chain validation, temperature monitoring, or insulated packaging. There is no reconstitution step. The product is not a controlled substance and is not scheduled under the US Controlled Substances Act. The dispensing requirement is therefore a DRAP-licensed hospital outpatient pharmacy aligned with a hematology service (for PNH) or nephrology service (for IgA nephropathy and C3G) capable of REMS-equivalent vaccination coordination, complement-inhibitor infection surveillance, and the indication-specific biomarker monitoring (hemoglobin, LDH, and reticulocyte count for PNH; proteinuria and eGFR for the kidney indications).

Pakistani institutions with adult hematology or nephrology services that handle named-patient imports as routine workflow include Aga Khan University Hospital (AKUH) in Karachi, with both hematology and nephrology subspecialty programs and a 24/7 pharmacy network with temperature-controlled storage; Shaukat Khanum Memorial Cancer Hospital and Research Centre (SKMCH&RC) in Lahore for hematology cases; Pakistan Kidney and Liver Institute (PKLI) in Lahore for nephrology cases, with kidney transplant integration; Indus Hospital and Health Network in Karachi with strong hematology and nephrology capability; Liaquat National Hospital in Karachi; Combined Military Hospitals (CMH) in Rawalpindi and Lahore for military and civilian referrals; and Shifa International Hospital in Islamabad. Vaccination coordination is straightforward at these institutions (and at most major Pakistani vaccination clinics) for the meningococcal, pneumococcal, and Hib protocols the FABHALTA REMS requires. For families whose treating specialist is at a smaller hospital, the practical route is to coordinate with one of the above centres as the dispensing facility.

5. Real cost picture for Fabhalta in Pakistan

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, with annual CPI inflation rising to 10.9 percent in April 2026. Reserve Meds quotes in USD and accepts wire transfers from any USD-accessible source. Three line items frame the cost.

First, drug cost. The US wholesale acquisition cost for Fabhalta at the approved 200 mg twice-daily regimen is approximately USD 566,500 per year, with the WAC per 30-day fill reported at approximately USD 46,562 as of March 2025, equivalent to roughly PKR 13 million per 30-day pack and PKR 158 million per annual course at the May 2026 exchange rate. This is the manufacturer-stated US list price before payer negotiation. Novartis US patient services and copay support are US-only and do not extend to international named-patient cases.

Second, international logistics. Fabhalta is room-temperature stable and ships under standard pharmaceutical-grade ambient conditions with DSCSA-compliant serialisation. International logistics for an ambient shipment to Karachi or Lahore typically runs USD 400 to USD 800 per shipment. Cold-chain insurance riders are not required. The principal logistics risk is regulatory (export documentation, REMS-channel sourcing, country NPP filing) rather than physical.

Third, regulatory, vaccination, and coordination. DRAP documentation handling fees, FABHALTA REMS vaccination coordination (if not already completed), FBR Customs clearance, and Reserve Meds' concierge fee are itemised separately. On the insurance side, Adamjee, Jubilee, EFU, State Life, IGI, and Pak-Qatar Family Takaful rarely cover ultra-rare-disease imports of this kind outside formulary. Sehat Sahulat's Rs. 1,000,000 per family per year ceiling is well below the Fabhalta annual list price. The realistic operating default is cash-pay with annual planning from the first case, and Pakistani families frequently fund chronic complement-inhibitor therapy through pooled household resources including remittances from relatives in the GCC, UK, US, and Canada.

6. Typical timeline for Fabhalta in Pakistan

The DRAP timeline for routine Personal Use Import cases runs four to eight weeks from a complete submission; complex first-import complement-inhibitor cases requiring REMS vaccination verification can extend to ten to sixteen weeks. Vaccination is the dominant intake-stage friction point because it must be completed at least two weeks before initiation. Fabhalta is an ambient oral capsule, so cold-chain transit time does not apply. End-to-end, a typical Fabhalta case in Pakistan runs as follows: 24 to 48 hours from intake to eligibility confirmation by Reserve Meds; one to four weeks for the treating specialist and the local vaccination clinic to complete the meningococcal ACWY and B, pneumococcal, and Hib protocol if not already in place; three to seven days for the treating specialist and the dispensing hospital's import pharmacy to assemble the application with indication-specific biomarker documentation and REMS vaccination records; four to eight weeks for DRAP review through the OIES portal; three to five days for US sourcing through Biologics by McKesson or Onco360 under DSCSA-compliant chain-of-custody and Novartis's REMS-certified channel; one to three days for qualified ambient shipment; two to four days for FBR Customs clearance; and final receipt and release at the dispensing pharmacy. Because Fabhalta is dosed twice daily on a chronic indefinite basis, Reserve Meds plans repeat-shipment cadence and annual budget conversations at the case-acceptance stage rather than treating each 30-day bottle as a one-off.

7. What your physician needs to provide

The clinical justification letter is the cornerstone of the DRAP application. The treating Pakistani hematologist (PNH) or nephrologist (IgA nephropathy, C3G) documents the indication-specific diagnosis with ICD-10 coding and explicit reference to the supporting evidence: flow cytometry confirming GPI-deficient cell population and percentage for PNH; kidney biopsy with mesangial IgA deposition pattern and proteinuria threshold documentation indicating risk of rapid disease progression for IgA nephropathy; kidney biopsy with C3-dominant deposition pattern for C3G. The letter states the planned dosing regimen (200 mg orally twice daily, approximately 12 hours apart, continuous with or without food, no loading dose, chronic indefinite duration as long as the patient is benefiting and tolerating therapy under specialist supervision); confirms vaccination status against meningococcal (ACWY and B), pneumococcal, and Hib organisms per the most current ACIP recommendations for patients receiving complement inhibitors, with documented completion at least two

weeks before initiation or the approved alternative of appropriate antibiotic prophylaxis when delay risk outweighs infection risk; and describes the monitoring plan.

The monitoring stack includes patient education on infection-warning-sign recognition with a patient safety card carried at all times; clinical monitoring for signs of serious infection throughout therapy with prompt evaluation of any fever or systemic symptom; baseline and periodic liver function tests (ALT, AST, total bilirubin); lipid monitoring per clinician judgement; CYP2C8 interaction review (avoid gemfibrozil) and CYP3A4 inducer review (avoid strong inducers); and indication-specific biomarker monitoring (hemoglobin, LDH, reticulocyte count, and transfusion-status review for PNH; urine protein-to-creatinine ratio and eGFR for IgA nephropathy and C3G).

The letter is co-filed with the physician's PMDC license verification, the institutional pharmacy license, the requested pack count and refill plan for chronic indefinite therapy, the vaccination record documentation, and the chain-of-custody plan for the ambient shipment. Post-import, the treating physician and dispensing pharmacy commit to adverse-event reporting through the DRAP Pharmacovigilance Centre with explicit infection-reporting protocol.

8. Common questions about Fabhalta in Pakistan

Is the vaccination requirement non-negotiable? Yes. Vaccination against meningococcal (ACWY and B), pneumococcal, and Hib organisms is both a FABHALTA REMS requirement and a label-mandated prerequisite. The label permits initiation before completion of vaccinations only when the risk of delay outweighs the infection risk, in which case appropriate antibiotic prophylaxis must be considered. Reserve Meds requires documentation of the treating physician's vaccination plan before coordinating supply.

Will Adamjee, Jubilee, EFU, or State Life cover Fabhalta? Coverage for named-patient ultra-rare-disease imports is uncommon across Pakistani health plans, particularly given the annual list price of roughly USD 566,500 and the chronic indefinite nature of therapy. Some plans pay a partial percentage on a case-by-case basis. We supply the documentation; the claim is yours or your hospital's to file. The realistic default is cash-pay with annual planning.

How does Sehat Sahulat interact with named-patient Fabhalta imports? The Sehat Sahulat Program's Rs. 1,000,000 per family per year ceiling does not stretch to cover Fabhalta at the US WAC price point. Patients can still use Sehat Sahulat for hospitalisation, transfusion support, and dialysis where applicable while Fabhalta is procured separately on cash-pay.

Will my PMDC-licensed hematologist's or nephrologist's letter be sufficient if DRAP queries the case? Yes. PMDC-licensed hematologists and nephrologists at AKUH, SKMCH&RC, PKLI, Indus Hospital, Liaquat National, CMH, and Shifa International all have signing authority on Personal Use Import applications. DRAP may request additional clarification on the indication-specific biomarker confirmation or the REMS vaccination plan; the treating specialist answers those queries directly.

What is the safety profile I should know about? Fabhalta carries a boxed warning for serious infections caused by encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis, Haemophilus influenzae type b). Serious infections occurred in 6.7 percent of iptacopan-treated patients versus 2.1 percent on placebo in APPLAUSE-IgAN. The most common adverse reactions include headache, nasopharyngitis, diarrhea, abdominal pain, bacterial infection, nausea, and elevated lipids. Liver function abnormalities warrant baseline and periodic monitoring. The FABHALTA REMS is mandatory and is the principal safety architecture for the therapy.

Why this drug versus an anti-C5 antibody in PNH? The decision is made by the treating hematologist. APPLY-PNH demonstrated that two-thirds of patients with residual anemia on eculizumab or ravulizumab achieved hemoglobin of 12 g/dL or higher without transfusion after switching to iptacopan, versus zero in the control arm. APPOINT-PNH showed 92.2 percent of treatment-naive patients achieved transfusion independence at 24 weeks. The oral route also avoids chronic intravenous or subcutaneous administration. Reserve Meds does not make this clinical recommendation.

9. Where Reserve Meds fits in Fabhalta cases

Reserve Meds is a US-based concierge coordinator. We do not replace your treating hematologist or nephrologist, DRAP, the dispensing hospital pharmacy, your local vaccination clinic, or your insurer. What we do for a Fabhalta case is verify

eligibility within 24 to 48 hours; supply your physician's team with a documentation kit referencing the FDA prescribing information, the 200 mg twice-daily continuous dosing schedule, the indication-specific biomarker framing (flow cytometry for PNH; kidney biopsy and proteinuria documentation for IgA nephropathy and C3G), and the FABHALTA REMS vaccination protocol; coordinate the documentation of meningococcal ACWY and B, pneumococcal, and Hib vaccination completion (or the physician's appropriate antibiotic prophylaxis plan); align US-side sourcing through Novartis's REMS-certified specialty pharmacy partners Biologics by McKesson and Onco360 under DSCSA-compliant chain-of-custody; coordinate ambient shipment with a qualified specialty 3PL; and provide a single named Patient Concierge Coordinator across repeat shipments and chronic refill cadence. Because Fabhalta is logistically simple but document-heavy on the regulatory side (REMS vaccination evidence, indication-specific diagnostic confirmation, country-specific NPP filing), the case complexity sits in the front-end documentation choreography. No prior Reserve Meds case experience predates this page; standard NPP coordination applies.

10. Next step

If your Pakistani hematologist or nephrologist has confirmed PNH, IgA nephropathy at risk of rapid progression, or C3 glomerulopathy and recommends Fabhalta, start the request and we will reach out within 24 to 48 hours.

Reserved for you.

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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