Fabhalta access in the UAE: the MOHAP and EDE named-patient pathway

How patients in the United Arab Emirates access Fabhalta (iptacopan) for paroxysmal nocturnal hemoglobinuria, primary IgA nephropathy, or C3 glomerulopathy, with REMS vaccination prerequisites documented in the case file.

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1. Quick orientation

Fabhalta is the brand name for iptacopan, an oral small-molecule selective inhibitor of factor B in the alternative complement pathway, developed and commercialised by Novartis Pharmaceuticals. The US Food and Drug Administration approved Fabhalta on 5 December 2023 for adults with paroxysmal nocturnal hemoglobinuria (PNH) (the first oral monotherapy approved for that indication), on 8 August 2024 under accelerated approval for adults with primary IgA nephropathy (IgAN) at risk of rapid disease progression, and on 20 March 2025 for adults with C3 glomerulopathy (C3G) (the first treatment approved for that rare kidney disease). In the United Arab Emirates, Fabhalta has no local MOHAP marketing authorisation as of the date of this page. UAE patients meeting any of the three FDA-approved indications reach the medicine through the federal unregistered-medicine import permit administered by MOHAP and, from 29 December 2025, through the Emirates Drug Establishment (EDE) portal at ede.gov.ae, with documented completion or appropriate timing of REMS-mandated vaccinations against encapsulated organisms before initiation. Reserved for you.

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

The UAE pharmaceutical regulatory framework recognises three structural access gaps: registered but not stocked, registered for a different indication, and not registered locally at all. Fabhalta sits in the third pattern. Outside the United States, EU, UK, and Japan (for C3G), Fabhalta has no local marketing authorisation in major MENA, GCC, South Asian, or East Asian markets. There is no local-agent supply route for UAE patients as of this page's review date.

Four factors converge to route UAE Fabhalta access through the named-patient pathway. First, the international registration gap is wide. The European Medicines Agency granted centralised marketing authorisation on 24 May 2024 for PNH, with subsequent EMA approvals for C3G; UK MHRA approval for PNH was granted via the international recognition procedure following the EMA decision; Japan's PMDA granted approval for C3G in 2025. No GCC or MENA market has local marketing authorisation. Second, the underlying diseases are ultra-rare or under-served. PNH affects roughly one to two per million population. C3 glomerulopathy is rarer still. IgA nephropathy is more common but has historically had limited disease-modifying options, and Fabhalta is the first complement-targeted therapy approved for it. National payers in non-approved jurisdictions have limited incentive to expedite local registration for such small patient pools. Third, UAE patient populations are highly motivated. Patients facing chronic transfusion dependence (PNH) or progressive kidney decline (IgAN, C3G) are well-informed via international advocacy networks (the Aplastic Anemia and MDS International Foundation, NephCure, the IgA Nephropathy Foundation) and seek the first oral monotherapy across borders. Fourth, the modality is logistically simple. Oral capsules, room temperature, no cold chain. The complexity is regulatory (REMS vaccination, country NPP filing) rather than physical.

3. The MOHAP and EDE named-patient pathway for Fabhalta

The federal pathway for a UAE-licensed physician to obtain Fabhalta is the unregistered-medicine import permit, historically administered by MOHAP and, from 29 December 2025, through the EDE portal under Federal Decree-Law No. 38 of 2024. The framework allows hospitals and licensed pharmaceutical establishments to import a specific medicine for a

specific patient when the medicine is approved by a recognised reference authority (the US FDA, EMA, MHRA, PMDA Japan, or Health Canada) and a clinically equivalent locally registered alternative is not suitable. Compassionate-use provisions in the UAE legal framework apply.

A complete application typically includes:

- A clinical justification letter from the treating UAE hematologist (PNH) or nephrologist (IgAN, C3G) naming Fabhalta and the indication
- The treating physician's UAE medical license verification (MOHAP, DHA, DOH, or Sharjah Health Authority, depending on practice location)
- An anonymised patient identifier where the EDE submission allows
- Indication-specific diagnostic confirmation: flow cytometry confirming PNH clone for PNH, kidney biopsy confirming IgA nephropathy for IgAN, kidney biopsy confirming C3 glomerulopathy for C3G
- Full product details: Fabhalta, iptacopan, 200 mg hard gelatin capsules, manufacturer Novartis Pharmaceuticals, bottles of 60 capsules (a 30-day supply at the approved twice-daily regimen)
- The destination dispensing hospital outpatient pharmacy name, license number, and pharmacy in charge
- A chain-of-custody plan describing how the ambient capsules will move from the US REMS-certified specialty pharmacy through the importer to the dispensing pharmacy
- REMS vaccination documentation (see the dedicated paragraph below)

The clinical-justification angle for Fabhalta is the combination of indication-specific diagnostic confirmation and the REMS vaccination prerequisite. The FABHALTA REMS programme is mandatory in the United States because of the boxed warning for serious infections caused by encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis, Haemophilus influenzae type b). Reserve Meds requires documentation that the treating UAE physician has confirmed completion or appropriate timing of meningococcal (ACWY and B), pneumococcal, and Haemophilus influenzae type b vaccination per the most current ACIP recommendations for patients receiving complement inhibitors, before coordinating supply. 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 and the rationale is documented in the case file. Approval timelines for routine UAE EDE cases are typically 5 to 15 business days; first imports of Fabhalta into a given facility can extend toward the upper end of that range.

4. Where Fabhalta gets dispensed in the UAE

Fabhalta is an oral capsule, room-temperature stable, not requiring infusion infrastructure or cold-chain storage. The dispensing requirement is a UAE-licensed hospital outpatient pharmacy or specialised import pharmacy, paired with a UAE-licensed hematologist (for PNH cases) or nephrologist (for IgAN and C3G cases) supervising the case. Because the REMS vaccination prerequisite and the boxed warning for ser