

## Pyrukynd

Qatar · access guide

# How to access Pyrukynd from Qatar, the named-patient import pathway, 2026

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

A Qatar patient with pyruvate kinase (PK) deficiency may receive a prescription for Pyrukynd (mitapivat) from their treating haematologist. Pyrukynd is FDA-approved for haemolytic anaemia in adults with PK deficiency, and it is manufactured by Agios Pharmaceuticals. It is the first disease-modifying therapy approved for PK deficiency, a rare, inherited chronic haemolytic anaemia caused by PKLR gene mutations that impair erythrocyte glycolysis. Pyrukynd is a small-molecule PK activator that restores glycolytic function in red blood cells. In Qatar, Pyrukynd may not yet be broadly registered, which is why your haematologist may be navigating a named-patient import pathway with you.

This guide explains the legal pathway, what documentation your physician needs, typical costs and timing, and where Reserve Meds fits in.

## The clinical situation

Pyrukynd is an oral tablet taken twice daily, with dose titration over several weeks based on tolerability and haemoglobin response. Eligibility requires genetically or enzymatically confirmed PK deficiency and clinical evidence of chronic haemolytic anaemia (baseline haemoglobin, transfusion history, bilirubin, reticulocyte count, LDH). Your treating haematologist confirms diagnosis and the titration plan per FDA labeling. Pyrukynd carries warnings about acute haemolysis with abrupt interruption or discontinuation.

## Is Pyrukynd legally importable into Qatar?

Yes, through the Qatar Ministry of Public Health (MOPH) named-patient framework, with parallel authority through DoH Abu Dhabi and DHA Dubai depending on the prescribing facility. The pathway allows a Qatar-licensed physician to import a medicine not locally registered when: (a) the medicine is approved by a recognised reference authority (FDA qualifies), (b) no locally available alternative is clinically equivalent for the patient, (c) the physician takes clinical responsibility, and (d) chain of custody is documented.

For mitapivat specifically, the application is routine, an oral tablet with standard room-temperature handling and no REMS or controlled-substance complexity.

## How the pathway works, step by step

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1. **Consultation with your treating haematologist.** PK deficiency diagnosis (genetic testing of PKLR or enzyme activity assay), baseline haemolysis labs, and the clinical rationale for Pyrukynd.
2. **MOPH / DoH / DHA named-patient application.** Your physician files the application including clinical letter, patient identifier, and product details.
3. **US-side sourcing.** Reserve Meds coordinates with our US-licensed specialty wholesale partner.
4. **Shipment.** Pyrukynd ships at controlled room temperature; no cold-chain is required.
5. **Arrival and titration start.** The treating haematologist initiates and supervises the titration schedule.

## What documentation your physician needs

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Your physician will typically need to provide:

- Clinical rationale letter confirming PK deficiency diagnosis with PKLR genotype or enzyme activity evidence, baseline haemolysis labs, and Pyrukynd as the indicated treatment
- Verification of Qatar medical licence
- Patient identifier
- Planned titration schedule and projected tablet supply
- Plan for response monitoring (haemoglobin, haemolysis markers)

Reserve Meds provides a physician documentation kit that bundles the templates MOPH reviewers expect to see for rare-haemolytic-anaemia therapy.

## Costs and timing

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Pyrukynd's US cash-pay drug-only reference price sits in a broad indicative range, the annualised treatment cost falls in the USD 300,000-400,000 range in US list pricing. International logistics, MOPH documentation, and concierge coordination add incremental cost; Reserve Meds issues a full transparent quote at the start of intake. Indicative range.

Indicative timing for the first dispensed supply after cohort intake opens is 7-14 days from the moment a complete application is submitted. Titration runs over several weeks, so your physician will plan multi-month supply accordingly.

*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

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

- **Sourcing.** Through our US-licensed specialty wholesale partner, operating under DSCSA chain-of-custody.
- **Documentation.** Regulatory package for your physician and for MOPH/DoH/DHA review.
- **Logistics.** Controlled-room-temperature shipment.
- **Concierge case lead.** A named point of contact.

**What we do not do:** We are not the prescriber. We do not practise medicine. We are not the dispensing pharmacy. All clinical decisions remain with your treating haematologist.

## Frequently asked

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**Is this legal in Qatar?** Yes, when executed through the MOPH named-patient framework with appropriate documentation.

**Does every PK-deficiency patient respond?** No. Response is typically assessed over several titration cycles; roughly 30-40% of non-transfusion-dependent patients achieved the primary haemoglobin-response endpoint in the pivotal trial. Your haematologist guides response assessment and dose adjustment.

**What about sudden discontinuation?** Abrupt interruption can cause acute haemolysis. Your haematologist manages any dose interruption carefully.

**How does this compare with splenectomy or supportive care?** Supportive care (transfusions, iron-overload monitoring, folate supplementation) and splenectomy have historically been the mainstays of PK-deficiency management. Pyrukynd is the first targeted disease-modifying therapy and is often considered alongside these conventional approaches; your haematologist weighs the full clinical picture.

**Will private insurance cover this?** Cash-pay is the default. Some Qatar private insurers reimburse rare-haemolytic-anaemia therapy on escalated review; we supply documentation 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.

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### **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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