**Crysvita**

Qatar · access guide

# How to access Crysvita for X-linked hypophosphatemia or tumor-induced osteomalacia from Qatar: 2026 pathway via Sidra Medicine paediatric endocrinology, Hamad Medical Corporation adult metabolic bone, and oncology coordination

*By Reserve Meds clinical & regulatory team. Last reviewed 2026-05-20.*

Qatar has the deepest paediatric rare-disease and genetics infrastructure in the Gulf, anchored at Sidra Medicine Doha. Sidra Medicine paediatric endocrinology with the genetics depth IS THE NATURAL paediatric centre for Crysvita cases in Qatar: the FDA paediatric XLH label down to age 6 months, the PHEX-sequencing capability, the established paediatric metabolic-bone clinic, and the multidisciplinary genetics service make Sidra the destination of choice for paediatric XLH in the country and across the region. Hamad Medical Corporation (HMC) endocrinology handles adult XLH and adult TIO. The National Center for Cancer Care and Research (NCCCR) at HMC handles oncology coordination for TIO tumor localisation and resection. The Ministry of Public Health (MOPH) governs imported-medicine registration. Crysvita (burosumab-twza, Ultragenyx Pharmaceutical with Kyowa Kirin as ex-US partner) is the anti-FGF23 humanized IgG1 monoclonal antibody, dosed subcutaneously every 2 to 4 weeks.

For a Qatar-resident child age 6 months and older with genetic or biochemical XLH, an adult with XLH continuing into adulthood, or an adult with TIO awaiting or following tumor resection, the operational reality is that Qatar can run the entire case end-to-end in country: paediatric XLH at Sidra Medicine with genetic confirmation, adult XLH at HMC endocrinology, and adult TIO at HMC with NCCCR oncology coordination for tumor localisation. Cross-border referral is not the default expectation; the prescribing-physician relationship is locally anchored.

This page explains how the pathway works in 2026 for a Qatar-resident patient: who qualifies, where the prescribing paediatric endocrinologist or adult metabolic bone specialist conversation happens, how Crysvita is dispensed and stored, what the dose-titration rhythm looks like over the first year, what the cost band is in QAR, and how the chronic-treatment course fits into a Qatari family's routine.

## Why Crysvita, and why now

Crysvita is burosumab-twza, a humanized IgG1 monoclonal antibody that binds and neutralises fibroblast growth factor 23 (FGF23). In XLH, an inactivating mutation in the PHEX gene on the X chromosome causes circulating FGF23 to be inappropriately elevated. Excess FGF23 reduces phosphate reabsorption at the renal proximal tubule and suppresses renal 1-alpha-hydroxylase, leading to chronic phosphate wasting, low serum phosphorus, low active 1,25-dihydroxyvitamin D, defective bone mineralisation, paediatric rickets, short stature, dental abscess vulnerability, and adult osteomalacia with bone pain, fractures, and enthesopathy. In TIO, a mesenchymal phosphaturic tumor secretes FGF23 ectopically and produces the same biochemical and skeletal picture in an adult.

The historic conventional therapy was lifelong high-dose oral phosphate salts combined with active vitamin D analogs (calcitriol or alfacalcidol). Conventional therapy is partially effective and does not address the underlying FGF23 excess. Crysvita addresses the upstream mechanism: serum phosphorus moves toward the lower-normal range within 4 to 8 weeks; paediatric radiographic rickets scores improve over 1 to 2 years; adult bone pain reduces over months. The FDA approved Crysvita for paediatric XLH age 1 year and older in April 2018, for adult XLH in September 2018, expanded paediatric XLH to age 6 months in March 2020, and added TIO age 2 years and older in June 2020.

Reserve Meds does not advocate Crysvita over conventional therapy in cases where conventional response is adequate. The page describes the Crysvita pathway because Crysvita is the therapy the family has asked about.

## What Crysvita is, in plain language

Crysvita is a subcutaneous injection given every 2 to 4 weeks. There is no infusion centre, no inpatient stay. After a supervised first dose at Sidra Medicine paediatric endocrinology or HMC adult endocrinology, the family or patient may be trained for home self-injection, although many Qatari families prefer clinic-administered dosing during the titration phase. The vials are 10 mg, 20 mg, and 30 mg single-dose presentations; the dispensed dose is weight-based and titrated by serial phosphorus measurement. Paediatric XLH starting dose is 0.4 to 0.8 mg per kg every 2 weeks. Adult XLH dosing is 1 mg per kg every 4 weeks, capped at 90 mg. TIO dosing is weight-based every 2 weeks.

This is not a short-course therapy. XLH is a lifelong genetic condition; Crysvita is taken for as long as it controls the phosphate-wasting biochemistry. TIO patients may discontinue if and when the underlying tumor is fully localised and resected.

## Eligibility at Sidra Medicine paediatric endocrinology or HMC adult metabolic bone clinic

For Qatar-resident patients, the services apply the FDA-label and EMA-label eligibility:

1. Confirmed diagnosis. For XLH: genetic confirmation of a PHEX mutation (Sidra Medicine genetics service handles paediatric PHEX sequencing in country), OR a clinically compatible picture (low serum phosphorus, normal serum calcium, elevated alkaline phosphatase, elevated FGF23, low or low-normal 1,25-dihydroxyvitamin D) with a positive family history. For TIO: an adult with acquired hypophosphatemia, elevated FGF23, NCCCR oncology team coordination for tumor localisation (Ga-68 DOTATATE or octreotide-based functional imaging plus anatomic localisation at HMC radiology), and a resection plan. 2. Age. Paediatric XLH age 6 months and older (Sidra paediatric endocrinology). Adult XLH age 18 and older (HMC adult endocrinology). TIO age 2 and older. 3. Baseline biochemistry. Serum phosphorus, calcium, alkaline phosphatase, 1,25-dihydroxyvitamin D, 25-hydroxyvitamin D, intact PTH, urine phosphate, creatinine and eGFR. 4. Discontinuation plan for conventional therapy. Oral phosphate supplements and active vitamin D analogs must be discontinued before Crysvita is started. This is essential. 5. Renal imaging baseline. Renal ultrasound. 6. Hypersensitivity history review. 7. Pregnancy planning discussion for women of childbearing potential.

A Qatari family should arrive at the prescribing conversation with: Sidra Medicine paediatric endocrinology or HMC adult metabolic bone documentation, the Sidra genetics service PHEX sequencing report if completed or the family-history pedigree, the most recent biochemistry panel, the radiographic rickets score documentation or skeletal survey, the complete conventional therapy history, and MOPH / insurance paperwork.

## The Qatar prescribing and supply picture, plainly

Crysvita MOPH registration status is verified at intake. Ultragenyx commercial supply runs through regional distributors. Where in-country registration is complete, in-country pharmacy dispensing applies. Where registration has not yet caught up, the named-patient European-import pathway covers the case. The pathway is:

1. **Prescribing physician**: at Sidra Medicine paediatric endocrinology for paediatric XLH and paediatric TIO; at HMC adult endocrinology for adult XLH and adult TIO. The Sidra paediatric endocrinology and genetics service IS THE NATURAL centre for paediatric Crysvita in Qatar (≥6 months XLH label) and for the wider region's complex paediatric rare-disease referrals. 2. **Pharmacy dispensing**: Sidra Medicine pharmacy or HMC pharmacy with cold-chain refrigeration. Crysvita must be stored at 2 to 8 degrees Celsius; do not freeze; protect from light. 3. **Insurance preauthorisation**: For Qatari nationals, MOPH-administered National Health Insurance and the institutional rare-disease pathway at Sidra and HMC apply. Commercial insurers handle expatriate cases with confirmed-diagnosis documentation and prescribing physician rationale. 4. **Conventional therapy discontinuation**: the most important operational gate. The prescribing endocrinologist at Sidra paediatric endocrinology or HMC adult endocrinology sequences discontinuation of oral phosphate supplements and active vitamin D analogs in the days before the first Crysvita dose. Phosphorus and calcium are monitored at baseline, week 2, and serially. 5. **Self-injection or clinic injection training**: typically a supervised first dose at Sidra or HMC, then a training session if the family elects home administration. 6. **Ongoing monitoring**: serum phosphorus, calcium, alkaline phosphatase, 1,25-dihydroxyvitamin D, PTH at week 2, week 4, then monthly during titration, then every 3 months during maintenance. Renal ultrasound annually. Paediatric height and rickets-score reassessment every 6 months.

## Cost band

US WAC pricing is weight-dependent. Paediatric XLH annual band approximately USD 165,000 to 250,000. Adult XLH at 1 mg/kg every 4 weeks (typical dose 70 to 90 mg per cycle) approximately USD 240,000 to 340,000. TIO follows adult XLH range. At 2026 indicative cross rates, the QAR-equivalent annual band is approximately QAR 600,000 to 910,000 paediatric XLH and QAR 875,000 to 1.24 million adult XLH and TIO. MOPH-administered National Health Insurance and Sidra Medicine institutional coverage reduce out-of-pocket exposure substantially for eligible cases.

## What to expect on Crysvita

Serum phosphorus moves toward the lower end of the age-appropriate normal range within 4 to 8 weeks. In paediatric XLH patients, the radiographic rickets score improves over 1 to 2 years, height velocity improves over the first 12 months, and bowing of the lower extremities slowly remodels. In adult XLH patients, bone pain reduces over months, stiffness improves, and stress-fracture healing accelerates. In TIO patients, biochemical correction precedes definitive surgical tumor resection if resection is delayed.

Most common adverse events: injection-site reactions, headache, restless legs symptoms, dizziness, rarely hypersensitivity. Hyperphosphatemia is possible if conventional therapy is not properly discontinued or if dose titration overshoots; serial phosphorus monitoring is the central operational discipline.

## When Crysvita is the wrong drug

Crysvita is the wrong drug for hypophosphatemia that is not FGF23-mediated. It is the wrong drug in severe renal impairment with elevated baseline serum phosphorus, in familial-tumoral-calcinosis-like states, and where the family cannot reliably attend the monthly phosphorus-monitoring visits. For TIO, definitive surgical resection of the localised tumor (NCCCR coordination at HMC) remains the preferred curative pathway.

## What Reserve Meds does on this case

We are a US-based concierge coordinator. We are not the prescriber and not the dispensing pharmacy. On a Qatar Crysvita case we build the documentation pack with the treating Sidra Medicine paediatric endocrinology or HMC adult endocrinology office, confirm MOPH registration status and the appropriate dispensing pathway, run the institutional or insurance preauthorisation conversation, coordinate the cold-chain supply logistics, organise the conventional-therapy discontinuation sequencing, and stay with the case through the first year of titrated dosing. Clinical decisions remain with your treating endocrinologist or metabolic bone team.

***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.

*reserved for you.*

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