Defitelio

Qatar · access guide

How to access Defitelio for severe veno-occlusive disease after stem-cell transplant from Qatar: 2026 pathway via NCCCR adult haematology, Sidra Medicine paediatric HSCT, and KFSHRC Riyadh cross-border referral

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

Qatar's haematopoietic stem-cell transplant (HSCT) infrastructure is concentrated across two centres with very different patient populations. The National Center for Cancer Care and Research (NCCCR) at Hamad Medical Corporation runs the adult haematology and oncology programme; Sidra Medicine Doha runs the paediatric HSCT centre. Most complex adult HSCT cases route cross-border to KFSHRC Riyadh for transplant, with NCCCR managing pre-transplant workup and post-transplant outpatient care. Sidra Medicine Doha is one of the small number of MENA paediatric HSCT centres equipped to manage paediatric severe post-HSCT VOD in-country. Defitelio (defibrotide sodium) is the only FDA-approved therapy for severe hepatic veno-occlusive disease (VOD) with renal or pulmonary dysfunction post-HSCT, and Defitelio is one of the rare adult-and-paediatric drugs where Sidra Medicine IS the relevant Qatar centre, specifically for paediatric HSCT recipients who develop severe VOD. For a Qatari patient who develops severe post-HSCT VOD, the operational question is whether the patient is adult or paediatric (which determines which Qatar centre or which cross-border centre is in scope), whether Defitelio is on hand at the treating centre, and how the inpatient transplant unit team coordinates the q6h infusion schedule.

This page explains how the pathway works in 2026 for a Qatari adult or paediatric patient already inside an HSCT admission or about to be transferred for severe-VOD management: when Defitelio is indicated, who confirms the diagnosis, how the supply pathway works for an indication that is not yet on the MOPH-registered formulary, and what to expect operationally for the 21 to 60 day inpatient course.

Why Defitelio, and when

Defitelio is defibrotide sodium, a purified mixture of single-stranded oligodeoxyribonucleotides extracted from porcine intestinal mucosa. The FDA approved it in March 2016 for adult and paediatric patients aged 1 month and older with hepatic VOD with renal or pulmonary dysfunction following HSCT. Severe post-HSCT VOD is a catastrophic-prognosis condition with day-100 mortality approaching 75 percent in historical control data. The pivotal Phase 3 trial reported day-100 survival of 38.2 percent in the Defitelio arm versus 25.0 percent in a historical control arm.

The Defitelio conversation in Qatar is not a referral conversation in the same sense as a chronic outpatient drug. The patient is already inside the HSCT admission, either at Sidra Medicine for a paediatric case or cross-border at KFSHRC Riyadh for an adult case (Sidra is paediatric-only; NCCCR adult HSCT capability is limited and most complex adult cases route cross-border to KFSHRC Riyadh for transplant). The treating transplant haematologist makes the diagnosis at the bedside and the question shifts immediately to drug procurement and administration.

What Defitelio is, in plain language

Defibrotide is endothelium-targeted: it increases tissue plasminogen activator in the hepatic sinusoidal endothelium, decreases plasminogen activator inhibitor 1, and reduces endothelial activation that drives the post-HSCT VOD pathology. It does not measurably prolong global coagulation tests at therapeutic doses, which is the operational distinction from heparin, warfarin, and direct oral anticoagulants (all contraindicated in post-HSCT severe VOD).

The drug is given as 6.25 mg/kg IV every 6 hours over 2 hours each, for a minimum of 21 days and a maximum of 60 days. No dose adjustment for renal or hepatic impairment. The drug is given inpatient through a central line, in a transplant unit or ICU setting.

Eligibility for Defitelio at a Qatari or cross-border HSCT centre

Standard FDA-aligned eligibility:

1. Prior HSCT within the prior 21 days. 2. VOD per Baltimore or Modified Seattle criteria: bilirubin 2 mg/dL or greater plus two or more of hepatomegaly, ascites, weight gain greater than 5 percent. 3. Severe VOD: renal or pulmonary dysfunction attributable to VOD. 4. Age 1 month or older. 5. No active uncontrolled bleeding. 6. No concurrent systemic anticoagulant or fibrinolytic that cannot be held. 7. Adequate central venous access.

For paediatric Qatari cases the diagnosis is made at Sidra Medicine; for adult Qatari cases the diagnosis is most commonly made at KFSHRC Riyadh where the transplant was performed.

The Qatar prescribing and supply picture, plainly

Qatar Ministry of Public Health (MOPH) is the national regulator. Defitelio does not have current MOPH-registered formulary status as of 2026. Qatar HSCT centres access Defitelio via the hospital named-patient import mechanism, with supply-chain lead time the operational risk for an indication where treatment-start timing is measured in hours. [VERIFY: current MOPH status and Sidra Medicine and NCCCR hospital stocking patterns at intake.]

The Qatar centre network for severe post-HSCT VOD:

- **Sidra Medicine, Doha**: the paediatric HSCT centre. The relevant Qatar centre for paediatric severe post-HSCT VOD. Sidra Medicine paediatric HSCT and ICU teams coordinate Defitelio acquisition through the hospital pharmacy. Defitelio is one of the rare adult-and-paediatric drugs where Sidra IS the relevant Qatar centre, specifically for the paediatric subset. - **NCCCR (National Center for Cancer Care and Research), Hamad Medical Corporation, Doha**: adult haematology and limited adult BMT capability. Complex adult HSCT cases routinely route cross-border to KFSHRC Riyadh. NCCCR manages pre-transplant workup, post-transplant outpatient care, and the small subset of adult HSCT cases managed in-country. - **Hamad General Hospital, Doha**: adult medicine and ICU capability; not an HSCT centre directly but supports cross-pathway coordination for adult haematology referrals.

For adult Qatari patients, the operational reality is that severe post-HSCT VOD typically develops while the patient is still at the transplant centre. If the patient was transplanted at KFSHRC Riyadh (the most common pathway for complex adult Qatari HSCT cases), the VOD episode is managed at KFSHRC where Defitelio is stocked. The Reserve Meds role in this scenario is family logistics coordination at the KFSHRC inpatient setting and financial pre-authorisation alongside the clinical pathway.

For paediatric Qatari patients, the operational reality is that severe post-HSCT VOD is managed at Sidra Medicine where the transplant was performed. The Reserve Meds role is family logistics support and financial pre-authorisation, with the Defitelio acquisition coordinated by the Sidra pharmacy and clinical team.

Cost band and insurance positioning

US list price approximately USD 825 per 200 mg vial; 21-day adult drug-only course approximately USD 156,000 (approximately QAR 568,000 at indicative 2026 cross rates). Paediatric 21-day drug-only course approximately USD 53,000 to USD 70,000 (approximately QAR 195,000 to QAR 255,000). Full cost of care including the inpatient transplant unit episode commonly USD 350,000 to USD 1.2M (approximately QAR 1.3 to 4.4M). HMC public funding covers Qatari nationals; commercial cover for expatriates is case-by-case.

What to expect on the Defitelio pathway

Day 0 (HSCT day 8 to day 21): clinical deterioration; severe VOD diagnosis confirmed by the transplant team at the treating centre (Sidra for paediatric in-country; KFSHRC Riyadh for adult cross-border; NCCCR for the small adult-in-country subset). Systemic anticoagulants and fibrinolytics held. Defitelio acquisition coordinated.

Day 0 to day 21: minimum course of 6.25 mg/kg IV q6h over 2 hours each. Daily inpatient monitoring; transfusion support; renal replacement therapy and mechanical ventilation if indicated.

Day 21 onwards: continue up to 60-day maximum if VOD has not resolved. Transition to standard post-HSCT supportive care once VOD resolves.

When Defitelio is the wrong drug

For a Qatari patient who does not meet severe-VOD criteria, who has active uncontrolled bleeding, who has a porcine-product hypersensitivity, or who is on a systemic anticoagulant that cannot be held, the operational alternative is supportive care alone. The porcine-derived sourcing is a documented religious-ethical consideration; the published Islamic jurisprudence consensus for porcine-derived medicines used in life-threatening conditions follows the principle of dharura, and the medical-necessity framing of severe VOD with no alternative therapy supports use in this clinical setting.

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 Qatari Defitelio case we coordinate the cross-border referral logistics (KFSHRC Riyadh for adult, Sidra Medicine in-country for paediatric), run financial pre-authorisation alongside clinical pre-authorisation, support the family through the inpatient experience, and stay with the case through the 21 to 60 day Defitelio course and broader HSCT recovery. Clinical decisions remain with your treating transplant haematologist and the HSCT centre care 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.

reservemeds.com · hello@reservemeds.com