Defitelio

United Arab Emirates · access guide

How to access Defitelio for severe veno-occlusive disease after stem-cell transplant from the UAE: 2026 pathway via Cleveland Clinic Abu Dhabi and the wider UAE adult and paediatric HSCT network

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

The UAE has built a meaningful adult and paediatric haematopoietic stem-cell transplant (HSCT) capability over the last decade. Cleveland Clinic Abu Dhabi runs the deepest adult HSCT programme in the country; Sheikh Shakhbout Medical City carries an MD Anderson affiliation and an adult haematology service with paediatric BMT capability in build; Burjeel Medical City runs an adult oncology and BMT programme; and Tawam Hospital runs the principal paediatric oncology programme with HSCT referral pathways. Defitelio (defibrotide sodium) is the only FDA-approved therapy for the most catastrophic post-HSCT complication: severe hepatic veno-occlusive disease (VOD) with renal or pulmonary dysfunction. Defitelio is not on the standard UAE EDE formulary as of 2026; UAE adult and paediatric HSCT centres access it via hospital named-patient stock or single-patient Article 5 import under MOHAP. For a UAE patient who develops severe post-HSCT VOD in the second or third week after transplant, the operational question is whether the treating HSCT centre already has Defitelio in stock, what the supply-chain lead time looks like if it does not, and how the inpatient transplant unit team coordinates Defitelio start within hours of diagnosis.

This page explains how the pathway works in 2026 for a UAE-resident adult or paediatric patient already inside an HSCT admission: when Defitelio is indicated, who confirms the diagnosis, where the drug comes from when it is not on shelf, what the 21-day minimum inpatient course looks like, and what the realistic cost band is for the drug and for the underlying inpatient transplant unit episode.

Why Defitelio, and when

Defitelio is defibrotide sodium, a purified mixture of single-stranded oligodeoxyribonucleotides extracted from porcine intestinal mucosa. It was approved by the FDA in March 2016 for the treatment of adult and paediatric patients aged 1 month and older with hepatic VOD (also called sinusoidal obstruction syndrome) with renal or pulmonary dysfunction following HSCT. Severe post-HSCT VOD is a catastrophic-prognosis condition: day-100 mortality without Defitelio approaches 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. That difference is operationally meaningful in a clinical setting where the alternative is supportive care alone with a hepatology, nephrology, and pulmonary consult and a family conversation that is honest about the prognosis.

For a UAE-resident patient already inside an HSCT admission at Cleveland Clinic Abu Dhabi or another UAE adult or paediatric BMT centre, the Defitelio conversation is not a referral conversation. The patient is already at the centre. The treating transplant haematologist makes the diagnosis on clinical and laboratory grounds, and the question shifts immediately to drug procurement, drug administration, and concurrent inpatient supportive care. Reserve Meds is engaged in this scenario for the families who want the cross-border backstop coordinated in parallel (transfer to KFSHRC Riyadh if UAE supply-chain timing is incompatible with disease tempo) and for the financial pre-authorisation work that runs alongside the clinical pathway.

What Defitelio is, in plain language

Defibrotide does not work the way a systemic anticoagulant works. It is endothelium-targeted: it increases tissue plasminogen activator (t-PA) in the hepatic sinusoidal endothelium, decreases plasminogen activator inhibitor 1 (PAI-1), reduces endothelial cell activation, and decreases the leukocyte-endothelial interaction that drives the post-HSCT VOD pathology. It does not measurably prolong PT, aPTT, or INR at therapeutic doses, which is the operational distinction from heparin, warfarin, direct oral anticoagulants, and fibrinolytic agents, all of which are contraindicated in post-HSCT severe VOD because the patient already has thrombocytopenia, mucosal injury, and the underlying coagulopathy of VOD.

The drug is given as 6.25 mg/kg IV every 6 hours (four doses per day) over 2 hours each. The minimum course is 21 days. The maximum course is 60 days. Treatment continues until VOD resolves or until the 60-day cap is reached. There is no dose adjustment for renal or hepatic impairment, which is the operational simplification for a patient population that may already be on renal replacement therapy and may already have severe liver dysfunction from the VOD itself.

This is not a chronic medication. It is an acute-care inpatient drug, given through a central line that is already in place from the HSCT admission, in a transplant unit or ICU setting, for a defined 21 to 60 day course, with a clearly defined start point (diagnosis of severe VOD) and a clearly defined endpoint (VOD resolution or 60-day cap).

Eligibility for Defitelio at a UAE HSCT centre

The diagnostic criteria are operational and time-sensitive. The UAE adult and paediatric HSCT centres apply the FDA-aligned eligibility criteria:

1. Prior HSCT (autologous or allogeneic) within the prior 21 days, most commonly day 8 to day 21 post-stem-cell infusion. 2. Diagnosis of VOD per Baltimore or Modified Seattle criteria: bilirubin 2 mg/dL or greater within 21 days post-HSCT, plus two or more of (a) hepatomegaly (typically painful), (b) ascites, (c) weight gain greater than 5 percent of baseline. 3. Severe VOD: presence of renal dysfunction (creatinine greater than 1.2 times upper limit of normal, or oliguria, or requirement for dialysis) or pulmonary dysfunction (oxygen requirement, mechanical ventilation, or pulmonary haemorrhage) attributable to the VOD episode. 4. Age 1 month or older. 5. No active uncontrolled bleeding at the time of Defitelio start. 6. No concurrent systemic anticoagulant or fibrinolytic therapy (contraindicated; must be held before Defitelio start). 7. Adequate central venous access for q6h IV infusions over 2 hours each for at least 21 days, typically via a central line already in place from the HSCT admission.

The workup is the standard inpatient post-HSCT workup performed daily: weights, liver function tests, renal function tests, complete blood count with differential, coagulation panel, abdominal ultrasound with Doppler of the hepatic veins and portal vein, and transthoracic echo when pulmonary involvement is present. The differential diagnosis is critical because the management diverges substantially: graft-versus-host disease of the liver, infection, drug-induced injury, and progressive disease all present with overlapping features. The transplant haematologist, the hepatology consult, and the BMT pharmacist drive the diagnosis. Transjugular liver biopsy is rarely performed because the bleeding risk in severe VOD is prohibitive; Defitelio is started on clinical and laboratory grounds.

A UAE patient or family member is not making this eligibility determination. The diagnosis is made at the bedside by the transplant team. The Reserve Meds concierge role in this pathway is to coordinate the cross-border backstop in parallel (transfer to KFSHRC Riyadh if UAE supply-chain or clinical capacity considerations warrant) and to manage the financial pre-authorisation work that runs alongside the clinical pathway, not to influence the eligibility determination.

The UAE prescribing and supply picture, plainly

The Emirates Drug Establishment (EDE) is the federal regulator. Defitelio is not on the standard EDE registered formulary as of 2026. UAE HSCT centres access Defitelio via two operational pathways:

1. **Hospital named-patient stock**: Cleveland Clinic Abu Dhabi and other major UAE HSCT centres maintain a small named-patient stock of Defitelio in the HSCT pharmacy formulary for the foreseeable severe VOD case. When that stock is available, treatment can start within hours of diagnosis. [VERIFY: current hospital stocking pattern at the treating UAE centre at intake.] 2. **Single-patient Article 5 import under MOHAP**: when hospital stock is not available, the UAE MOHAP Article 5 single-patient import mechanism authorises emergency-import of Defitelio from Jazz Pharmaceuticals via a regional distributor. The lead time is the operational risk in a disease where treatment-start timing is measured in hours, not days. Jazz Pharmaceuticals' Middle East commercial team and UAE hospital pharmacy directors coordinate Article 5 imports as part of standard severe-VOD escalation.

The UAE adult HSCT centre network concentrates in Abu Dhabi: Cleveland Clinic Abu Dhabi (the deepest adult HSCT programme in the country), Sheikh Shakhbout Medical City (adult haematology with paediatric BMT capability in build), Burjeel Medical City (adult oncology and BMT), and Yas Clinic Hospital (cellular therapy programme expanding). Tawam Hospital in Al Ain runs the principal paediatric oncology programme with HSCT referral pathways for paediatric cases. Dubai hospital infrastructure handles workup, bridging therapy, and post-transplant outpatient care; commercial HSCT administration is concentrated in Abu Dhabi adult centres with cross-emirate referral. Insurance for Emirati nationals operates through Thiqa; resident commercial cover varies by carrier. The financial pre-authorisation conversation needs to start at the time of severe-VOD diagnosis, not retrospectively after the 21-day course is complete.

For UAE-resident patients where treating-centre supply-chain timing is incompatible with disease tempo, or where the treating centre lacks the ICU or transfusion medicine integration required to manage severe multi-organ-dysfunction VOD, cross-border transfer to KFSHRC Riyadh (the regional reference centre for adult and paediatric post-HSCT VOD management) is the operational alternative. KFSHRC Riyadh has Defitelio stocked in the HSCT pharmacy formulary and runs the deepest adult and paediatric HSCT programme in the Gulf.

Cost band and insurance positioning

US list price for Defitelio is approximately USD 825 per 200 mg vial. A typical 70 kg adult receives 6.25 mg/kg q6h (approximately 437.5 mg per dose, four doses per day, or approximately 1.75 g per day in nine 200 mg vials). At the 21-day minimum course that is approximately 189 vials at USD 825, or approximately USD 156,000 in drug acquisition cost alone. A 60-day maximum course approaches USD 445,000 in drug acquisition cost alone for a 70 kg adult. A paediatric 25 kg patient receives proportionally less drug, at approximately USD 53,000 to USD 70,000 for a 21-day course.

Full cost of care including the inpatient transplant unit or ICU bed (which dominates total cost in severe VOD), transfusion support, renal replacement therapy if needed, mechanical ventilation if needed, hepatology and nephrology and pulmonary consult time, and the underlying HSCT admission cost runs USD 350,000 to USD 1.2M for a complete severe-VOD episode. At indicative 2026 cross rates the 21-day adult drug-only band is approximately AED 573,000; full cost of care for a severe-VOD episode is approximately AED 1.3 to 4.4M. Outliers run higher when prolonged ICU support or multi-organ failure drive admission length.

What to expect on the Defitelio pathway, day by day

Day 0 (HSCT day 8 to day 21): clinical deterioration; weight gain greater than 5 percent of baseline, rising bilirubin, abdominal pain, hepatomegaly. Transplant haematologist orders abdominal ultrasound with hepatic vein Doppler; nephrology and hepatology consults engaged. Severe VOD diagnosis confirmed on clinical and laboratory grounds.

Day 0, within hours: systemic anticoagulants and fibrinolytics held. Central line confirmed adequate for q6h infusions. Defitelio drawn from hospital pharmacy stock if available, or Article 5 emergency import initiated through MOHAP and the regional Jazz distributor.

Day 0 to day 21: minimum course of 6.25 mg/kg IV q6h over 2 hours each. Daily haemodynamic monitoring; daily weights; daily liver and renal function; daily complete blood count and coagulation; daily neurological assessment; transfusion support; renal replacement therapy if indicated; mechanical ventilation if indicated.

Day 21 onwards: continue Defitelio if VOD has not resolved, up to a 60-day maximum. Transition to standard post-HSCT supportive care once VOD resolves. Resume systemic anticoagulants if otherwise indicated only after Defitelio is complete and bleeding risk has stabilised.

When Defitelio is the wrong drug

For a UAE patient who does not meet Baltimore or Modified Seattle severe-VOD criteria (no renal or pulmonary dysfunction component), who has active uncontrolled bleeding at the time of proposed Defitelio start, who has a hypersensitivity to porcine-derived products documented in the medical history, or who is on a concurrent systemic anticoagulant or fibrinolytic that cannot be held, the operational alternative is supportive care alone with hepatology, nephrology, and pulmonary consultation, plus the family prognostic conversation that is honest about the catastrophic mortality of untreated severe VOD. There is no alternative agent with a survival benefit demonstrated against historical controls in this indication.

For a UAE patient where the treating HSCT centre lacks ICU, transfusion medicine, or hepatology consult integration adequate for severe multi-organ-dysfunction VOD, the operational alternative is urgent transfer to KFSHRC Riyadh, which is the regional reference centre for adult and paediatric post-HSCT VOD management and which has Defitelio stocked in the HSCT pharmacy.

What Reserve Meds does on this case

We are a US-based concierge coordinator. We are not the prescriber and not the dispensing pharmacy, and on a Defitelio case we are not driving the inpatient clinical decision-making at the UAE HSCT centre. On a UAE Defitelio case we coordinate the cross-border backstop in parallel with the in-country pathway (KFSHRC Riyadh transfer pathway pre-positioned in case UAE supply-chain or clinical capacity considerations warrant), 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 the 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.

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