

Defitelio

Abu Dhabi · access guide

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

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

Abu Dhabi houses the deepest adult haematopoietic stem-cell transplant (HSCT) capability in the UAE. Cleveland Clinic Abu Dhabi (CCAD) runs the principal adult HSCT programme; Sheikh Shakhbout Medical City (SSMC) runs an MD Anderson-affiliated adult haematology service with paediatric BMT capability in build; Burjeel Medical City runs an adult oncology and BMT programme; Sheikh Khalifa Medical City (SKMC) and Yas Clinic Hospital provide supporting adult haematology and cellular therapy services; and Tawam Hospital in Al Ain handles paediatric oncology and haematology referral. Defitelio (defibrotide sodium) is the only FDA-approved therapy for severe hepatic veno-occlusive disease (VOD) with renal or pulmonary dysfunction post-HSCT, and it is not on the standard UAE EDE formulary as of 2026. Abu Dhabi adult HSCT centres access Defitelio via hospital named-patient stock or single-patient Article 5 import under MOHAP. For an Abu Dhabi-resident patient who develops severe post-HSCT VOD in the second or third week after transplant, the operational question is whether the treating Abu Dhabi HSCT centre already has Defitelio in stock or needs to initiate Article 5 emergency import.

This page explains how the pathway works in 2026 for an Abu Dhabi-resident adult or paediatric patient already inside an HSCT admission at CCAD, SSMC, Burjeel, or cross-border at KFSHRC Riyadh: when Defitelio is indicated, who confirms the diagnosis, how the supply pathway works in the absence of standard EDE registration, and what the 21 to 60 day inpatient course looks like operationally.

Why Defitelio, and when

Defitelio is defibrotide sodium, a purified mixture of single-stranded oligodeoxyribonucleotides from porcine intestinal mucosa. FDA-approved March 2016 for adult and paediatric patients aged 1 month and older with hepatic VOD with renal or pulmonary dysfunction post-HSCT. Severe post-HSCT VOD has day-100 mortality approaching 75 percent without Defitelio; pivotal data show day-100 survival of 38.2 percent on Defitelio versus 25.0 percent on historical control.

For an Abu Dhabi-resident patient the Defitelio decision is made by the treating HSCT-centre transplant haematologist at the bedside, most commonly at Cleveland Clinic Abu Dhabi for an adult case or cross-border at KFSHRC Riyadh or Sidra Doha for a paediatric case. The Reserve Meds role is the cross-border backstop coordination and the financial pre-authorisation work.

What Defitelio is, in plain language

Defibrotide is endothelium-targeted. It increases tissue plasminogen activator in the hepatic sinusoidal endothelium and reduces endothelial activation. It does not measurably prolong PT, aPTT, or INR at therapeutic doses, which is the operational distinction from systemic anticoagulants (all contraindicated in severe post-HSCT 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. Given through a central line, in a transplant unit or ICU setting.

Eligibility for Defitelio at an Abu Dhabi HSCT centre

Standard FDA-aligned eligibility, applied at the bedside:

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. 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.

The diagnosis is made at the bedside by the transplant team at CCAD, SSMC, Burjeel, or the cross-border referral centre. The Abu Dhabi-resident patient or family is not making this eligibility determination.

The Abu Dhabi prescribing and supply picture, plainly

The Emirates Drug Establishment (EDE) is the federal regulator. Defitelio is not on the standard EDE formulary as of 2026. The Abu Dhabi Department of Health (DoH) coordinates Abu Dhabi-emirate dispensing pathway oversight. UAE adult HSCT centres access Defitelio via two operational pathways: hospital named-patient stock (CCAD and SSMC maintain a small named-patient stock for the foreseeable severe-VOD case) and single-patient Article 5 import under MOHAP (the emergency-import mechanism with Jazz Pharmaceuticals' regional commercial channel). [VERIFY: current EDE registration status and Abu Dhabi HSCT centre hospital stocking patterns at intake.]

The Abu Dhabi adult HSCT centre network:

- **Cleveland Clinic Abu Dhabi (CCAD)**: the deepest adult HSCT programme in the UAE, with dedicated adult transplant ICU capability, hepatology consult service, and transfusion medicine integration. Defitelio access via hospital named-patient stock or Article 5 import. - **Sheikh Shakhbout Medical City (SSMC)**: adult haematology partner with MD Anderson Cancer Center affiliation; paediatric BMT capability in build. Cross-pathway referral to CCAD for adult HSCT and severe-VOD cases. - **Burjeel Medical City**: adult oncology and BMT programme; coordinates with CCAD for severe-VOD cases. - **Sheikh Khalifa Medical City (SKMC)**: adult haematology referral support. - **Yas Clinic Hospital**: cellular therapy programme expanding; coordinates with CCAD and SSMC for severe-VOD escalation. - **Tawam Hospital, Al Ain**: paediatric oncology and haematology referral; paediatric HSCT cases referred cross-border to KFSHRC Riyadh or Sidra Medicine Doha; coordinates with the referral centre for Defitelio in the paediatric subset.

Insurance pathways: Thiqa for Emirati nationals; Daman and commercial cover for residents. Cross-border referral pre-authorisation is coordinated by the Abu Dhabi referring haematologist with Reserve Meds support when intra-UAE supply-chain or clinical capacity considerations warrant.

For Abu Dhabi-resident patients where treating-centre supply-chain timing is incompatible with disease tempo or where the case clinical complexity exceeds Abu Dhabi capability, cross-border transfer to KFSHRC Riyadh (the regional reference centre for adult and paediatric post-HSCT VOD management) is the operational alternative.

Cost band

US list price approximately USD 825 per 200 mg vial; 21-day adult drug-only course approximately USD 156,000 (approximately AED 573,000 at indicative 2026 cross rates). Full cost of care for a severe-VOD episode including the inpatient transplant unit episode commonly USD 350,000 to USD 1.2M (approximately AED 1.3 to 4.4M).

What to expect on the Defitelio pathway

Day 0 (HSCT day 8 to day 21 at the treating centre): clinical deterioration; severe VOD diagnosis confirmed by the treating transplant team. Systemic anticoagulants and fibrinolytics held. Defitelio drawn from CCAD or SSMC hospital pharmacy stock or Article 5 import initiated.

Day 0 to day 21: minimum course of 6.25 mg/kg IV q6h over 2 hours each. Daily inpatient monitoring at the treating centre; 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 an Abu Dhabi-resident patient at the treating HSCT centre 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. CCAD and the wider Abu Dhabi HSCT centre network have established protocols for documenting the family conversation around porcine-derived therapeutics.

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 an Abu Dhabi-resident Defitelio case the patient is at CCAD, SSMC, Burjeel, or a cross-border referral centre in the inpatient setting; we coordinate the cross-border backstop logistics in parallel with the in-country pathway, run financial pre-authorisation alongside clinical pre-authorisation with the Abu Dhabi payer, 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 the 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.

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.

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