Defitelio

Saudi Arabia · access guide

How to access Defitelio for severe veno-occlusive disease after stem-cell transplant from Saudi Arabia: 2026 pathway via KFSHRC Riyadh, KFSH Jeddah, KKUH, and the wider Saudi adult and paediatric HSCT network

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

Saudi Arabia operates the deepest haematopoietic stem-cell transplant (HSCT) infrastructure in the wider Gulf region for both adult and paediatric patients. King Faisal Specialist Hospital and Research Centre (KFSHRC) Riyadh runs the regional reference adult and paediatric BMT programmes; KFSHRC Jeddah runs the principal secondary adult and paediatric BMT site; King Khalid University Hospital (KKUH) Riyadh runs an established paediatric BMT programme; and King Abdulaziz Medical City (KAMC) Riyadh and Jeddah run National Guard Health Affairs adult and paediatric BMT programmes. 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 registered with the Saudi SFDA for adult and paediatric severe VOD. KFSHRC Riyadh, KFSHRC Jeddah, and KKUH stock Defitelio in their HSCT pharmacy formularies. For a Saudi patient who develops severe post-HSCT VOD in the second or third week after transplant, the operational question is whether the treating BMT centre has Defitelio in stock (most KSA adult and paediatric HSCT centres do), how the inpatient transplant unit team coordinates the q6h infusion schedule for the 21 to 60 day course, and how the financial pre-authorisation work runs alongside the clinical pathway.

This page explains how the pathway works in 2026 for a Saudi-resident adult or paediatric patient already inside an HSCT admission: when Defitelio is indicated, who confirms the diagnosis, how the SFDA-registered supply pathway works, 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. 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. For a Saudi patient already inside an HSCT admission, the survival benefit is operationally meaningful in a clinical setting where the alternative is supportive care alone with a hepatology, nephrology, and pulmonary consult.

The Defitelio conversation in a Saudi BMT centre is not a referral conversation. The patient is already inside the transplant unit. The treating transplant haematologist makes the diagnosis on clinical and laboratory grounds and the question shifts immediately to drug administration. KFSHRC Riyadh, KFSHRC Jeddah, KKUH, and KAMC Riyadh and Jeddah all maintain Defitelio in the HSCT pharmacy formulary for severe-VOD escalation. Reserve Meds is engaged in this scenario for the families who want financial pre-authorisation and post-transplant repatriation logistics coordinated alongside the clinical pathway.

What Defitelio is, in plain language

Defibrotide is endothelium-targeted rather than systemic-anticoagulant. 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 the 60-day cap is reached. There is no dose adjustment for renal or hepatic impairment.

This is an acute-care inpatient drug, given through a central line already in place from the HSCT admission, in a transplant unit or ICU setting, for a defined 21 to 60 day course.

Eligibility for Defitelio at a Saudi HSCT centre

The Saudi BMT 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: renal dysfunction or pulmonary dysfunction 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). 7. Adequate central venous access for q6h IV infusions over 2 hours each for at least 21 days.

The differential diagnosis (graft-versus-host disease of the liver, infection, drug-induced injury, progressive disease) is the critical inpatient discipline. 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 Saudi patient or family member is not making this eligibility determination. The diagnosis is made at the bedside by the transplant team.

The Saudi prescribing and supply picture, plainly

The Saudi Food and Drug Authority (SFDA) is the national regulator. Defitelio is registered with SFDA for adult and paediatric severe post-HSCT VOD via Jazz Pharmaceuticals' regional commercial channel. The SFDA-registered status means Saudi BMT centres can stock Defitelio in the HSCT pharmacy formulary without recourse to single-patient import authorisation. [VERIFY: current SFDA registration status and hospital stocking pattern at the treating Saudi centre at intake.]

The Saudi adult and paediatric BMT centre network:

- **KFSHRC Riyadh**: the deepest adult and paediatric BMT programme in the Gulf, with dedicated adult and paediatric transplant ICU capability, hepatology consult service, and transfusion medicine integration. Defitelio stocked. The regional reference centre for adult and paediatric post-HSCT VOD management. - **KFSHRC Jeddah**: secondary adult and paediatric BMT site. Defitelio stocked. - **KKUH Riyadh**: King Saud University paediatric BMT programme. Defitelio stocked for paediatric severe VOD. - **KAMC Riyadh and Jeddah**: National Guard Health Affairs adult and paediatric BMT programmes. Defitelio access via hospital formulary. - **King Fahad Medical City (KFMC), Riyadh**: adult haematology referral capacity with established BMT pathway coordination to KFSHRC and KAMC. - **Princess Noorah Oncology Center, Jeddah, and Princess Sultan Military Medical City (PSMMC), Riyadh**: adult haematology referral with BMT pathway coordination to KFSHRC, KFSHRC Jeddah, and KAMC.

Insurance pathways: the Ministry of Health public funding pathway through NUPCO covers Defitelio for Saudi nationals in MoH facilities; Tameen the general medical insurance scheme covers expatriates and private-sector employees with case-by-case pre-authorisation; GOSI covers the social insurance subset. Financial pre-authorisation runs in parallel with the clinical pathway and is initiated at the time of severe-VOD diagnosis, not retrospectively.

For Saudi-resident patients where the treating centre lacks the ICU or transfusion medicine integration adequate for severe multi-organ-dysfunction VOD, intra-Saudi transfer to KFSHRC Riyadh is the operational alternative. Cross-border transfer is rarely required for Saudi-resident patients.

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, 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 SAR 585,000; full cost of care for a severe-VOD episode is approximately SAR 1.3 to 4.5M. Saudi national patients in MoH facilities have NUPCO coverage for Defitelio; private-sector and expatriate cover varies.

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.

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.

When Defitelio is the wrong drug

For a Saudi patient who does not meet Baltimore or Modified Seattle severe-VOD criteria, 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. There is no alternative agent with a survival benefit demonstrated against historical controls in this indication.

The porcine-derived sourcing of defibrotide is a documented religious-ethical consideration in observant Muslim contexts. The published Islamic jurisprudence consensus for porcine-derived medicines used in life-threatening conditions where no alternative is available follows the principle of dharura, and the medical-necessity framing of severe VOD with no alternative therapy supports use in this clinical setting. KFSHRC, KAMC, and the wider Saudi BMT centre network have established protocols for documenting the family conversation around porcine-derived therapeutics. The conversation with the family is brief and supportive, framed around medical necessity and the established jurisprudential precedent.

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 Saudi HSCT centre. On a Saudi Defitelio case we run financial pre-authorisation alongside clinical pre-authorisation, support the family through the inpatient experience, coordinate any out-of-kingdom referral logistics when warranted, 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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