

SPECIALISED COMMISSIONING – RESPONSE TO AMENDMENTS REQUESTED TO EVIDENCE REVIEW DURING ENGAGEMENT OR CONSULTATION

URN	B04/P/c
POLICY TITLE	Use of defibrotide in severe veno-occlusive disease following stem cell transplant (all ages)
CRG:	Blood and Marrow Transplantation
NPOC:	Blood and Infection
Date	22/04/2020

<p>Description of comments during consultation (If studies have been suggested please provide a list of references)</p>	<p>Suggested that the “Criteria” section be amended to take into account the EBMT guidelines for the diagnosis of VOD/SOS specifically for children (Corbacioglu, 2018). Recommended for two reasons: (1)The EBMT guidelines by Mohty et al (2016) are limited to making a diagnosis in adults. The proposed policy amendment is to use these adult EBMT criteria in the clinical assessment of children, which is not as EBMT intended; “...a proposal for distinct diagnostic criteria for adults and children seems to be mandatory. This paper focuses on adult patients; diagnostic criteria for children will be developed in another article.” (Mohty, 2016) (2) Extant guidelines specifying that VOD must be diagnosed in <21 days (Baltimore, Modified Seattle) have been superseded in paediatric (but not adult) clinical practice by EBMT guidelines for children (Corbacioglu, 2018) which are agnostic of timing of VOD symptom onset post-HSCT. This could be achieved by amending the first bullet point in the “Criteria” section to: “Adults or children who have a diagnosis of severe veno-occlusive disease following BMT based on clinical criteria (EBMT, modified Seattle or Baltimore criteria) or histopathological findings”</p>
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<p>Action taken by Public Health lead</p>	<p>Reviewed the references within the Policy Proposition evidence summary and that of the EBMT guidelines by Mohty et al (2016) with the references for the suggested changes for a revised timing of VOD symptom onset post-HSCT contained within the additional EBMT guidelines for children (Corbacioglu, 2018).</p> <p>7 (seven) references are provided within the updated EBMT guidelines for children (Corbacioglu, 2018) that relate to the removal of the <21 days post onset criteria. Of those the following 2 references are new references for evidence not previously considered.</p> <p>Barker CC, Butzner JD, Anderson RA, Brant R, Sauve RS. Incidence, survival and risk factors for the development of veno-occlusive disease in pediatric hematopoietic stem cell transplant recipients. Bone Marrow Transplant 2003; 32: 79–87.</p> <p>Cesaro S, Pillon M, Talenti E, Toffolutti T, Calore E, Tridello G et al. A prospective survey on incidence, risk factors and therapy of hepatic veno-occlusive disease in children after hematopoietic stem cell transplantation. Haematologica 2005; 90: 1396–1404.</p> <p>The removal of the criteria that symptom onset must be within 21 days of transplant does not materially affect the conclusions of the evidence review.</p>
<p>Outcome for studies suggested during consultation</p>	
<p>1. Evidence already identified during the evidence review</p>	<p>Corbacioglu et al, 2012; Carreras et al, 2007; McDonald et al, 1984; Toh et al, 1993; Pai et al, 2012 – are already considered within the EBMT guidelines by Mohty et al (2016).</p>
<p>2. New evidence identified by stakeholders that does not fall within PICO and search methodology</p>	<p>Not applicable.</p>

<p>3. New evidence identified by stakeholders that falls within PICO and search methodology but does not materially affect the conclusions of the existing evidence review</p>	<p>Barker CC, Butzner JD, Anderson RA, Brant R, Sauve RS. Incidence, survival and risk factors for the development of veno-occlusive disease in pediatric hematopoietic stem cell transplant recipients. <i>Bone Marrow Transplant</i> 2003; 32: 79–87.</p> <p>Cesaro S, Pillon M, Talenti E, Toffolutti T, Calore E, Tridello G et al. A prospective survey on incidence, risk factors and therapy of hepatic veno-occlusive disease in children after hematopoietic stem cell transplantation. <i>Haematologica</i> 2005; 90: 1396–1404.</p>
<p>4. New evidence identified by stakeholders that falls within PICO and search methodology, that does materially affect the conclusions of the existing evidence review. Updated evidence review to be undertaken (agreed with CET)</p>	<p>Not Applicable.</p>