9th October 2019

SPECIALISED SERVICES CLINICAL PANEL
CONSIDERATION OUTCOME OF A PRELIMINARY POLICY PROPOSAL (PPP)

Intervention Title	Defibrotide
Indication Title	Treatment of late veno-occlusive disease of the liver in the period following 21 days post bone marrow transplantation
PPP ID	P006018
Proposer is requesting for the intervention to be:	Routinely commissioned

National Programme of Care (PoC)	Blood and Infection
Clinical Reference Group	Bone and Marrow Transplantation
Recommendation by PoC	The revision of the current use of defibrotide in severe veno-occlusive disease following stem cell transplant policy is recommended by the PoC. The EBMT diagnostic criteria were published in 2016, they post-date the current NHSE Clinical Commissioning Policy (2015). It is recommended that the policy is reviewed to include EBMT diagnostic criteria for patients that exhibit signs and symptoms consistent with veno-occlusive disease after 21 days. The current Modified Seattle criteria used with in current policy stipulate that symptoms and signs of veno-occlusive disease must have emerged within 21 days in order to make the diagnosis.
Media Interest	Low

Date of Clinical Panel	16th October 2019

The proposal:

- Is for a specialised commissioned service
- Defibrotide is indicated for the treatment of severe hepatic veno-occlusive disease (VOD) also known as sinusoidal obstruction syndrome (SOS) in haematopoietic stem-cell transplantation (HSCT) therapy. It is indicated in adults and in adolescents, children and infants over 1 month of age. There appears to be no restriction within the licence on the number of days after HSCT and the onset of VOD when defibrotide can used. It is excluded from tariff
- There is a routinely commissioned Clinical Commissioning Policy Statement: Use of defibrotide in severe veno-occlusive disease following stem cell transplant for patients who develop VOD up to 21 days.

Work Programme Recommendation

- The proposal should proceed on the work programme as an update of the existing Policy Statement
- A 3 paper evidence review is recommended

Clinical Panel discussion:

Panel discussed that there is an existing published clinical policy relating to this indication and intervention commissioning for symptoms identified up to 21 days. This PPP requests an extension to that, past 21 days.

Three papers presented, Paper one demonstrated the better evidence base although difficult to find evidence for the specific criteria expected.

Panel agreed that amendments would be made to the existing policy.

- Need an added section on late onset patients in patient selection section
- Refer to additional new paper in the current policy
- Section 13 – remove the wording in the current policy
- Cost effectiveness section on page 9 needs removing
- Remove reference to 'rare' and leave in 'uncommon'

Panel agreed to a rapid turn around and for Chair's action.

Post Panel note from the Blood & Infection PoC:

The above amendments have been made in the revised policy proposition.