



## Final Appraisal Recommendation

Advice No: 0515 – March 2015

### Defibrotide (Defitelio<sup>®</sup>▼) 80 mg/ml concentrate for solution for infusion

Submission by Jazz Pharmaceuticals

#### Recommendation of AWMSG

Defibrotide (Defitelio<sup>®</sup>▼) is recommended for use within NHS Wales for the treatment of severe hepatic veno-occlusive disease (VOD) also known as sinusoidal obstructive syndrome (SOS) in haematopoietic stem-cell transplantation (HSCT) therapy.

This recommendation applies only in circumstances where the approved Wales Patient Access Scheme is utilised.

#### Additional note(s):

- AWMSG considered that defibrotide (Defitelio<sup>®</sup>▼) satisfies the AWMSG criteria for ultra orphan drug status.
- Defibrotide (Defitelio<sup>®</sup>▼) must be prescribed and administered to patients by specialised physicians experienced in the diagnosis and treatment of complications of HSCT.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 508), which includes the AWMSG Secretariat Assessment Report (ASAR), the Preliminary Appraisal Recommendation (PAR) and the applicant company's response to the PAR, clinical expert opinion (where available), the views of patients/patient carers (where available) and the lay member perspective.

This recommendation has been ratified by Welsh Government and will be considered for review every three years.

Marketing authorisation holder on first issue	Jazz Pharmaceuticals
Date of first issue	March 2015
Last reviewed	March 2018



NICE has accredited the process used by the All Wales Medicines Strategy Group (AWMSG) to produce its final appraisal recommendation. Accreditation is valid for 5 years from October 2011. More information on accreditation can be viewed at [www.evidence.nhs.uk](http://www.evidence.nhs.uk).

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