AWTTC
All Wales Therapeutics & Toxicology Centre
Canolfan Therapiwteg a Thocsicoleg Cymru Gyfan

Enclosure No:	2/AWMSG/0526
Agenda Item No:	6 – Emicizumab (Hemlibra®) for the routine prophylaxis of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors who have moderate disease (FVIII ≥ 1% and ≤ 5%) with severe bleeding phenotype (4742)
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Action for AWMSG:
Members are asked to consider the decision rationale and endorse the recommendation made by the Licensed One Wales Medicines Assessment Group (LOWMAG) to confirm that the decision-making process has been completed robustly and consistently.

Purpose:
Licensed medicines may be assessed by the AWMSG limited assessment route if the AWMSG Scrutiny Panel determine that specific criteria have been met which justify that the review of clinical evidence (if appropriate), budget impact, equity of access and wider societal issues is sufficient to make a robust recommendation on access to the medicine in NHS Wales. LOWMAG has considered and discussed the evidence presented in an Evidence Summary Report (ESR) compiled by AWTTC, the views of clinicians and patients, and comments from the Marketing Authorisation holder, and has provided a recommendation with rationale to the All Wales Medicines Strategy Group (AWMSG). AWMSG is being asked to endorse the LOWMAG recommendation which will then be forwarded to Welsh Government for ratification.

Overview:
The AWMSG Scrutiny Panel decided that emicizumab (Hemlibra®) was suitable for a limited assessment via the Licensed One Wales Medicines Assessment Group (LOWMAG) as the medicine is recommended as a prophylactic option for moderate haemophilia A in international guidelines, the anticipated budget impact is expected to be low, and it is available to the same patient population in England. The AWMSG Scrutiny Panel agreed that the case for clinical effectiveness was established and did not warrant further review and so the limited assessment considered current use, equity of access and budget impact only.

Attached is the LOWMAG recommendation and accompanying rationale for:

Emicizumab (Hemlibra®) for the routine prophylaxis of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors who have moderate disease (FVIII ≥ 1% and ≤ 5%) with severe bleeding phenotype.

The ESR has been provided separately for information.

If you have any questions regarding the ESR, LOWMAG recommendation or decision rationale, please email awttc@wales.nhs.uk by 13th of May 2026.

The assessment lead will respond to your query by email ahead of the meeting and will also provide a verbal summary at the meeting of any significant issues raised. It is anticipated that there will be no discussion required on the day, however, there will be opportunity to clarify any outstanding issues.

Once the LOWMAG recommendation has been endorsed by AWMSG, it will be forwarded to Welsh Government for ratification. In exceptional circumstances, where AWMSG is unable to endorse the recommendation by LOWMAG, the reasons for non-endorsement must be fully justified and will be raised with LOWMAG for further consideration. This will allow for recommendations to be re-considered by AWMSG subject to further information being supplied by LOWMAG.

Licensed One Wales Medicines Assessment Group Recommendation
Emicizumab (Hemlibra®) 150 mg/ml solution for injection

Date of advice: April 2026
AWTTC reference number: 4742

Recommendation to the All Wales Medicines Strategy Group (AWMSG)

Emicizumab (Hemlibra®) is recommended as an option for use in NHS Wales for the routine prophylaxis of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors who have moderate disease (FVIII ≥ 1% and ≤ 5%) with severe bleeding phenotype.

This recommendation applies only in circumstances where the agreed commercial access arrangement (CAA) between the MA holder and NHS Wales is utilised or where the list / contract price is equivalent or lower than the CAA price.

This recommendation will be reviewed if there is new evidence that is likely to change it.

Licensed One Wales Medicine Assessment Group summary of decision rationale

Medicine: **Emicizumab (Hemlibra®)**
Assessment type: **Limited**
Indication under consideration: **For the routine prophylaxis of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors who have moderate disease (FVIII ≥ 1% and ≤ 5%) with severe bleeding phenotype.**
Meeting date: **15 April 2026**

Criteria	LOWMAG opinion
Clinical effectiveness	The scrutiny panel acknowledged that clinical effectiveness is already established and therefore no clinical effectiveness evidence was presented to or considered by LOWMAG.
Cost-effectiveness	As this is a limited assessment cost effectiveness is not considered.
Budget impact	LOWMAG considers the estimates of patient numbers to be reasonable. LOWMAG agrees that the most appropriate comparators are the extended half-life rFVIII products - Esperoct® for adults and children aged 12 years and older and Elocta® for children under 12 years. LOWMAG notes that assumptions about the comparators and their costs used to inform the company base case are not reflective of the situation in Wales and so predicted cost-savings may not be realised. LOWMAG considers that the BI analysis by AWTTC may provide a more reasonable estimate of the costs associated with the use of emicizumab to NHS Wales. However, LOWMAG acknowledges the difficulty in estimating with any certainty an overall budget impact for the predicted 10 patients that may receive this treatment for moderate HA in Wales as costs are weight-dependent and so will vary between different individuals, and budget impact will be substantially impacted by the proportion of children to adults contributing to this overall number, and hence the rFVIII comparator product displaced. LOWMAG acknowledges that the budget impact estimates consider medicine acquisition costs only; other resource use is not included (e.g. monitoring costs and costs associated with adverse events). As both emicizumab and rFVIII treatments are generally self-administered at home, LOWMAG considers that any additional resource costs

	(such as initiation of treatment in a healthcare setting and homecare delivery costs etc) will be similar. LOWMAG notes that some of this patient population is already receiving emicizumab prophylaxis for this indication via Individual Patient Funding Request and so a proportion of the predicted patient numbers and associated budget impact is already accounted for.
Resource use	LOWMAG notes that treatment with emicizumab is unlikely to be associated with significant additional resource use compared with comparator rFVIII products and no additional monitoring is required.
Other factors	LOWMAG notes that prophylaxis is recommended in international guidelines for people with moderate haemophilia A (HA) with a severe bleeding phenotype. LOWMAG notes that emicizumab is an alternative prophylaxis treatment to FVIII replacement and is routinely available in Wales and in England for people with severe HA without inhibitors and for people with HA with inhibitors. LOWMAG notes that the current prophylaxis offered in Wales for moderate HA is FVIII replacement although some patients opt for on-demand treatment only. LOWMAG notes the view of the clinical expert present at the meeting who emphasised the importance of prophylaxis for patients with moderate HA as joint damage by mid-adulthood caused by spontaneous bleeding was similar to that seen in people with severe HA in the absence of prophylaxis. LOWMAG also notes that routine access to emicizumab for moderate HA with a severe bleeding phenotype has recently been enabled for people in England and in Northern Ireland and similar access for patients in Wales is supported by clinical experts from the Cardiff Haemophilia Centre and by the patient organisation, Haemophilia Wales. LOWMAG acknowledges that emicizumab has lower treatment burden for patients than rFVIII; this is due to its subcutaneous administration in comparison to rFVIII prophylaxis which has to be administered intravenously and the significantly longer time intervals between doses. LOWMAG notes the views of the clinical expert present at the meeting and in the submission from Haemophilia Wales who highlighted that the easier administration of emicizumab by subcutaneous injection is particularly

	important in babies and young children and in older adults with damaged veins. LOWMAG notes that clinicians state that prophylaxis with emicizumab can be started much earlier in babies than rFVIII treatment due to its subcutaneous delivery and that young children will not require a port-a-cath. Clinicians highlight the importance of being able to start prophylaxis soon after birth to optimise outcomes and enable children with moderate HA to reach adulthood with normal joints and live a full and active life, in the absence of bleeds. LOWMAG also acknowledges that Haemophilia Wales report positive experiences of people receiving emicizumab stating that it has been life-changing by providing them with a freedom they had not previously experienced and that parents of affected children express that stress levels are lessened as emicizumab removes the constant worry of unexpected bleeding.
Final recommendation	LOWMAG recommends the use of emicizumab (Hemlibra®), for the routine prophylaxis of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors who have moderate disease (FVIII ≥ 1% and ≤ 5%) with severe bleeding phenotype. This recommendation applies only in circumstances where the agreed commercial access arrangement (CAA) between the MA holder and NHS Wales is utilised or where the list / contract price is equivalent or lower than the CAA price.
Summary of rationale	LOWMAG notes that prophylaxis is recommended in international guidelines for people with moderate haemophilia A (HA) with a severe bleeding phenotype. LOWMAG notes that emicizumab is an alternative prophylaxis treatment to FVIII replacement products. LOWMAG also notes that emicizumab is routinely available for this patient population in England and in Northern Ireland. LOWMAG recognises that emicizumab offers a lower treatment burden for patients than rFVIII prophylaxis and would particularly benefit those who would gain advantage from receiving prophylaxis but are not able to manage the treatment burden of intravenous rFVIII treatment. LOWMAG considers that making emicizumab prophylaxis routinely available throughout NHS Wales for the small

	number of people with moderate haemophilia A with a severe bleeding phenotype will likely modestly increase medicine acquisition costs in comparison to rFVIII prophylaxis but that this would be reasonable in considering the potential benefit gained from this intervention.