

C.1 Cryoprecipitate, pathogen-reduced cryoprecipitate, plasma-derived clotting factor concentrates – EML and EMLc

Reviewer summary	<input checked="" type="checkbox"/> Supportive of the proposal <input type="checkbox"/> Not supportive of the proposal <p>Justification (based on considerations of the dimensions described below):</p> <p>This submission seeks to update the EML and EMLc to conform to widely practiced treatment standards for people with hemophilia A, hemophilia B, and VWD.</p> <p>I support to:</p> <ol style="list-style-type: none"> 1. Remove cryoprecipitate (Cryo) from the EML for all listed indications. Cryo has not been subjected to a pathogen reduction process, which introduces safety risks. Considering the availability of safer and more efficacious factor VIII (FVIII) concentrates, we suggest its removal. 2. Limit the use of pathogen-reduced cryoprecipitate (PR Cryo) to evidence-based indications and only for the treatment of severe bleeding including in patients with hemophilia A if FVIII concentrates are not available for these chronic bleeding disorders. 3. Transfer the listings of plasma-derived FVIII concentrates for the treatment of hemophilia A and VWD and the plasma-derived Factor IX (FIX) concentrates for the treatment of hemophilia B from the Complimentary List to the Core List, given the superior efficacy, safety, and cost-effectiveness of these products. Prophylaxis has been the standard of care to minimize bleeding sequelae for both hemophilia A and B for 30 years, which cannot be accomplished with Cryo or PR Cryo. 4. Remove FIX complex as a therapeutic alternative to FIX concentrates for the treatment of hemophilia B, given the risk of thrombosis associated with this alternative therapy and the increased cost-effectiveness of FIX concentrates.
<p>Does the EML and/or EMLc currently recommend alternative medicines for the proposed indication that can be considered therapeutic alternatives?</p> <p>(https://list.essentialmeds.org/)</p>	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable
<p>Does adequate evidence exist for the efficacy/effectiveness of the medicine for the proposed indication?</p> <p>(e.g., evidence originating from multiple high-quality studies with sufficient follow up. This may be evidence included in the application, and/or additional evidence identified during the review process;)</p>	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable
<p>Does adequate evidence exist for the safety/harms associated with the proposed medicine?</p> <p>(e.g., evidence originating from multiple high-quality studies with sufficient follow up. This may be evidence included in the application, and/or additional evidence identified during the review process;)</p>	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable
<p>Overall, does the proposed medicine have a favourable and meaningful balance of benefits to harms?</p>	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable
<p>Are there any special requirements for the safe, effective and appropriate use of the medicines?</p> <p>(e.g. laboratory diagnostic and/or monitoring tests, specialized training for health providers, etc)</p>	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable
<p>Are there any issues regarding price, cost-effectiveness and budget implications in different settings?</p>	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable

25th WHO Expert Committee on Selection and Use of Essential Medicines
Expert review

Is the medicine available and accessible across countries? (e.g. shortages, generics and biosimilars, pooled procurement programmes, access programmes)	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable
Does the medicine have wide regulatory approval?	<input checked="" type="checkbox"/> Yes, for the proposed indication <input type="checkbox"/> Yes, but only for other indications (off-label for proposed indication) <input type="checkbox"/> No <input type="checkbox"/> Not applicable