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A.23 Recombinant coagulation factor VIII and IX therapeutics for hemophilia

MSF supports the proposal from the World Federation of Hemophilia for the inclusion of recombinant FVIII (rFVIII) and recombinant FIX (rFIX) with a square box (□) indicating suitable therapeutics alternatives, on the Core list of both the WHO Model List of Essential Medicines (EML) and the WHO Model List of Essential Medicines for Children (EMLc), for treatment of hemophilia.

The treatment of hemophilia has evolved tremendously over the past five decades from fresh frozen plasma as the only available therapy to more specific plasma-derived and recombinant-derived factor replacement. The goal of therapy remains on-demand treatment given at the time of bleeding, routine prophylaxis to reduce bleeding risk and frequency of bleeding episodes, perioperative hemostatic control, management of menorrhagia and uterine bleeding in carriers and women with hemophilia.

Both rFVIII and rFIX are synthetic proteins created using recombinant DNA technology and are used in adults and children with hemophilia A or B, respectively, for episodic treatment of acute bleeding and regular prophylactic treatment to reduce bleeding risk and frequency.

MSF recognizes that a large body of evidence has shown that both plasma-derived and recombinant clotting factor concentrates (CFCs) are safer and more efficacious therapy than treatment with blood and FFP transfusions, and MSF supports the proposal that recombinant clotting factor concentrates should be used in preference to plasma-derived CFCs.

MSF recommends that the 25th Expert Committee on the Selection and Use of Essential Medicines includes both recombinant FVIII and recombinant FIX with a square box (□) indicating suitable therapeutics alternatives, on the Core list of both the WHO Model List of Essential Medicines and the WHO Model List of Essential Medicines for Children, for treatment of hemophilia. MSF recommends that special attention should be given to patients with inhibitors against FVIII or FIX or with long term joint disease and suggests recommendations about inhibitors testing and management should be more developed to support the introduction of recombinant CFCs in clinical practice.

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