25th WHO Committee of Experts on Selection and Use of Essential Medicines
World Health Organization
20 Appia Avenue, 1211 Geneva 27
Switzerland

Subject: Essential medicines for the treatment of hemophilia, von Willebrand Disease and rare congenital coagulation disorders

Dear Members of the 25th WHO Committee of Experts on the Selection and Use of Essential Drugs:

The Hemostasis and Thrombosis Cooperative Group (Grupo CLAHT in Spanish) is a non-profit international medical-scientific association, a legal entity, and registered under the law, representing by 18 countries and more than 400 members from Latin America. On this occasion we refer to the request by the World Federation of Hemophilia for the modification of the Essential Medicines List and the Essential Medicines List for Children regarding the treatment of Hemophilia A, Hemophilia B and von Willebrand Disease (Applications: C.1 Changes to listings of cryoprecipitate, pathogen-reduced cryoprecipitate, and plasma-derived clotting factor concentrates; A.23 Recombinant coagulations factors – haemophilia; and A.12 Emicizumab – haemophilia A).

Essential medicines are defined as those that effectively and safely treat the priority health needs of the population. These drugs are selected on the basis of their relevance to public health and the available evidence of their benefits and harms, as well as cost, affordability and other relevant factors.

Operational health systems must always have sufficient quantities of essential medicines to meet patients' needs.

These medicines must be available in pharmaceutical forms suitable for their intended uses and patients, be quality assured and affordable for both individuals and the healthcare system.

The medicines included in this list are for orphan, rare and ultra-orphan and neglected diseases.

With this in mind, and attending at the experience of the scientific committee on hemophilia and other clotting deficiencies of CLAHT – WFH that the following changes to the essential medicines list are suggested:

1) Modify: Regarding hemophilia A and B and vWD, cryoprecipitates and fresh frozen plasma should not be included as a therapeutic option. Cryoprecipitates should be recommended only for the treatment of life-threatening emergencies in patients with

congenital fibrinogen or factor XIII deficiency due to lack of timely access to the respective concentrates.

2) Include as an essential medicine Emicizumab bi-specific factor VIII mimetic antibody for prophylaxis of hemophilia A patients with and without inhibitors, both in children and adults.

3) Emphasize the inclusion in the Essential Medicines List and Essential Medicines List for Children of factor VIII and factor IX concentrates of both human and recombinant origin for the treatment of hemophilia A and B without inhibitors, both in pharmacological prophylaxis and for the treatment of life-threatening and non-life-threatening hemorrhagic events. Likewise, consider their use for treatment regimens, such as the Immuno-Tolerance Induction for inhibitor elimination.

4) Include von Willebrand Factor concentrates as an essential medicine for the treatment of bleeding episodes in patients with von Willebrand's Disease, as well as for prophylactic treatment in those patients with specific clinical indications.

5) Include sterile, frozen solution of multi-donor human plasma treated with a solvent-detergent process for the treatment of life-threatening and non-life-threatening bleeding caused by congenital factor V or factor XI deficiency, as well as coagulopathies other than factor VIII and factor IX deficiency.

6) Include: Recombinant Factor VII for pharmacologic prophylaxis in patients with congenital hemophilia A and B with high-titer inhibitors and for the treatment of life-threatening and non-life-threatening bleeds. Indicated for bleeding in patients receiving Emicizumab. Useful for prophylaxis and treatment of bleeding in patients with congenital factor VII deficiency.

7) Modify: Activated prothrombin complex for pharmacological prophylaxis in patients with congenital hemophilia A with highly responsive inhibitors also for the treatment of life-threatening and non-life-threatening bleeding. Useful for prophylaxis and treatment of patients with congenital deficiency of factors II, VII and X.

8) Emphasize desmopressin as an essential drug for surgical prophylaxis and treatment of minor bleeding episodes in mild and moderate hemophilia A, as well as in von Willebrand Disease.

9) Emphasize the use of tranexamic acid in tablet and ampoule presentations for the prevention and treatment of life-threatening and non-life-threatening bleeding in patients with congenital hemorrhagic coagulopathies.

Dear Committee, I'd be remissive if I didn´t point out that any improvement in the selection and inclusion of therapies for people with inherited bleeding disorders, especially Hemophilia A, Hemophilia B and von Willebrand's Disease, is an important step, not only to improve their quality of life, but also to significantly reduce the risk of joint damage, disability and premature death.

Without further ado, best regards.

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