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Outcome of the workshop - Joint European Medicines Agency / European Directorate for the Quality of Medicines and Healthcare workshop on characterisation of new clotting factor concentrates (factor VIII and factor IX) with respect to potency assays used for labelling and testing of post-infusion samples

On 28-29 November 2013, the European Medicines Agency (EMA), with the support of the EDQM (Council of Europe), held a workshop to discuss potency assays used for labelling and testing of post-infusion samples for new clotting factor VIII and IX concentrates, which will be used to treat Haemophilia A[1] and B[2]. At present, a number of clotting factor products are authorised in Europe. However, many new generation recombinant products are in the late stages of development and it was felt that a more harmonised approach to assigning potency to these clotting factor concentrates was required.

The workshop was attended by participants representing European, Canadian and American regulatory authorities, manufacturers, industry associations, patient associations and clinicians. Members of the European Pharmacopoeia[3] Group of Experts on Blood and Blood Products also participated in the discussions. Manufacturers presented data on their new generation recombinant products and how they have assigned potency to these products.

Participants welcomed the opportunity to meet other stakeholders and to discuss issues that were important in relation to the selection of methods for potency assignment. It was also an opportunity for participants to be informed about the work of the EMA and EDQM in this area.

Similarly, this interaction allowed the EMA, regulators, the EDQM and the European Pharmacopoeia Group of Experts on Blood and Blood Products to understand different manufacturers' approaches to potency labelling. Together, they will review the information discussed at the workshop and will use this as a point of reference when making future decisions for licensing or changes to the relevant European Pharmacopoeia texts for this important scientific area.

[1] Haemophilia A is a genetic deficiency in clotting factor VIII in which the ability of a person's blood to clot is impaired. People with haemophilia A need injections of factor VIII to restore the coagulation process and prevent frequent bleeds that could otherwise lead to pain, irreversible joint damage and life-threatening haemorrhages.
[2] Haemophilia B is the second most common type of haemophilia, sometimes referred to as "Christmas disease" or "factor IX deficiency." It is a largely inherited condition caused by absent or low-levels of the blood clotting factor IX, which is necessary for normal blood clotting.
[3] The European Pharmacopoeia Commission has thirty-eight members: *Austria, Belgium, Bosnia and Herzegovina, Bulgaria, Croatia, Cyprus, the Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, Montenegro, Netherlands, Norway, Poland, Portugal, Romania, Serbia, Slovakia, Slovenia, Spain, Sweden, Switzerland, the former Yugoslav Republic of Macedonia, Turkey, Ukraine, United Kingdom and the European Union.* There are twenty-six observers: *Albania, Algeria, Argentina, Armenia, Australia, Brazil, Canada, China, Georgia, Israel, Madagascar, Malaysia, Moldova, Morocco, Republic of Belarus, Republic of Guinea, Republic of Kazakhstan, Republic of Singapore, the Russian Federation, Senegal, South Africa, Syria, Tunisia, United States of America, the Taiwan Food and Drug Administration (TFDA) of the Ministry of Health and Welfare and the World Health Organization (WHO).*

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