WFH Statement on Orphan Medicine Status for Hemophilia Treatment Products

Orphan medicine laws aim to help patients with rare diseases by creating incentives for the development of treatments where none exist. With small patient populations, the costs and challenges of developing treatments may be greater than the return on investment for an approved product. In the past decades, orphan medicines laws have succeeded in encouraging and facilitating the development of treatments for rare diseases.

Hemophilia A and B are rare diseases by any definition. The first treatments for hemophilia, plasma-derived factor VIII and factor IX concentrates developed by multiple manufacturers around the world, greatly improved the lives of people with hemophilia. Then, in response to the transmission of hepatitis C and HIV in clotting factor products, manufacturers responded in two ways. First, fractionators greatly improved their processes to increase the safety of plasma-derived products; second, recombinant products were developed to avoid the risks inherent in products derived from human plasma pools.

Hemophilia differs in one significant way from other orphan diseases. Thanks to competition in the market for clotting factor concentrates, manufacturers have continued to innovate and improve the variety of available treatment products. Patients with access to sufficient levels of care value the ability to select, with their doctors, the most appropriate product for their individual treatment regimen.

Now there is a new type of clotting factor in the product pipeline, namely longer-lasting or longer-acting concentrates. Several companies are working on different techniques to achieve this goal, all using the well-characterized factor VIII or factor IX molecules modified or enhanced through combination with different chemical or biological materials to have a longer half-life in vivo.

The WFH supports the development of new and innovative treatment modalities for hemophilia A and B. We note that there is no experience with long-term therapy with these new products. Because of the different changes in structure in each of these proteins, each of these new products has the possibility to have a different risk and efficacy profile compared to existing products and to each other. Today, the greatest risk for patients receiving adequate levels of treatment is the development of inhibitors. Only good post-marketing studies will demonstrate which of the new clotting factor products gives the best clinical result with the lowest incidence of inhibitor formation.

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The WFH recommends that Orphan Drug designation should not be used to hinder, for the same condition or indication, the development, licensing and marketing of other products which have demonstrably different protein modification or enhancement. We see a danger that market exclusivity could create a monopoly rather than allowing for competition that will ensure the widest possible access at the most affordable prices to products which are actually different on the molecular level. Furthermore, the product with the orphan marketing authorization may not have the best efficacy or safety profile of the possible products. We urge regulators to consider these issues when deciding questions of market exclusivity for various products for the treatment of hemophilia.

*This statement was approved by the WFH Blood Products Safety, Supply and Availability Committee on June 13, 2012, and adopted by the WFH Executive Committee on July 8, 2012.*