



## NATIONAL HEMOPHILIA FOUNDATION

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MASAC Document #208

### MASAC RECOMMENDATION ON ORPHAN DRUGS

*The following recommendation was approved by the Medical and Scientific Advisory Council (MASAC) on May 6, 2012, and adopted by the NHF Board of Directors on June 22, 2012.*

Hemophilia A and B meet criteria for rare disorders because they occur in less than 1 in 200,000 individuals. Development of new medications for rare disorders is costly for manufacturers, who have no assurance that they will recover their costs because of the limited market for these drugs. One way to ensure that they recoup their research and development costs is to have their proposed new product designated as an orphan drug.

The Orphan Drug Act (ODA) provides for granting special status to a product to treat a rare disease. Orphan drug status qualifies the sponsor for the tax credits and marketing incentives of the ODA. More than one sponsor may receive orphan drug status for the same drug for the same rare disease. A sponsor may request orphan drug designation for a previously unapproved drug, for a new orphan indication for an already marketed drug, or for a subsequent drug for the same rare disease if it can present a plausible hypothesis that the new drug may be clinically superior to the first drug.

We know from clinical experience that some of the existing clotting factor concentrates work differently in individual patients. We will only learn if this may also be true for the new clotting factor concentrates through their long-term clinical use. Furthermore, by having a range of clotting factor concentrates available for clinical use, a clinician will have the best opportunity to ensure that the most appropriate and efficacious therapy is available for each individual patient, taking into account his/her genetic/phenotypic profile.

To date, three new recombinant FIX products and several new recombinant FVIII products under development are manufactured using different methods of protein modification or enhancement. Therefore, the method of action is considered to be different in each of the drugs. The most significant adverse event facing people with hemophilia today is the risk of developing an inhibitory antibody. Because of the different changes in structure in each of these proteins, each of these new products has the possibility to have a different inhibitor risk profile than existing products. So far, there is no experience with long-term therapy with these new products. Only good post-marketing studies will demonstrate which of these clotting factor products gives the best clinical result with the lowest incidence of inhibitor formation.

MASAC supports the development of new and novel clotting factor concentrates that offer the prospect of more efficacious therapy. However, Orphan Drug designation should not be used to hinder the development, licensing and marketing of other products for the same condition which have demonstrably different protein modification or enhancement. We see a danger that market exclusivity could potentially create a monopoly rather than allowing for market competition that would ensure the widest possible access at the most affordable price.

Therefore MASAC recommends that each new clotting factor concentrate be considered as a separate new drug under the Orphan Drug Act, based on their differing methods of protein modification or enhancement.

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