



**NATIONAL HEMOPHILIA FOUNDATION**  
*for all bleeding and clotting disorders*

February 9, 2012

Mr. Peter Lee  
Executive Director  
California Health Benefit Exchange  
2535 Capitol Oaks Drive  
Suite 120  
Sacramento, CA 95833

***Title: Comments Regarding the State's Benefit Exchange***

Dear Mr. Lee:

The National Hemophilia Foundation (NHF) is the nation's leading advocacy organization working to raise awareness of issues impacting access to care for persons affected by hemophilia and related bleeding disorders. We are writing to provide comments regarding future benefits that might be offered to our members through the state's Health Benefit Exchange.

Hemophilia and related bleeding disorders are rare, hereditary conditions in which there is an absence or impaired function of one of the proteins necessary for blood to clot. These conditions increase the tendency for excessive and spontaneous bleeding into joints and muscles, which can result in chronic pain, swelling and, if left untreated, can cause permanent damage and premature death. Hemophilia, the most well-known bleeding disorder due to the 1980s contaminated blood product fiasco, affects primarily males. Von Willibrand's disease, estimated to affect more than two million people in the U.S, the majority of which are undiagnosed, occurs equally in men and women. The other factor deficiencies are less prevalent.

There is no cure for any of these conditions. But there are highly effective therapies, called clotting factor, made from human blood plasma or non-blood sources that helps the blood clot. With proper treatment, individuals can have relatively normal lives. Treating bleeding disorders, however, can be complicated given that patients need access to both clotting factor (a biologic product for which no generics or substitutions exist) and health care specialists (both medical and pharmacy) who are knowledgeable of bleeding disorders. In addition, patients often have very diverse needs that vary based on the severity of the condition, age and the existence of other complications.

As the state considers the benefits to offer through the Exchange, it is important that provisions are made to ensure that individuals with bleeding disorders have appropriate access to care and treatment. Specifically, this means guaranteeing the following:

***1) Access to specialists at federally recognized hemophilia treatment centers (HTC)***

Established by Congress in 1974, hemophilia treatment centers (HTC) were created to provide comprehensive, specialized services for individuals with bleeding disorders and their families. These centers, which operate through a team of healthcare professionals that includes hematologists, nurses, physical therapists, social workers and dentists, provide coordinated and continuous care for

individuals with bleeding disorders.<sup>1</sup> Examples of some of the services provided by HTC include nursing, disease and case management, blood safety surveillance, and pharmacy services for centers participating in the drug discount program authorized by section 340B of the Public Health Service Act (PHSA).

Various U.S. Centers for Disease Control and Prevention (CDC) studies have found that individuals who received care through an HTC experienced significantly reduced morbidity and mortality as well as fewer complications and hospitalizations.<sup>2][3]</sup> We believe that over time this results in lower healthcare costs for both the patient and their health insurance provider.

## ***2) The full range of FDA-approved blood clotting factor products***

NHF's Medical and Scientific Advisory Council (MASAC) has found the different brands of clotting factor products to have unique characteristics that often result in varying effectiveness and tolerability among individuals, even those within the same family.<sup>4</sup> Because having timely access to products could mean the difference between life and death for some patients, it is crucial that treating physicians have the discretion to determine which product(s) would work best for a particular patient. For these reasons, it is important that patients not be subjected to formulary restrictions such as prior authorizations, preferred drug lists and step products. These policies delay access to necessary therapies and forces patients to use suboptimal therapies that may not work well for them.<sup>5</sup>

## ***3) A range of specialty pharmacy providers***

Unlike medications bought at retail pharmacies, clotting factor often requires refrigeration and specialized handling. These products and services, however, are only available through specialty pharmacies that specialize in dispensing clotting factor and whose employees are trained to recognize the varying medical and nursing needs of each patient. Given the diverse needs of patients, a network of specialty pharmacy providers must be accessible and available to help patients navigate the healthcare system and manage their conditions. However, it is also necessary for entities dispensing clotting factor products to adhere to state-of-the-art standards similar to those recommended by MASAC.<sup>6</sup>

## ***4) Adequate reimbursement for life-saving clotting factor products and specialty services***

Technological advancements have redefined how bleeding disorders are diagnosed and treated, ensuring that most children and adults today are no longer disadvantaged by having a bleeding disorder. To continue on this positive trek, clotting factor manufacturers must continue developing safer and more effective products; physicians must have the flexibility to create treatment regimens tailored specifically to each patient; and a network of specialty pharmacies must be available to dispense these unique products. All of these service providers will need to be appropriately

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<sup>1</sup> MASAC Document #132. (2002). Standards and Criteria for the Care of Persons with Congenital Bleeding Disorders. [www.hemophilia.org](http://www.hemophilia.org).

<sup>2</sup> Soucie JM et al. Mortality among males with hemophilia: relations with source of medical care. Blood 2000; 96:437-442.

<sup>3</sup> Soucie JM et al. Home-based factor infusion therapy and hospitalization for bleeding complications among males with hemophilia. Haemophilia 2001; 7:198-206.

<sup>4</sup> MASAC Document #159. (2005). Recommendation Regarding Factor Concentrate Prescriptions and Formulary Development and Restrictions. [www.hemophilia.org](http://www.hemophilia.org).

<sup>5</sup> MASAC Document #179. (2007). Recommendation Concerning Prophylaxis. [www.hemophilia.org](http://www.hemophilia.org).

<sup>6</sup> MASAC Document #188. (2008). Recommendation Regarding Standards of Service for Pharmacy Providers of Clotting Factor Concentrates for Home Use to Patients with Bleeding Disorders. [www.hemophilia.org](http://www.hemophilia.org).

compensated for the services they provide. It is thus crucial that the Exchange take into consideration the higher costs associated with treating bleeding disorders as well as those manufacturing and dispensing of clotting factor products. Not doing so could prevent patients from obtaining the necessary products and services they need to avoid potentially debilitating and costly complications that can result from less than optimal care.

Thank you for taking the time to review our comments. We would welcome the opportunity to meet with the Exchange's leadership to discuss options for our members. In the interim, if you have questions, please feel free to contact Michelle Rice, Director of Public Policy, (317) 517-3032 or [mrice@hemophilia.org](mailto:mrice@hemophilia.org); or Ruthlyn Noel, Senior Manager of Public Policy, (212) 328-3755 or [rnoel@hemophilia.org](mailto:rnoel@hemophilia.org).

Sincerely,

Val Bias  
Chief Executive Officer