



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

February 16, 2012

Texas Health and Human Services Commission  
Attn: Stacey Johnston  
Policy Analyst  
Medicaid/Children's Health Insurance Program (CHIP) Division  
P.O. Box 85200  
Austin, TX 78708-5200,

***Title: Comments on the Proposed New Rule Concerning Specialty Drugs***

Dear Commission Members:

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. The Lone Star Chapter of NHF and Texas Central Hemophilia Association (“TexCen”) assists persons affected by hemophilia and related bleeding and clotting disorders living in the State of Texas, providing education, advocacy, support services and promoting research towards a cure. We are writing to provide comments on the proposed new rule to Title 1, Part 15, Chapter 354, Subchapter F, Division 3, §354.1853, concerning Specialty Drugs.

Hemophilia and other 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, often spontaneous bleeding into joints and muscles, which can result in chronic pain, swelling and, if left untreated, can cause permanent damage and premature death. 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 one of the most expensive medication on the market for which no generics or substitutions exist and health care specialists (both medical and pharmacy) who are knowledgeable of bleeding disorders.

As the Commission works to define what constitutes a “specialty drug” as explained by the proposed rule change, **it is important to not lose sight of the bigger impact managed care could have on overall access to care for Medicaid recipients with bleeding disorders.** Particularly, the state must ensure the following regardless of whether coverage is provided through the fee-for-service program or managed care.

- 1) ***Specialists at federally-recognized hemophilia treatment centers (“HTC”)***. Established by Congress in 1974, HTCs were created to provide comprehensive, specialized services for individuals with bleeding disorders and their families. HTCs operate through a team of healthcare professionals that includes hematologists, nurses, physical therapists, social

workers and dentists.<sup>1</sup> Examples of some of the services provided by HTC's 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 had lower 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 therapies. These policies delay access to necessary treatment and can force patients to use suboptimal therapies that may not work well for them.<sup>5</sup>
- 3) ***A range of specialty pharmacy providers.*** As outlined in the proposed rule change, clotting factor often requires refrigeration, specialized handling and storage. Moreover, patients will have to be trained to infuse at home. These products and services, however, are only available through specialty pharmacies (including 340B programs) 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

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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).

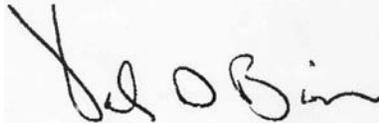
<sup>6</sup> MASAC Document #188. (2008). Recommendation Regarding Standards of Service for Pharmacy Providers of Clotting Factor Concentrates for Home Use to Patients with Bleeding Disorders. [www.hemophilia.org](http://www.hemophilia.org).

products. All of these service providers will need to be appropriately compensated for the services they provide. It is thus crucial that the Commission 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.

**We therefore urge the Commission (and Department of Health and Human Services, for that matter) to take the necessary precautions to ensure that all eligible Medicaid and CHIP beneficiaries with bleeding disorders continue to have adequate and appropriate access to care under managed care.**

Thank you for taking the time to review our comments. Should you wish to discuss our comments, please do not hesitate to contact Ruthlyn Noel, Senior Manager of Public Policy, (212) 328-3730 or [rnoel@hemophilia.org](mailto:rnoel@hemophilia.org).

Sincerely,



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