



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

July 6, 2018

Joanne LaFleur, PharmD, MSPH  
Professor and Director  
Pharmacotherapy Outcomes Research Center  
University of Utah College of Pharmacy  
30 South 2000 East Room 4922  
Salt Lake City, UT 84112

**Re: Review of Hemophilia Factor VIII therapies**

Dear Ms. LaFleur:

I am writing today on behalf of the National Hemophilia Foundation (NHF) to inquire about the upcoming July 19 Pharmacy and Therapeutics Committee review of Hemophilia Factor VIII products. NHF is the nation's leading advocacy organization for individuals with bleeding disorders. Our mission is to ensure that individuals affected by hemophilia and other inherited bleeding disorders have timely access to quality medical care, therapies and services, regardless of financial circumstances or place of residence.

NHF recognizes that the complexities involved in treating hemophilia and related bleeding disorders can result in high medical expenses for patients and their health insurance plans. While the need to identify cost containment strategies is necessary, it is critical that such strategies not compromise continuity of care for those with complex medical conditions. Hemophilia and related bleeding disorders are rare, complex genetic conditions for which there are no known cures. Individuals often experience spontaneous and prolonged internal bleeding into the joints and soft tissues. To effectively manage these disorders, patients often require life-long infusions of clotting factor therapies that replace the missing or deficient blood proteins, thus preventing debilitating and life-threatening internal bleeding. While today's therapies are safer and more effective than ever, they are also more costly than other types of medication. For example, cost of treatment for a person with severe hemophilia can reach \$250,000 per year or more.

Developing an inhibitor (i.e., an immune response to treatment) or other complications such as HIV/AIDS, hepatitis, chronic joint disease, or bleeding as a result of trauma or surgery can increase those costs to over \$1 million.

Clotting factor and non-factor replacement therapies are biological products derived from human blood plasma or by using recombinant technology for which there are no generic equivalents. Moreover, because of the nature of bleeding disorders, an individual's response and tolerability for a specific product is unique. For these reasons, NHF's Medical and Scientific Advisory



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Council (MASAC) recommends that individuals retain access to the full range of FDA-approved clotting factor products.<sup>1</sup> Limiting access through the use of restrictive drug formularies such as those requiring prior authorization, preferred drug lists, and fail first/step therapy, could have a negative impact on patient care and ultimately result in higher drug spends. Therefore, drug benefit designs employing these methods should be avoided, and the choice of product used by an individual should remain a decision between patient and physician.<sup>2</sup>

On behalf of individuals in the State of Utah affected by bleeding disorders, we urge you to prioritize the practice of allowing patient access to all FDA-approved therapies available to treat hemophilia and related bleeding disorders.

Thank you for the opportunity to share our concerns. If you would like additional information or have questions, please feel free to me at 317-517-3032 or [mrice@hemophilia.org](mailto:mrice@hemophilia.org).

Thank you,

Michelle Rice  
Sr. Vice President, External Affairs  
National Hemophilia Foundation

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<sup>1</sup> MASAC Document #253 (2018) Recommendation Concerning Products Licensed for the Treatment of Hemophilia and Other Bleeding Disorders. [www.hemophilia.org](http://www.hemophilia.org)

<sup>2</sup> MASAC Document #166 (2005) MASAC Resolution Regarding Preferred Drug Lists. [www.hemophilia.org](http://www.hemophilia.org)