

November 12, 2020

Iowa Medicaid Enterprise
Attn: Pharmacy Services
1305 East Walnut Street
Des Moines, Iowa 50319-0114

Re: Review of the Therapeutic Class - Hemophilia Treatments

Dear Iowa Pharmaceutical & Therapeutics Committee Members,

We are writing you today on behalf of Hemophilia of Iowa (HOI) and the National Hemophilia Foundation (NHF) to inquire about the upcoming November 19th Iowa Medicaid's Pharmacy & Therapeutics Committee Meeting review of the therapeutic class for Hemophilia treatments. HOI is a non-profit organization that is in Iowa City and they are dedicated to enriching the lives of those living with bleeding disorders in the state of Iowa. NHF is the nation's leading advocacy organization for individuals with bleeding disorders. Both of our missions are 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.

HOI and NHF recognize 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 the 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, the cost of treatment for a person with severe hemophilia can reach \$350,000 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 direct 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 Council (MASAC) recommends that individuals retain access to the full range of FDA-approved clotting factor products.¹ Limiting access through the use of restrictive drug formularies such as those requiring prior authorization, preferred drug lists (PDLs), 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.²

On behalf of individuals in the State of Iowa 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. However, if the state decides to manage clotting factor products we recommend the following: (1) new Medicaid patients be grandfathered in on their current medication; (2) the medication of existing Medicaid patients be included on the PDL; (3) any prior authorization criteria result in a clear, direct, and timely process that does not delay access to patient care. Also, because bleeding disorders are lifelong conditions without a cure, we request that the authorization period be as long as is reasonable to prevent any negative consequences from patients running out of medication, e.g. six to twelve months. Adopting these policies will help mitigate any potential excess costs associated with our patients not having access to the medication they need.

Thank you,

¹ MASAC Document #253 (2018) Recommendation Concerning Products Licensed for the Treatment of Hemophilia and Other Bleeding Disorders. www.hemophilia.org

² MASAC Document #166 (2005) MASAC Resolution Regarding Preferred Drug Lists. www.hemophilia.org