



## NATIONAL HEMOPHILIA FOUNDATION

www.hemophilia.org

February 21, 2013

Marilyn Tavenner, Acting Administrator  
Centers for Medicare and Medicaid Services  
Department of Health and Human Services  
P.O. Box 8016  
Baltimore, MD 21244-8016

*Re: CMS-2334-P: Medicaid, Children's Health Insurance Programs and Exchanges*

Dear Ms. Tavenner,

The National Hemophilia Foundation (NHF) is the nation's leading advocacy organization working to ensure that individuals affected by hemophilia and related bleeding disorders have timely access to high quality medical care and services, regardless of financial circumstances or place of residence. We appreciate the opportunity to provide the Centers for Medicare and Medicaid Services (CMS) our comments on the above referenced proposed rule. Our comments seek clarification regarding the proposed policies on cost-sharing for drugs and emphasize the need for the Essential Health Benefits under Alternative Benefit Plans as allowed by Medicaid state plans to include critically important services for people with bleeding disorders.

Hemophilia and related bleeding disorders are rare, complex genetic conditions for which there are no known cures. Individuals experience spontaneous and prolonged internal bleeding in the joints and tissues, which can be disabling and life-threatening. To effectively manage these disorders, patients require life-long infusions of clotting factor therapies to replace missing or deficient blood proteins. These therapies are safer and more effective than ever, but also very expensive. Drug costs for a person with severe hemophilia can be \$300,000 a year or more. Developing an inhibitor (an immune response to treatment), complications such as HIV/AIDS, hepatitis and joint diseases, or bleeding as a result of trauma or surgery can increase those costs to \$1 million.

### **Cost-Sharing for Drugs**

The proposed rule offers flexibility for states to require higher cost-sharing for drugs that are non-preferred, which could be as high as 20% of the drug's cost for people with incomes above 150% of the Federal Poverty Level (FPL). Clotting factor therapies are biologic products with no generic equivalents, so they typically do not appear on preferred drug lists. Families with income at 150% of the FPL simply cannot afford to pay 20% of the cost. Moreover, individuals with bleeding disorders could reach the 5% of income limit on out-of-pocket spending by filling their first prescription. Requiring individuals with bleeding disorders to pay 20% of the cost of their drugs could impede access to their life-saving treatment.

Under current Medicaid rules, CMS allows patients to access non-preferred drugs at lower cost sharing rates if the prescribing physician determines that a therapeutically equivalent or similar preferred drug is less effective or would cause adverse effects. **We ask that this policy also apply when a patient is prescribed drugs on a non-preferred list without therapeutically-similar versions on the preferred drug list if the physician determines the treatment is medically necessary.**

We believe that the choice of therapy should be made by patients working with their physicians, and not based on cost-sharing requirements. We respectfully request that CMS not restrict access to these treatments through cost-sharing requirements that patients will not be able to meet. In addition, please ensure that any appeals process related to cost sharing requirements be timely and not impede access to care. Rapid time-to-

treatment is critical for people with bleeding disorders to avoid complications and unnecessary hospitalizations.

### **Essential Health Benefits**

With respect to Alternative Benefit Plans, we would like to emphasize the importance of defining the Essential Health Benefits so it is adequate for people with bleeding disorders, which includes:

#### *Access to Specialists at Hemophilia Treatment Centers (HTCs)*

Since 1974, Congress has authorized and funded a national network of HTCs to provide comprehensive, specialized care for individuals with bleeding disorders. These centers are comprised of multi-disciplinary providers including hematologists, physical therapists, nurses, dentists and social workers that work as a team to provide coordinated care for this complex patient population. In addition to disease and case management, HTCs monitor blood safety and offer educational programs for individuals and their families. Studies show that individuals that receive care at HTCs have a 40% reduction in morbidity and mortality rates, despite the fact that more severe patients are seen at HTCs. Patients who use HTCs also experience fewer long-term complications and hospitalizations, increasing quality of life and reducing total healthcare care costs.

The ACA specifies that entities covered under section 340B(a)(4) of the Public Health Service Act (which includes federally recognized HTCs) be designated as essential community providers. This designation requires qualified health plan networks to include HTCs, ensuring access to these specialized healthcare providers. To facilitate continuity of care as low income individuals move between Medicaid and subsidized private plans, we respectfully request that state Medicaid programs also be encouraged or required to include essential community providers in their plan networks.

#### *Access to the full range of FDA approved clotting factor products*

Due to the nature of bleeding disorders, an individual's response and tolerability for a specific clotting factor therapy is unique. For these reasons, NHF's Medical and Scientific Advisory Council (MASAC) recommends that individuals have access to the full range of available clotting factor products. Limiting access through the use of restrictive drug formularies, such as requiring prior authorization and preferred drug lists, negatively impacts patient care. We believe that the choice of therapy should be made by patients working with their physicians.

#### *Access to a range of specialty pharmacy providers*

Unlike other types of medication typically bought at a retail pharmacy, clotting factor therapies require special handling, shipping and refrigeration. Additionally, patients often require other products (i.e., syringes, saline), nursing services, and intensive education to manage their complex health condition. These requirements are beyond the ability of a traditional retail pharmacy. These services are only available through specialty pharmacy providers that are specially trained to handle the unique needs of the bleeding disorder community and are expected to adhere to high quality standards. Considering the variability of patient needs and provider services, patients need access to a network of pharmacy providers to properly manage their conditions allowing patients to live longer, healthier lives.

Thank you for your consideration. If you would like more information, please contact our Washington Policy Advisor, Ellen Riker, at [eriker@dc-crd.com](mailto:eriker@dc-crd.com) or 202-484-1100.

Sincerely,



Val Bias  
Chief Executive Officer