

July 16, 2018

The Honorable Alex M. Azar, II
US Department of Health and Human Services
200 Independence Ave., SW
Room 600E
Washington, DC 20201

Re: *HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs*, [RIN 0991-ZA49](#)

Dear Secretary Azar:

The National Hemophilia Foundation (NHF) and Hemophilia Federation of America (HFA) are national non-profit organizations that represent individuals with bleeding disorders across the United States. 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.

We are writing to submit comments in connection with your Department's Request for Information (RFI) regarding the HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs. As patient advocacy organizations, our particular expertise and our members' interests center on a particular subset of the drug pricing question: patient access and patient spending. Our comments will focus on the cost issues that create obstacles for patient access to the medications that are so essential to their lives.

About Bleeding Disorders and their Current Treatments

Hemophilia is a rare, genetic bleeding disorder affecting about 20,000 Americans that impairs the ability of blood to clot properly. Without treatment, people with hemophilia bleed internally, sometimes as a result of trauma, but sometimes simply as a result of everyday activities. This bleeding can lead to severe joint damage and permanent disability, or even – with respect to bleeds in the head, throat, or abdomen – death. Additional related bleeding disorders include Von Willebrand disease (VWD), another inherited bleeding disorder, which is estimated to affect more than three million Americans.

People with hemophilia administer prescription clotting factor or other anti-hemophilia medication to prevent or treat painful or potentially life-threatening bleeding episodes. People who develop inhibitors (an immune intolerance to regular treatment) require additional treatment options beyond clotting factor, including bypassing products, recombinant factor VIIa, and/or emicizumab. VWD is treated by DDAVP (desmopressin acetate) and clotting factor concentrates that are rich in von Willebrand Factor.

Bleeding disorders treatments are biological products derived from human blood plasma or created by recombinant technology for which there are no generic equivalents. Moreover, because of the nature of bleeding disorders, products are not interchangeable and 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. Ultimately, patients working with their physicians must decide the most effective treatment option. Limiting access through the use of restrictive drug formularies can negatively affect patient care and ultimately result in higher health care spending.

Bleeding disorders treatments today are very effective, and very expensive. The annual cost of clotting factor and other bleeding disorder treatments often exceeds \$250,000 for those with severe hemophilia and treatments for those with inhibitors can be much higher. Due to these costs, people with bleeding disorders have significant out-of-pocket expenses each year to access their drugs. In fact, our patients typically meet their out-of-pocket maximum each year, sometimes within the first few months of the year. There is no way to incentivize hemophilia patients to use a less expensive treatment, because there are no cheaper treatments – no generics – available to treat hemophilia. Individuals with severe hemophilia have no choice but to use hundreds of thousands of dollars’ worth of clotting factor each year, for life, in order to prevent or treat bleeding episodes and preserve their health.

Comments on the RFI Proposals

The RFI proposes policies and raises questions in a number of areas that would affect the bleeding disorders community. Due to our high costs, our patient community is very interested in ideas that will lower patient out-of-pocket costs. However, we are concerned about the potential for unintended consequences that may result from some of the proposals in the RFI. We respectfully make the following comments by topic of the RFI:

Part B Coverage for Bleeding Disorders Treatments

By statute, clotting factor and other bleeding disorders treatments are covered under Medicare Part B, whether the products are self-administered or provided incident to a physician service. Optimally, a person with a bleeding disorder self-administers his or her medicine at home to assure the promptest and most clinically- and cost-effective treatment. Individuals who cannot self-infuse may visit clinical settings for outpatient infusion. Coverage under Part B allows individuals to access treatment in all of these settings.

We are very concerned about the proposal to move drugs covered under Part B to Part D and believe that this would jeopardize access to treatment for our patient community for several reasons:

First, bleeding disorders treatments have been covered under Part B since they were developed. As a result, our organizations have focused on Medicare Part B and Medigap coverage and policies to ensure patient access to needed costly treatments. In fact, we are concerned that people with hemophilia may not be enrolled in Part D drug plans at all, or if they are, they may have chosen narrow coverage since they were choosing a plan to access more common medications, not their bleeding disorders treatments. As a result, moving treatments to Part D would impede patient access to treatments, and could significantly increase patient costs.

Second, if moved to Medicare Part D, it is unclear whether the additional unique Medicare coverage and payment policies that Congress and CMS have implemented for bleeding disorders treatments would still apply. In the 1990s, Congress established a per-unit furnishing fee under Medicare (section 1842(o)(5) of the Act) to cover the costs incurred in the delivery of clotting factor to an individual, including special inventory management and storage requirements, and in providing the ancillary supplies and patient training necessary for the self-administration of the products. Currently set at \$0.215 per unit, the furnishing fee recognizes the significant costs that providers incur in dispensing

bleeding disorders treatments. These policies work together to ensure that our vulnerable patient community has access to life-saving treatments.

In addition, Part D drug formularies are typically very narrowly managed, while Part B coverage provides for open formularies. People with bleeding disorders respond differently to different treatments, which requires access to a broad range of treatments. Patients who are forced to switch to a less effective product may experience breakthrough bleeding, which imperils their health, requires yet more treatment (at a higher cost) to resolve, and can require hospitalization. Elsewhere in the RFI, you propose to increase flexibility for Part D formularies and to give pharmacy benefit managers (PBMs) more authority to set rules for the program's coverage. If bleeding disorders treatments were moved into Part D, we are concerned that these additional policies would make it even more difficult to access needed care. These approaches would also increase patient out-of-pocket costs, as well as costs for Medicare overall, both of which are in opposition to the stated goals of the blueprint.

Accordingly, we are very concerned that your proposal to move Medicare Part B drugs into Medicare Part D would jeopardize access to care for our patient community. We respectfully urge you not to finalize this policy.

Medicaid Demonstration to Test Limited Formularies

We are similarly concerned about your proposal to allow five states to test limited formularies in their Medicaid programs. NHF's MASAC has recognized that limiting access through the use of restrictive drug formularies such as those requiring prior authorization, preferred drug lists, and fail first/step therapy, could harm patient care and health (ultimately resulting in higher health care spending). Therefore, MASAC recommends that 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.¹ We joined with others in the patient community to oppose the recent waiver proposed by Massachusetts, which would have limited coverage to one drug per category or class. We ask that you not move forward with this demonstration or other efforts that would allow for restrictive formularies in Medicaid.

Importance of 340B Program to Hemophilia Treatment Centers

The RFI asks several questions about the 340B Drug Discount Program and suggests that the Administration is considering a number of policies to change which entities or patients are eligible for the program. We wish to affirm the importance of the 340B program to hemophilia treatment centers (HTCs) and ask that you not finalize any policies that would limit the benefits of the program for HTCs and the bleeding disorders patients they treat.

Most people with bleeding disorders receive medical care from the national network of 135 HTCs, which specialize in treating bleeding disorders and their long-term complications, including inhibitors, liver disease and HIV/AIDS. HTCs provide access to comprehensive, multidisciplinary, specialized care for people with bleeding disorders. Studies from the Centers for Disease Control and Prevention (CDC) have

¹ MASAC Document #166 (2005) MASAC Resolution Regarding Preferred Drug Lists.
<https://www.hemophilia.org/Researchers-Healthcare-Providers/Medical-and-Scientific-Advisory-Council-MASAC/MASAC-Recommendations/MASAC-Resolution-Regarding-Preferred-Drug-Lists>

shown that mortality and hospitalization rates are 40% lower for people who use HTC than for those who do not, despite the fact that more severely affected patients are more likely to be seen in HTCs. HTCs have operated with federal authorization and funding for more than 40 years, receiving funding from the CDC and the Health Resources and Services Administration's (HRSA) Maternal and Child Health Bureau.

Designated as original covered entities when the 340B program was created in 1992, HTCs are eligible to participate in the 340B Drug Discount Program via their HRSA grant. Grantees are provided access to 340B to "stretch scarce federal resources" to provide comprehensive services to their patients. Currently, the approximately 100 HTCs that participate in the 340B program are required to reinvest any program income generated to provide services for their bleeding disorders patients in accordance with their grant. An analysis performed by the HRSA-funded National Hemophilia Program Coordinating Center demonstrates that HTCs are indeed reinvesting their 340B program income to support the provision of essential patient services. This analysis found that 340B program income supported 569 full-time equivalents (FTEs) at 83 HTCs and an average of more than 5,500 telephone triage, care coordination and case management patient encounters per center.

Finally, we especially want to express concern about rumored changes to 340B that would limit the definition of an eligible patient to only those who are uninsured. People with bleeding disorders simply have to maintain insurance, due to their high costs and life-long condition. Limiting the use of 340B to only uninsured patients would essentially disqualify HTCs from participating in the program, jeopardizing their funding and the infrastructure of this national network of treatment centers. Please do not finalize this or any other proposals that would eliminate the benefits of the program for HTCs and our patient community.

Medicaid and Gene Therapy

Hemophilia is one of a handful of chronic diseases where gene therapy clinical trials are currently underway. These therapies hold the potential for improvements in quality of life for patients with the severest form of the disorder as well as a reduction in cost of treatment. Patient quality of life improvements might include an increase in the ability to be an active member of the workforce and a contributing member of society, leading to a reduced dependence on federal and state based programs. Our organizations recognize that gene therapy will be expensive, however, the opportunity to realize reductions in costs of treatment over the lifetime of a patient could be significant with "break even" occurring within 2-5 years depending on the manifestation of the disease. We are very interested in working with policymakers to design reimbursement strategies that are fair and equitable, and provide drug manufacturers with incentives to continue research and development while enabling payers to have balanced risk management protocols.

Copay Discount Cards

The RFI asks whether the use of manufacturer copay cards helps lower consumer cost, or (alternatively) helps drive manufacturer price increases.

Copay cards are a response to health plans' increasing use of tiering, high deductibles, and coinsurance. Insurers' stated aim, in adopting these cost-shifting tactics, is to encourage patients to try low-cost medications before they resort to higher-cost drugs. But patients with bleeding disorders have no lower

priced alternatives, and no generic options, to use in place of their specialty medications. As a result, high deductibles and coinsurance place patients who use hemophilia medications in a precarious situation: these patients may have to spend thousands of dollars every year (often, at the beginning of the plan year) in order to obtain the medications they need. Because there is, as of now, no cure for bleeding disorders, patients must bear these crushing out-of-pocket costs associated with their essential therapies year-in and year-out, for life.

In the bleeding disorders community, manufacturer assistance programs, including copay cards, therefore provide patients with critical support in accessing their life-saving medications. Copay assistance programs help patients access their essential medications and adhere to the treatment plans that preserve their health. We also note that the arguments marshalled against copay programs don't hold true in the bleeding disorders context. Copay cards **don't** increase demand for or use of hemophilia therapies: the medical needs of the disorder are undeniable and do not disappear if financial support evaporates. Copay programs **don't** drive hemophilia patients toward more expensive therapies: all available therapies are expensive, and cheaper alternatives do not exist. Copay programs **do** make it easier for hemophilia patients to adhere to doctors' prescribed course of treatment, reducing the likelihood of adverse health outcomes and increased expense from patient non-adherence to their prescribed plan of treatment.

In 2018, some people with bleeding disorders are already experiencing financial hardship from a new insurer/PBM strategy that limits the value of manufacturer copay assistance. Some insurers and PBMs have begun implementing "accumulator adjustment programs" (AAPs). Under an AAP, the plan accepts manufacturer copay assistance from the patient but then doesn't credit that amount toward the patient's overall deductible. As a result, patients with chronic and expensive disorders still have to personally pay deductibles, copays, and other out-of-pocket expenses up to the yearly out-of-pocket maximum, even as the health plan draws down the full value of the copay card. AAPs are often couched in confusing language and can surprise even savvy insurance purchasers – leaving them exposed to unexpected costs that can prevent them from filling their prescriptions and adhering to their plan of treatment. NHF and HFA have joined with other patient groups in asking state insurance commissioners and other lawmakers to look into this harmful practice, and we respectfully ask you to do the same.

Biosimilars

The RFI poses a series of questions relating to biosimilars, focusing on how to improve the efficiency of the biosimilar and interchangeable product development and approval process. As noted, individuals with bleeding disorders depend on biological therapies (clotting factor or newer, non-factor products) to treat their disorder. This dependence on biologics makes the safe development, approval, and use of biosimilars of factor products an extremely vital issue for individuals with bleeding disorders.

Biosimilars are not generic versions of the original biologic they seek to mimic. Biosimilars may be less expensive than current biologics available and thus hold out the promise that more patients could have access to less expensive options. But easier access to biosimilars also comes with risks to patient safety that requires rigorous review and evaluation. As patient advocacy organizations, we strongly urge HHS and FDA not to slight patient health and safety in the pursuit of cost savings.

Accordingly, we oppose policies that promote automatic substitution of a biosimilar for an innovator product. Since biosimilars cannot be exact copies of biologics, they should not be automatically

substituted without the fully-informed permission of patients and their providers. Each patient's immune response and associated health risks are unique, so substitution cannot be made safely without input from both groups. If the practice of automatic substitution is adopted through either legislative or regulatory pathways, both physician and patients should be notified of the intent to switch products. Providers must be able to observe how the medicines interact with and affect their patients.

The hemophilia community was ravaged by HIV and HCV due to tainted clotting products produced in the 1980s. This unique and devastating experience bears witness to the need for maintaining stringent FDA review and approval processes. No community should have to bear what happened to patients with hemophilia because of an inadequate drug regulatory regime.

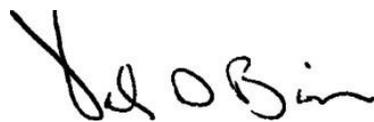
Affordable Care Act Patient Protections

Finally, as you consider policies that seek to reduce patient out-of-pocket costs, we urge you to maintain existing Affordable Care Act (ACA) patient protections that ensure access to insurance coverage and protect patients from financial ruin. The requirement that plans offer comprehensive coverage to people with pre-existing conditions and the out-of-pocket limit and the ban on lifetime and annual limits are foundational to ensure that people with bleeding disorders can access treatment and be protected from exorbitant costs. These policies likewise require a robust definition of the essential health benefits (EHBs), since the financial protections only apply to spending on EHB services. We respectfully request that you do not finalize any policies that undermine these vital protections.

Conclusion

We appreciate the opportunity to provide these comments on your efforts to reduce drug prices and patient out-of-pocket costs. We seek to ensure that all people with hemophilia and other bleeding disorders have access to the treatments they need to lead healthy, successful lives. Please contact Michelle Rice, NHF's Senior Vice President for External Affairs and Kim Isenberg, Vice President for Policy, Advocacy and Government Education at HFA with any questions.

Sincerely,



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