



NATIONAL HEMOPHILIA FOUNDATION

for all bleeding disorders

The Honorable Andrew M. Slavitt
Acting Administrator
Centers for Medicare & Medicaid Services
Hubert H. Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

May 9, 2016

RE: CMS-1670-P, Medicare Program, Part B Drug Payment Model

Dear Acting Administrator Slavitt:

The National Hemophilia Foundation (NHF) appreciates the opportunity to comment on the Medicare Part B Drug Payment Model released in March. 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.

Overall, NHF is very concerned with the breadth and short timeline for implementation of the demonstration, which was released without input from patient and provider stakeholders. Due to the lack of data comparing treatments and the potential for harm for our patients, NHF respectfully requests that CMS delay implementation of Phase 1, and exclude hemophilia patients and treatments from Phase 2 of the demonstration.

About Bleeding Disorders

Hemophilia is a rare, chronic bleeding disorder affecting approximately 20,000 people in the US, who infuse high-cost clotting factor therapies to replace missing or deficient blood proteins. These complex plasma-derived or recombinant biologic therapies are safer and more effective than ever, but are also very expensive. Drug costs for a person with severe hemophilia can be \$250,000 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. There are also similar bleeding disorders, like von Willebrand Disease, that affect up to 1 million Americans.

The CDC estimates that 70% of people with bleeding disorders receive care at hemophilia treatment centers (HTCs), where a multi-disciplinary team provides comprehensive, highly-specialized care to assess and provide treatment for the long-term complications of bleeding disorders including inhibitors, liver disease and HIV/AIDS. Studies from the CDC have shown that mortality and hospitalization rates are 40% lower for people who use HTCs than in those who do not, despite the fact that more severely affected patients are more likely to be seen in HTCs. Approximately 140 HTCs across the country receive federal grants from the Health and Resources Services Administration's Maternal and Child Health Bureau (HRSA MCHB) and the Centers for Disease Control and Prevention (CDC). HTCs are also eligible to participate in the 340B Drug Discount Program and many have pharmacy programs that dispense clotting factor to their patients.

Recent data from the CDC hemophilia surveillance program indicates that approximately 8% of the hemophilia population is on Medicare. Clotting factor and other plasma-derived and recombinant products

used to treat bleeding disorders are covered under Medicare Part B and would be included in the demonstration program. In the 1990s, the Medicare law was amended to create a per-unit furnishing fee (section 1842(o)(5) of the Act) to cover the costs incurred in purchasing, storing, and dispensing clotting factor.

Comments on the Demonstration

Phase 2 – Value-Based Purchasing

The value-based purchasing (VBP) methodologies CMS is considering for Phase 2 are inappropriate for the bleeding disorders community and likely will impede access to appropriate treatments for our population. Each VBP strategy mentioned in the rule, such as reference and indication-based pricing, advantages one product over others, which makes it significantly more challenging to access these other products.

CMS has asked for comments regarding which therapies or patient communities are ripe for inclusion in the VBP methodologies of Phase 2. **Given our small patient population on Medicare, lack of data comparing clotting factor treatments, and the potential harm that could result from patients having to switch products or not being able to access the most appropriate product, NHF asks that CMS exclude clotting factor from the VBP methodologies.** For example:

- Bleeding disorders treatments are complex, biologic products that are not interchangeable. Due to the nature of clotting factor therapies and an individual patient's metabolic rate and other reactions to certain medications, not every clotting factor product works effectively for every patient. As a result, people need access to the specific treatment as prescribed by their physician. If patients are forced to use a particular product that is not as effective for them, they could require more medication to prevent or control bleeding episodes, thereby driving up costs, or potentially suffer additional adverse health outcomes.
- There is no data comparing the effectiveness of different clotting factor treatments, so VBP strategies would have to select favored products based on price. This may lead a provider to stop dispensing other product options. This would complicate even the policy idea to discount or eliminate patient cost-sharing, because it is unclear how CMS will define "high in value." If this is purely based on price, then people may be forced to pay the 20% or switch products to access the lower cost-sharing.
- Lack of timely access to effective therapy can lead to adverse health outcomes. Delayed treatment puts patients at greater risk for an increased number of bleeding episodes, hospitalizations, joint disease and in worst case scenarios, premature death. These adverse events also lead to higher costs for payers, so while VBP strategies may allow CMS to save money on clotting factor reimbursement, it will undoubtedly see higher Part B costs in other ways.
- The size of the patient population on Medicare and number of hemophilia treaters are both extremely small. Recent CDC data indicate that approximately 1,500 individuals with hemophilia are Medicare beneficiaries, the majority receiving their care at one of the approximately 140 HTC. Moreover, neither the patient population nor treatment center locations are randomly distributed across the country. Due to the limited patient population and distribution of HTCs, it is unlikely that CMS will have a sufficient number of patients and treaters in each arm of the demonstration to have enough power to make statistically significant inferences.

Clotting Factor Furnishing Fee

CMS also seeks to waive the provision in the Medicare law that establishes the clotting factor furnishing fee. This furnishing fee is critical to ensuring that providers of clotting factor can cover their significant costs. To ensure maximum efficacy, clotting factor concentrate must be kept refrigerated and as such should be shipped overnight directly to the patient's home where it can be signed and verified. Providers also need to stock an inventory of clotting factor products in a wide range of assay sizes, and staff 24-hour, 7-day-a-week telephone lines to respond to physician and client questions and emergency requests.

Eliminating or significantly reducing the furnishing fee would jeopardize access to clotting factor therapies. Patients may be forced to switch specialty pharmacy providers to ones with less expertise in hemophilia. Alternatively, if CMS were to vary the furnishing fee as a way to incentivize the use of treatments with the most "value," then providers may only stock some but not all factor products, which will raise the concerns addressed earlier in this letter. **NHF respectfully requests that CMS maintain the statutory clotting factor furnishing fee to ensure that providers dispensing clotting factor are able to cover their costs and continue to serve our patient population.**

Process Comments

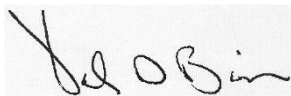
NHF joins many other stakeholders in the patient and provider communities to raise process concerns about the demonstration. For example, the broad, national scope of the demonstration and rapid implementation time frame means reimbursement for 75% of the nation's providers will be changing in as soon as 6 months, which is not enough time for patients and providers to anticipate and mitigate any access challenges that may result.

In addition, while CMS states that it seeks to test reimbursement methodologies that will reduce costs while "preserving or enhancing the quality of care provided to Medicare beneficiaries," the lack of patient safeguards and quality outcomes included in the rule is very troubling. There is little transparency regarding how CMS will measure quality and assess whether it is maintained or if patient access issues result. Before finalizing this proposal, NHF asks that CMS engage patient and provider advocates to better understand how patient communities – and individual patients themselves – may be harmed by the proposal. This is critical to CMS ensuring that quality care for patients is preserved.

Conclusion

Thank you for the opportunity to submit comments on the demonstration, which will have wide-ranging and potentially detrimental effects on our Medicare population. If you have any questions, please contact Johanna Gray, NHF's Federal Policy Advisor, at jgray@dc-crd.com.

Sincerely,



Val Bias
Chief Executive Officer
National Hemophilia Foundation