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NEW ENGLAND BLEEDING DISORDERS ADVOCACY COALITION
New England. Together.

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

HFA
Hemophilia Federation of America

To the Drug Utilization Review Committee (DUR Committee):

My name is Joseph Zamboni and I currently serve as the Advocacy Coordinator for the New England Bleeding Disorders Advocacy Coalition, also known as NEBDAC. I am here to submit this joint statement on behalf of NEBDAC, the Hemophilia Alliance of Maine (HAM), the National Hemophilia Federation (NHF) and the Hemophilia Federation of America (HFA). NEBDAC is a coalition of bleeding disorder advocacy organizations across New England. HAM is Maine's only bleeding disorder advocacy organization. NHF and HFA are a nation-wide community-based, patient-led advocacy groups representing those with hemophilia and other bleeding disorders.

We have learned that MaineCare is planning to review two novel hemophilia therapies. We are here to urge the DUR Committee to adhere to the longstanding practice (widely followed across state Medicaid programs) of carving out hemophilia therapies from the standard drug utilization review/preferred drug list (PDL) process. Limiting product options for individuals with bleeding disorders via PDLs or otherwise would put patient health at risk and could actually result in higher overall medical costs with respect to this patient population.

Our organizations understand that the Program is necessarily concerned with containing costs. However, while hemophilia treatment is undeniably expensive, limiting product options for patients with bleeding disorders is neither an effective nor a therapeutically appropriate way to manage this class of patients. It has always been the case that hemophilia treatments vary in a number of important respects, including half-life and immunogenicity, and as such are not therapeutically equivalent or interchangeable. That was true when the only treatment option available was clotting factor replacement with plasma-derived or recombinant clotting factor products. It remains true now that new products with novel mechanisms of action are becoming available to treat hemophilia. It remains the case, too, that **no generic hemophilia treatment exists.** Patient bleeding patterns and responses to different products vary widely.

Recognizing the diversity among available hemophilia treatments, the Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation has stated that product selection for bleeding disorder patients "require[s] a complex decision making process" between a patient and his or her physician: "it is critical that the bleeding disorder community has access to a diverse range of therapies and that prescriptions for specific clotting factor concentrates are respected and reimbursed."[1] Because the selection of the medically-optimal product for each

[1] National Hemophilia Foundation, Medical and Scientific Advisory Council. *MASAC Recommendation Regarding Factor Concentrate Prescriptions and Formulary Development and Restrictions,* Document #159. Accessed September 27, 2017. MASAC Document #159

patient is so individualized and so important, MASAC urges third-party payers to cover whichever factor product is prescribed by the patient's treating physician rather than resorting to a PDL or formulary approach.[2]

These concerns have become even more pressing with the entry to market of novel therapies that represent a major departure from the clotting factor products previously available to treat hemophilia. The new products may fill unmet needs for some hemophilia patients who experience bleeding that is not well-controlled by other hemophilia therapies.

Since August of 2015 HFA has been collecting data to determine how access issues impact patient health outcomes. Through an HFA initiative named Project CALLS, patients can report insurance issues affecting their access to care such as prior authorization, preferred drug lists/formularies, or step therapy. Data from Project CALLS shows that 52% of patients who experience an insurance issue of this type delay their care by either not treating when they are supposed to, or delaying a visit to their provider. When people with hemophilia delay care, they experience excess bleeds that can cause long term joint damage or other serious, more expensive health issues. Data from Project CALLS suggests that insurance issues that impede access lead to more expensive, negative health outcomes. We would expect the same results when state Medicaid programs implement PDLs, and place strict prior authorization or step therapy procedures in the way of access to hemophilia treatments. (*For more information on Project CALLS, and for the most recent CALLS data, please click here.)*

In order to best serve the medical needs of Maine's small but vulnerable population of bleeding disorder patients, our organizations respectfully request that the Program provide access to every available treatment for people with bleeding disorders. No one-size-fits-all product works equally well for all individuals with bleeding disorders. Placing barriers in the way of patient access to doctor-prescribed treatments can delay care, harm patient health, and ultimately raise costs for the payer.

Thank you for your consideration. If you have any questions, please do not hesitate to call any of the undersigned.

Jill Packard
Executive Director
HAM

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[2] National Hemophilia Foundation, Medical and Scientific Advisory Council. *MASAC Recommendation Regarding Factor Concentrate Prescriptions and Formulary Development and Restrictions,* Document #153. Accessed April 9, 2018. MASAC Document #153