

And the proposed change in criteria says, "despite prophylactic factor replacement." According to Item 2) of the Suggested Guidelines found in the National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) Recommendation #114 dated April 16, 2001 (attached), patients on primary prophylaxis were asked to consider reducing or suspending the dose of medication due to the extreme shortage of factor. Even if plenty of product was available for prophylactic treatment, consideration has to be given to increased claim amounts billed to and paid by insurance companies leading to maximization of lifetime caps sooner than necessary. Prophylaxis is not a universal standard of treatment, especially in this era of factor shortages. While I hope the factor shortage does not exist for the long term, a requirement for prophylactic treatment should not be included in the language.

Proposed 7.00 G. (3) (a and c) - Disorders of Hemostasis

How will the Administration define "frequency" or "severity"? In the Case Study presented, the patient bled frequently and with enough severity to threaten the mobility of the affected joint by decreasing range of motion and increasing pain. However, it took two appeals (and comparative x-rays) before the Administrative Law Judge ruled favorably for the patient, and all the while he bled frequently and severely. I would urge the Administration to use the broadest scope in defining frequency and severity.

Other complications besides inhibitors, intrusiveness to treatment, limitation of function, joint deformity and intracranial bleeding exist for a person with a bleeding disorder. It is a well-known fact that a high percentage of patients over the age of 15 have been infected with HIV and hepatitis through contamination of plasma-derived blood products used for treatment of the bleeding disorder. In the Case Study, the patient has Hepatitis C from this very thing. There is no vaccine for Hepatitis C, and someone infected will continue to experience hepatic problems associated with it.

Another "complication" is the real threat found in the subtle discrimination shown in the workplace. In my Case Study, the patient lost his job because of the amount of time required to take care of himself and his medical needs. He became "disabled" when he lost the means (wages and insurance) to do this. He became "disabled" by the system where no other employer would hire him because of the monetary risk involved versus hiring an "able-bodied" person. I would urge the Administration to consider the broader range of complications involved with a bleeding disorder.

Proposed 7.03 C. - Category of Impairments, Disorders of Hemostasis

While the patient in my Case Study does not have von Willebrand Disease, I do not see a valid reason for requiring hospitalization for more than 24 hours, occurring at least 3 times in a 12-month period in considering the disability of a person with vWD. In my 12 years experience, I have never encountered this scenario. However, I have seen the effects of this disease in the joints and muscles of persons with vWD; crutches were needed, knees or ankles fused together, quality of life diminished. I have seen a thigh bleed swell up to a circumference of 36 inches leading to repeated immobility and possible morbidity if left untreated. The standard of care in treating these results did not require routine hospitalization. I would submit that the criteria to ascertain disability be stated the same for both disease states.

While I am one small voice loudly advocating for patients who turn to the Social Security Administration in a desperate, final plea for help to live with their disability, I, undoubtedly along with others, want to commend the Administration for its past efforts in raising the standard of care for all persons with a bleeding disorder and for allowing comments to be made in this present effort. I hope you will find my comments (based on my experience) helpful as you work on this daunting task. Thank you.

Sincerely yours,



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MASAC Recommendation #114
Adopted by the NHF on April 16, 2001

These guidelines are not optimal therapy for patients with bleeding disorders and are intended only for the period of extreme shortage. Once the recombinant factor VIII shortage has eased, it is recommended that treaters return to previous prescribing practices for treatment of acute bleeding episodes, prophylaxis, and immune tolerance induction. NHF continues to recognize therapy with recombinant product as the standard of care, as does the U.S. Dept. of Health and Human Services.

We encourage all concerned parties to communicate freely in order to best alleviate product shortage and trust that as a community, we can concentrate efforts to ensure that product is available for those most in need. We also encourage Concerned parties to communicate their concerns and fears to industry and the government.

SUGGESTED GUIDELINES

- 1) Follow **MASAC** guidelines provided in the recent Medical Advisories #377 and 379 including curtailment of all elective surgery. For non-elective surgery and emergent in-patient bleeding management, strongly consider the use of continuous infusion of factor VIII, a strategy that has been shown to be safe and effective while reducing the total amount of infused product.
- 2) For patients on primary and secondary prophylaxis utilizing rFVIII, reduce the overall dose administered through one or a combination of the following steps:
 - a. Reduce the dose per infusion prior to reducing dosing frequency, remembering that the number of units per vial size available may make such adjustments more difficult. **All** units of every vial reconstituted must be infused.
 - b. Consider a temporary suspension of secondary prophylaxis in patients whose recent bleeding pattern has been minimal.
 - c. Measure the nadir factor VIII level (the level just prior to the next scheduled dose) to precisely define the minimum dosage necessary to achieve **1-2%** trough levels.
 - d. Shorten the interval between infusions but use significantly lower doses per infusion (e.g., change from three times a week to every other day at a lower dose).
- 3) For patients on immune tolerance induction (ITI):
 - a. **All** patients on ITI should have their regimens carefully reviewed towards decreasing doses wherever feasible without jeopardizing patient care.
 - b. Conduct recovery/survival studies (or at a minimum, inhibitor titers) every two months to reassess the inhibitor status.