



NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

MASAC Document #241
(Replaces #179)

MASAC RECOMMENDATION CONCERNING PROPHYLAXIS
(Regular Administration of Clotting Factor Concentrate to Prevent Bleeding)

The following recommendation was approved by the Medical and Scientific Advisory Council (MASAC) on February 27, 2016, and adopted by the NHF Board of Directors on February 28, 2016.

In view of the demonstrated benefits of prophylaxis (regular administration of clotting factor concentrate to prevent bleeding) begun at a young age in persons with hemophilia A or B, MASAC recommends that prophylaxis be considered optimal therapy for individuals with severe hemophilia A or B (factor VIII or factor IX <1%). (1-4) Prophylactic therapy should be instituted early (prior to the onset of frequent bleeding), with the aim of keeping the trough FVIII or FIX level above 1% between doses. Optimal dosing and frequency should be determined for each individual by appropriate laboratory monitoring. (5) It is also recommended that individuals on prophylaxis have regular follow-up visits to evaluate joint status, to document any complications such as inhibitors, and to record any bleeding episodes that occur during prophylaxis.

There are no clear cut guidelines as to when to stop prophylaxis. Joint bleeds with subsequent joint destruction are a lifelong problem for these individuals. (6) Therefore, they may continue to benefit from prophylaxis throughout their life.

As always, a careful analysis of health risks and benefits must be performed by consumers and their healthcare providers. After a thorough discussion with their medical team, persons with hemophilia and their families should decide if prophylaxis is appropriate for them or their child. This decision should be evaluated periodically, particularly in light of emerging data and changes in bleeding pattern and clotting factor usage.

As is the case with all recommendations, MASAC will periodically reexamine this recommendation as new data emerge.

References:

1. Petrini P, Lindvall N, Egberg N, Blombäck M: Prophylaxis with factor concentrates in preventing hemophilic arthropathy. *Am J Pediat Hematol Oncol* 1991; 12: 280-287.
2. Brackman HH, Eickhoff HJ, Oldenburg J et al.: Long-term therapy and on-demand treatment of children and adolescents with severe hemophilia A: 12 years of experience. *Haemost* 1992;22:251-8.
3. Nilsson IM, Berntorp E, Löfqvist T, Petterson H.: Twenty-five years experience of prophylactic treatment in severe haemophilia A and B. *J Intern Med* 1992; 232: 25-32.
4. Manco-Johnson M, et al. Prophylaxis versus episodic treatment to prevent joint disease in boys with severe hemophilia. *N Engl J Med* 2007; 357: 535-544.

5. Carlsson M, Berntrop E, Bjorkman S, Lindvall K: Pharmacokinetic dosing in prophylactic treatment of hemophilia A. *Eur J Haematol* 1993; 31: 247-252.
6. Aledort LM, Haschmeyer RH, Pettersson H. A longitudinal study of orthopaedic outcomes for severe factor-VIII-deficient haemophiliacs. The Orthopaedic Outcome Study Group. *J Intern Med* 1994; 236: 391-399.

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Appendix to MASAC Recommendation 179:

The National Hemophilia Foundation encourages persons with hemophilia and their families to consider these recommendations within the context of comprehensive care, which emphasizes patient and family involvement in the decision-making process, based on a thorough discussion of the risks and benefits – medical, social, psychological, and economic – of such care. Thus, in considering prophylaxis, the NHF encourages persons with hemophilia and their families to examine the following issues with their medical team:

- A. For parents of young children (age 1-2 years) with severe hemophilia who are being considered for prophylaxis, the following issues should be considered:
 - 1. Need for frequent clotting factor infusion versus joint damage and other morbidities associated with hemophilic bleeding.
 - 2. Potential quality of life and psychological implications.
 - 3. Potential costs and reimbursement implications.
 - 4. Requirement for frequent venous access, often necessitating use of a central venous access device (such as a surgically implanted port).
 - 5. Potential complications of such central venous access devices (see MASAC Recommendation #115 regarding central venous access devices).
 - 6. Possibility of other benefits and complications not yet identified.

- B. Recombinant factor products are the most appropriate choice for prophylaxis because of markedly reduced risk of blood-borne infections. (MASAC Document #169)

- C. If prophylaxis is being considered for an older child, adolescent, or adult, the following issues should be considered:
 - 1. Current joint damage and what can reasonably be expected from prophylaxis.
 - 2. Those considerations listed under A. above.

- D. If prophylaxis is selected as the therapeutic regimen of choice, the responsibilities and potential risks and benefits for the individual and the family need to be clearly delineated. The following points should be included in discussions held with patients and families:
 - 1. Frequency of infusion.
 - 2. Potential quality of life and psychological implications.
 - 3. Standard care for the central lines, if utilized, that the family must follow.
 - 4. Frequency of comprehensive follow-up.
 - 5. Statement of indications to alter or discontinue the protocol.
 - 6. Occurrences that prompt an immediate physician or nurse contact.
 - 7. Essential need for periodic follow-up education.
 - 8. A statement of continued risk of bleeding with trauma.
 - 9. An acknowledgement that there may be benefits and risks not yet identified.

- E. Once prophylaxis is begun, individuals may need to continue this therapy for life. Reasons to discontinue prophylaxis include
 - 1. Development of an inhibitor (lack of response to factor VIII or IX).
 - 2. Patient preference with physician concurrence.