



## Study Shows Benefit of Prophylactic Treatment

By Jeff Cornett, RN MSN, Director of Training, Research, & Advocacy  
*Horizons in Hemophilia*, Winter 2008

A study published in the August 9, 2007 issue of The New England Journal of Medicine has demonstrated one of the benefits of prophylactic treatment for severe hemophilia A. Prophylactic treatment consists of giving factor concentrate regularly (every other day) to prevent bleeding. It contrasts with episodic treatment, where factor is given only after bleeding has begun.

The research study was done by a group of physicians from hemophilia treatment centers across the country, including Dr. Tom Abshire from Emory University in Atlanta, and the Centers for Disease Control and Prevention. The researchers enrolled young boys (below the age of 33 months) with severe hemophilia A (factor VIII deficiency) in the study. The boys were randomly assigned to either a group that would receive prophylactic treatment or to one that would use episodic treatment. When the boys reached six years of age, they underwent MRIs and x-rays of their ankles, knees, and elbows to check for joint damage. It is well known that bleeding into a joint can damage it and, if left untreated, can lead to a crippling arthritis.

All of the ankle, knee, and elbow joints were normal in 93% of the boys who had received prophylactic treatment. For the boys who got episodic treatment, the percentage dropped to 55%. Prophylaxis resulted in an 83% reduction in the risk of joint damage. Two boys receiving prophylaxis developed inhibitors to factor VIII. Inhibitors are antibodies that destroy factor VIII, making it difficult to treat bleeds with factor concentrate. Three boys in the episodic group had bleeds that were considered life-threatening.

This research study presents strong evidence that prophylactic treatment can prevent joint damage in hemophilia. This type of evidence is important because prophylactic treatment is expensive. At age six, the boys in the prophylaxis group were using 6,000 IU of factor VIII per kilogram per year. The boys in the episodic treatment group were using less than half that amount, approximately 2,500 IU per kilogram. Assuming a price of \$1 per unit of recombinant factor VIII, it would cost \$300,000 a year to provide prophylactic treatment to a 110 pound boy. The study authors point out that this high price tag is one of the barriers to widespread use of prophylaxis.

The Centers for Disease Control and Prevention reported that only 51.5% of the children with severe hemophilia below the age of six were receiving prophylaxis in 2004.