

PROPHYLAXIS SUB-COMMITTEE REPORT: 2004

ASSOCIATION OF HEMOPHILIA CLINIC DIRECTORS OF CANADA

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The major activity of the Prophylaxis Subcommittee during 2003/2004 has been the Canadian hemophilia dose escalation prophylaxis study (CHPS 1) This study is funded by the CIHR Industry Partnership Fund (Industry partner Bayer). Co-Principal Investigators for the study are Dr. Victor Blanchette and Dr. Brian Feldman.

CHPS 1

The first patient entered the study in July 1997. At the completion of the initial study 25 boys were enrolled from 10 hemophilia treatment centres. Patients were followed for a total of 1150 patient months (mean 46 months /pt). The maximum length of enrollment is 60 months while the minimum is 16 months. Twenty-one boys have had at least 1 articular bleed (mean 4 per patient, range 0 - 12) over the course of the study. The average number of joint bleeds was 1.2 per patient/year. All 25 boys had a non-articular bleed.

An X-ray was taken of any joint that had a bleed either before entry or during the study. At exit X-rays were taken of all 6 index joints-ankles, elbows and knees. Physical therapy assessments were performed every 3 months. Joint scores were measured using a modified World Federation of Hemophilia (WFH) scoring system. All patient scores are in the normal range using this modified scale.

Ten of the 24 patients have port-a-catheters. By the end of the study all of the boys were on home therapy.

Blood samples are collected every three months for inhibitor levels and where possible 48 hr trough levels are measured. During the study one patient developed a low titre transient inhibitor. This was during his first year on study and in the last 3 years no inhibitor has been detected in this patient.

At the end of the initial study 12 boys (48%) remain on once weekly infusions (Step1). Nine boys have been escalated to Step 2 (twice a week infusions) (36%) and 4 have been escalated to Step 3 (alternate day infusions) (16%).

The results were presented at ISTH (2003) and ASH (2003). The investigators will submit 2 abstracts to the 2004 WFH meeting: an abstract on the port experience and a preliminary cost analysis.

CHPS 2

It was decided by the investigators to follow the boys for a further 5 years and to keep patient enrollment open. We have funding from Bayer for 3 years. There are currently a total of 36 boys enrolled(CHPS 1 and CHPS 2) in 10 centres. The goal is to enroll 40 boys.

Clinical Endpoints Survey

The purpose of this survey was to get consensus definitions of various terms in prophylaxis as well as classification of bleeds. The results from the first survey were compiled and a second survey was send out to all members of the AHCDC, CANHC and the Physiotherapy group. The results will be analyzed over the fall and winter.

International Prophylaxis Study Group (IPSG)

Members of the Prophylaxis Subcommittee, Dr's. Blanchette and Rivard and Pam Hilliard are actively involved in the IPSG. This group held its first annual meeting in Montreal in November 2003. The IPSG is actively involved in developing a single internationally approved MRI and physical therapy scoring system for use in prophylaxis studies.

Victor S. Blanchette
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