

PROPHYLAXIS SUB-COMMITTEE REPORT: 2006/2007

ASSOCIATION OF HEMOPHILIA CLINIC DIRECTORS OF CANADA

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The major activity of the Prophylaxis Subcommittee during 2006/2007 has been the Canadian hemophilia dose-escalation primary prophylaxis study (CHPS 1) and the extension study CHPS 2. 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 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 results of the study were published in Journal of Thrombosis and Hemostasis in June 2006.

CHPS 2

The investigators recommended continuation of follow up of boys enrolled on CHPS 1 and patient enrollment also be continued. The funding was obtained from Bayer for a further 3 years. There are currently a total of 55 boys now enrolled (CHPS 1 and CHPS 2) from 11 centres. The boys have been followed an average of 62 months (range: 5-117 months).

CHPS 3

In order to continue follow up of boys enrolled on CHPS 1 and CHPS 2 the investigators applied for, and received another cycle funding from Bayer, Canada (terms of current funding 2007-2009).

In CHPS 3 we will continue to record bleeding frequency; serial physiotherapy and imaging assessments will be performed.

Study aims are as follows:

- to determine risk factors for bleeding in boys with severe hemophilia A eg. thrombin generation when in a baseline state, presence of prothrombotic, genetic markers eg factor V leiden.
- to compare the cost and benefits of the Canadian dose-escalation (“tailored”) prophylaxis regime with intermediate dose prophylaxis (eg. as practiced in the Netherlands), aggressive on-demand therapy and full-dose prophylaxis (eg. the Malmö prophylaxis model as practiced in the USA randomized joint outcome study).

Prophylaxis Use Survey

The purpose of this survey was to determine current use of factor prophylaxis in all patients with hemophilia A or B in Canada. Data was collected on patients of all ages. The study has been completed and a report was submitted to the Bayer, who funded the initiative. Results of the survey will be presented at the 2007 AHCDC meeting in Quebec City.

International Prophylaxis Study Group (IPSG)

Members of the Prophylaxis Subcommittee, Dr. Blanchette Dr. Rivard and Pam Hilliard are actively involved in activities of the IPSG. This group continues to work in the area of Prophylaxis.

- On behalf of the IPSG the MRI Expert Working Group has published a single internationally approved MRI scoring scale.
- The PT Expert Working Group has received funding for a validation study of the Hemophilia Joint Health Score (HJHS) that was developed by the expert working group. Preliminary results will be presented at the upcoming WFH MSK meeting in Stresa, Italy.
- The Outcome Measures Expert Working Group is working to develop guidelines for the Quality of Life and Economic tools for use in prophylaxis clinical trials. Manuscripts are being preparation by both the HRQofL and Economic Analyses group.

Future Studies

- A study of once a day prophylaxis administration in adolescents and young adults has been submitted to Bayer for funding (deadline for grant submission 01/05/2007).
- An extended of factor prophylaxis in patients with inherited bleeding disorders and followed in AHCDC program has been submitted to Bayer for funding (deadline for grant submission 01/05/2007). This proposal builds on the recently completed survey of prophylaxis use.

Victor S. Blanchette
Chairman
Prophylaxis Sub Committee

Publications

Definitions for haemophilia prophylaxis and its outcomes: the Canadian consensus study.

S. Ota, M. McLimont, M. Carcao, Elizabeth Paradis, Nicole Graham, B.M. Feldman.
Haemophilia. 2007 Jan;13(1):12-20.

Tailored prophylaxis in severe hemophilia A: interim results from the first 5 years of the Canadian Hemophilia Primary Prophylaxis Study. Feldman BM, Pai M, Rivard GE, Israels S, Poon M-C, Demers C, Robinson S, Luke K-H, Wu JKM, Gill K, Lillicrap D, Babyn P, McLimont M, Blanchette VS, on behalf of the Association of Hemophilia Clinic Directors of Canada Prophylaxis Study Group. J Thromb Haemost 2006; 4:1228–36.