

PROPHYLAXIS SUB-COMMITTEE REPORT: 2003

ASSOCIATION OF HEMOPHILIA CLINIC DIRECTORS OF CANADA

DR. V. BLANCHETTE (CHAIRMAN)
DR. GEORGES RIVARD
DR. SUSAN ROBINSON
DR. SARA ISRAELS
DR. KAISER ALI
DR. MANUEL CARCAO
PAM HILLIARD
KATHY MULDER

The major activity of the Prophylaxis Subcommittee during 2002/2003 has been the Canadian Hemophilia dose escalation prophylaxis study. 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.

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. There were 11 cases that were ineligible for study (positive inhibitor at entry - 7 cases). The sites have received ethics approval for the boys to stay on study.

Patients have been followed on study 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. All 25 boys have had a non-articular bleed.

Ten of the 24 patients have port-a-catheters. Twenty-two of the boys receive their infusions at home while 3 of the boys receive them in a hospital or clinic setting.

An X-ray was taken of any joint that had a bleed either before entry or during the study. At exit Xrays were taken of all 6 index joints-ankles, elbows and knees. Physical therapy assessments were done 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.

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.

At the end of the initial study 12 boys (48%) have had their dose escalated. Of these escalations, 6 were due to the development of a target joint (3 bleeds into 1 joint in a 3-month consecutive period) and the remainder were due to ≥ 4 significant bleeds in a 3 month consecutive period. Ten have been escalated to Step 2 (twice a week) and 2 have been escalated to Step 3 (alternate day therapy).

The results will be presented at the ISTH meeting in July.

The multi-centre/multi-national Factor IX study will not proceed at this time due to lack of sufficient subjects and funding issues.

There are initiatives in the planning phase to look at a multicentre secondary prophylaxis study. This is a modification of a study that Dr. Carcao is conducting at The Hospital for Sick Children.

Last year, the subcommittee supported an initiative to conduct a survey of members of the AHCDC as well as Canadian Hemophilia Nurses and Physiotherapists. The purpose of this survey was to get consensus definitions of various terms in prophylaxis as well as types of bleeds. The first survey was conducted last summer. The results are being analyzed and a second survey will be sent out this summer.

Victor S. Blanchette
Chairman
Prophylaxis Sub Committee

Appendix A

Core Study Committee: Hospital for Sick Children

Dr. Victor Blanchette
Chief, Haematology/Oncology

Dr. Brian Feldman
Rheumatology

Dr. Paul Babyn
Study Radiologist

Dr. Doug Hedden
Orthopaedic Surgery

Ann Marie Stain
Hemophilia Program Nurse Co-ordinator

Pam Hilliard
Hemophilia Program, Physiotherapist

Marjorie McLimont
Study Co-ordinator

Data Safety Monitoring Committee

Dr. Jerry Teitel
Dr. Marilyn Manco-Johnson
Dr. Louis Aledort

Regional Steering Committee

Dr. Sara Israels
Division of Pediatric Hematology
Manitoba Cancer Treatment & Research
Centre
Foundation

Dr. Georges Rivard
Directeur, Clinique d'hématologie
Hôpital Ste-Justine

Dr. Sue Robinson
Director, Hemophilia Clinic
Q.E.II Health Sciences

Local Site Investigators

Dr. Dorothy Barnard
Division of Hematology/Oncology
IWK-Grace Health Centre

Dr. Robert Klaassen
Director, Hematology/Oncology
Children's Hospital of Eastern Ontario

Dr. Sean Dolan
Director, Hemophilia Program
Saint John Regional Hospital

Dr. Mohan Pai
Director, Hamilton/Niagara Regional Hemophilia
Centre
Hamilton Health Sciences Corporation

Dr. Kulwant Gill
Director, Hemophilia Program
Laurentian Hospital

Dr. Man-Chiu Poon
Hematology Department
Foothills Hospital

Dr. Christine Demers
Directeur, Centre di l'hémophilie
Hopital du Saint-Sacrement

Dr. Sheldon Rubin
Director, Hemophilia Program
The Moncton Hospital

Dr. Marie-France Scully
Hemophilia Program
Janeway Child Health Centre

Dr. John Wu
Division of Hematology/Oncology
B.C. Children's Hospital

Dr. John Akabutu
Department of Pediatrics
Walter Mackenzie Health Sciences

Dr. David Lilliecap
Department of Pathology
Queen's University

Dr. Kaiser Ali
Division of Pediatrics
Saskatoon Cancer Centre

Nurse Coordinators

Wilma McClure
University of Alberta Hospital

Lois Linder
Mary Pack Arthritis Centre

Carol Mayes
Saint John Regional Hospital

Heather Richards
Janeway Child Health Centre

Ann Marie Stain
Hospital for Sick Children

Elizabeth Paradis
Laurentian Hospital

Julia Sek
McMaster Division

Claudine Amesse
Hopital Ste-Justine,

Sylvie Lacroix
Montreal Children's Hospital

Pat Klein
Alberta Children's Hospital

Nora Schwetz
Health Sciences Centre

Dorine Belliveau
The Moncton Hospital

Sue Ann Hawes
IWK Grace Health Centre

Sherry Purcell
Kingston General Hospital

Diane Bissonnette
Children's Hospital of Eastern Ontario

Carol Edwards
Port Arthur Site

Ginette Lupien
Hopital du Saint-Sacrement

Physiotherapists

Betty Hale
University of Alberta Hospital

Amanda Ng
The Mary Patrick Arthritis Centre

Kristine Sweet
Saint John Regional Hospital

Andrea Hann
Janeway Children's Health Centre

Pam Hilliard
Hospital for Sick Children

Sally Spence
Laurentian Hospital

Heather Zucker
McMaster Campus

Nick Zourikian
Hopital Ste. Justine

Lorraine Fischer
Montreal Children's Hospital

Jennifer Aikenhead
Alberta Children's Hospital

Kathy Mulder
Child Health Sciences Centre

Karla West
South East Health Care Corp.

Bethany Lezama
IWK- Grace Health Centre

Elsie Culham
Queen's University

Brenda Elliot
Ottawa General Hospital

Donna Langen
Thunder Bay

Gaetan Thibaultt
Hopital Ste. Sacrement

Joanne Nilsen
Royal University Hospital

Appendix B

RECRUITMENT SUMMARY

Centre	# Identified	Recruited Patients	Potential Patients	Identified Ineligible	
				Inhibitors	Other
Hamilton	6	5	0	0	1
Toronto	8	4	2	1	1
Montreal	7	4	1	2	0
Calgary	4	3	1	0	0
Winnipeg	5	3	0	2	0
Quebec City	4	2	2	0	0
Edmonton	1	1	0	0	0
Halifax	2	1	0	0	1
Thunder Bay	1	1	0	0	0
Ottawa	1	1	0	0	0
Saint John	3	0	2	1	0
Sudbury	1	0	0	1	0
Vancouver	2	0	1	0	1
TOTALS	45	25	9	7	4