



Neutropenia Support Assoc. Inc.

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Local Winnipeg # 489-8454 Toll Free # 1-800-6-NEUTRO (663-8876)

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SCN GOAL ACHIEVED ! NEUPOGEN APPROVED FOR SCN PATIENTS

October 28th, 1993. The Health Protection Branch (Canada) approves NEUPOGEN (Filgrastim) for long term use in the treatment of patients with severe chronic neutropenia. Neupogen is now indicated for long term use to increase neutrophil counts and reduce the incidence and duration of infection in patients with a diagnosis of congenital, cyclic, or idiopathic neutropenia.

Dr. Bonnie Cham paediatric Haematologist, Children's Hospital H.S.C Winnipeg comments:

"As physicians our goal is to practice preventative medicine. This is a very exciting drug. We can provide medication to avoid frequent serious infections, rather than needing to treat severe chronic neutropenia patients with established repeated infections. The impact on the patient's quality of life will be dramatic."

Lorna Stevens, president, Neutropenia Support Association Winnipeg adds: "This is thrilling news, with this approval SCN patients will finally be able to access cost effective and life saving treatment more easily. Hopefully this will streamline the health care cost coverage on a national level."

Dosage and administration schedules are available through your physician. The medical community and AMGEN Canada can supply you with the official product monograph.

Should you require additional information please contact the Neutropenia Support Association Inc. (Reg. Canadian Charity) 1-800-6-NEUTRO or in Winnipeg call 489-8454

Neupogen Approved for SCN Patients in Australia

A simple bout of chicken pox could have had eight-year old Christopher Kenny knocking on death's door last year. When the disease hit this year a new drug had him back at school within a couple of weeks.

Christopher, who lives in Melbourne, is one of up to 50 people in Australia who have severe chronic neutropenia SCN, which leaves them highly vulnerable to infection.

Chicken pox poses a particular threat to these people because SCN causes persistent sores which promote the spread of chicken pox and increase the risk of blood poisoning.

People with SCN cannot produce enough white blood cells, called neutrophils, to fight infection and, before antibiotics were developed, they usually died in their first or second year of life.

Even with antibiotics, they still suffer life-threatening bacterial infections and painful ulcers of their mouth, throat and anal regions.

They may spend months or even years in hospital on intravenous antibiotics.

Christopher and his two-and-a-half-year-old sister Hannah, who also has SCN, now have daily injections of Neupogen, which stimulates the production of white blood cells.

The treatment's success was demonstrated when Christopher and Hannah recovered from chicken pox about as quickly as their sister Rachael, six, and a brother Andrew aged four months, who are not SCN affected.

"If they had the chicken pox without Neupogen they would have been very, very sick indeed," their mother Elizabeth Kenny said in an interview.

"They would have been at death's door if we hadn't had Neupogen. We were very grateful for it, it's wonderful stuff," she said.

"The risk of having a serious life-threatening infection would have been much higher without the Neupogen," said Dr George Kannourakis, a children's blood cancer specialist at the Royal Children's Hospital, Melbourne.

"For this condition, its doing a marvelous job," said Dr. Kannourakis.

Hannah and Christopher have another medical problem which

prevents their bodies from storing food energy, forcing them to eat frequently and have feeding tubes inserted at night to keep their blood-sugar levels up and prevent them from falling into comas. Mrs. Kennedy learned to cope with inserting the tubes into her children's throats but said she could not face the daily injections of Neupogen so husband Michael does those.

But the injections are worth it. Christopher had an ulcer the size of a ten-cent piece between his buttocks for more than 18 months before the Kennys found out about Neupogen.

It cleared within six weeks, relieving the agony the ulcer produced every time he had to defecate.

European trials of the drug, which Australia has just approved for treating SCN, showed it had a 90 per cent success rate, a German expert said during a recent visit to Sydney.

"Trials involving 51 people with SCN had been extraordinarily successful," paediatrician Prof. Karl Welte of the medical school of Hannover in Germany said in an interview.

"About one in a million people are born with SCN although the genetic defect occurs more frequently in cultures where cousins marry," Prof Welte said.

Neupogen, already approved in Australia for boosting white blood cell production in people having chemotherapy for cancer, has been available here within drug trials for SCN since early last year. Between 30 and 50 people in Australia have SCN, 17 of whom have taken part in Neupogen trials involving about 500 people world wide.

In Prof. Welte's trials in Hannover, just two children out of 51 failed to respond.

One was treated with a bone marrow transplant and the other one died.

Prof. Welte's patients have been taking Neupogen for up to five and a half years, suffering few side effects, although some have had bone pain.

"The drug reduced sufferer's need for antibiotics, cut stays in hospital and dramatically improved their quality of life," Prof. Welte said.

Prof. Welte was in Australia to talk to specialists around the country about the trials.

Apart from trials, the drug had only been available in Finland until July 12 when the Australia Drug Evaluation Committee approved its use for SCN here.

Submissions to approve the drug's use were under review in the United States, Canada, and Europe. According to makers pharmaceutical company Amgen.

For Erin, it's a shot a day

A simple daily injection is helping to keep two year old Erin Murnane alive by making her body do what just about everyone else's does naturally - fight infection.

Erin, who lives with her parents Margaret and Stephen and baby brother Jayson in the Newcastle suburb of Edgeworth, was born with Kostmann's Syndrome, a rare condition which means her bone marrow cannot produce enough white blood cells (called neutrophils) to fight off infection.

Her condition was diagnosed at eight weeks of age and It was

thought that without treatment she would not live beyond a year. In the past, the treatment in severe cases has to use intravenous antibiotics for every infection. Now patients are offered a bone-marrow transplant, if a compatible donor is found, or the human growth factor filgrastim, known commercially as Neupogen. Erin does not have a compatible donor, but the Prince of Wales Children's Hospital is involved in a trial of Filgrastim and she has been on the drug for more than two years.

The Australian Drug Evaluation Committee has just approved Filgrastim for the treatment of Kostmann's Syndrome, but government approval does not mean the end of the trial.

Mr Russell Edwards, the managing director of Amgen which manufactures the drug, said it would continue to be provided free of charge to the 17 Australians in the trial and to any new children who fit the guidelines for treatment.

Mrs Margaret Murnane is still uncomfortable about injecting her daughter every night, but thanks to the treatment Erin has recently celebrated her first year free of hospitalization

"The medicine keeps her safe," Mrs. Murnane said.

Erin's doctor, Professor Marcus Vowels, the director of children's cancer services at the Prince of Wales Hospital, said children with a severe form of Kostmann's Syndrome would in the past have died within the first year of life after recurrent infections, loss of weight, anaemia and a general failure to thrive.

He is treating three young patients and all have experienced an improvement in their quality of life.

Professor Vowels said that if he had a child with the condition who had a compatible bone marrow donor, he would seriously consider a transplant. Although Filgrastim worked, the long-term effects were not known and it was "a bit of a hassle" having an injection every day.

Neupogen has already been approved by the Federal Government for use in reducing the incidence of infection following chemotherapy, in some cancer patients and for some bone-marrow transplant patients.

2nd Annual Fashion Show Booming Success

We'd like to take this opportunity to extend our heartfelt thanks for your generous support of our fashion show which was held at the end of April.

We are already in the planning stages for next year's fund-raiser, possibly in the fall of 1994 and we hope we can count on your support again next year!

Special Thanks To: All our volunteers, All contributors
AND

The Calendar Firefighters, Panache Models, Danali's Men's wear, Peepers "Fun in the SUN Fashions", Just Maggie's, Addition-elle, Teri's Furs, Tom Q Milroy "The Morning Man" Q 94 FM, Dr. Nathan Kobrinsky and Radisson Suite Hotel.

The Volunteer Extraordinaire Certificate was presented to Mrs. Audrey Carlson. Medical Advisor Extraordinaire awards were presented to: Dr. Bonnie Cham; Dr. Jon Gerrard; and Dr. Nathan Kobrinsky. Many "Builder Awards" acknowledged our corporate and organizational contributions.

Thank you for choosing us as your Special Charity!

- * Eastern Star Chapters
 1. Jubilee Chapter #27
 2. Concord Chapter #39
- * Lions Foundation of Manitoba and N.W. Ontario
- * Nancy Knight and DELBRO Real Estate Ltd.
- * Executive Limousine Service
- * Apple Computers for Kids
- * Minnedosa District Health Auxiliary
- * #150 Army Navy & Airforce
- * PT Leamington Fireman's Dept. and Ladies Auxiliary
- * PT Leamington Dart League
- * Golfer: Todd Neale, River Oaks Golf Club
- * Ciociara Pizza Restaurant Ltd.
- * The Vitalizers
- * Toronto Dominion Bank Westrow Commercial Banking Centre, Diane Olivier
- * And all "In Memory of" donations. The family received acknowledgment of your generous and caring donation.

All of these wonderful efforts add up to helping us pay for our toll free telephone **1-800-6-NEUTRO** and to continue supporting our mandate as stated in this newsletter. We are 100% volunteers.



*Mike Carlson presenting award to his Mom .. Audry Carlson. She does most of the manual typing. Thanks Mom !
OK We've been caught .. The people behind the news letters.*

We have donated \$4,936.40 on December 30th, 1992 to the University of Manitoba Research Lab. We also donated \$250.00 to the Calendar Firefighters Burn Fund. **Thanks** for your participation in the fashion show.

\$500.00 went to the Department of Paediatric Haematology/Oncology at the Manitoba Cancer Foundation and Children's Hospital for the purchase of 15 handbooks titled:

"Supportive Care of Children with Cancer"

We express our gratitude to those who have made very kind donations. These gifts enhance the quality of the lives of those yet to be helped through research and education.

Current Wish List:

a) To develop a parental guidance workbook for children "to take the fear away" and help with the treatment.

b) To promote art by children; Touched by serious illness and printed on "Caring Cards" for sale as our special on-going fund-raiser. Proceeds in aid of research, development, funding a child life worker, etc.

This could be a common fund-raiser for all neutropenia chapters. We also encourage the partnership with other volunteer support groups.

c) To help facilitate other support groups in the U.S. and Canada as funds and the opportunity permits.

All Thanks to You !

Comments from Dr. Nathan Kobrinsky

It was a pleasure participating in the Neutropenia Support Association fund-raiser. The organization has certainly grown since its inception. I feel that you will play a pivotal role in optimizing the health care for neutropenic patients in Canada in the coming 10 to 20 years.

In view of the strength of the support group, I must say that the notion of establishing a cross-Canada "White Cross" to provide G-CSF and other growth factors is definitely a possibility.

I envision the organization as follows: Private sector funds, perhaps reached with government funds, would be used to produce G-CSF in large quantities. Facilities to produce G-CSF certainly exist in Winnipeg. Recently the Rh Institute on the University of Manitoba campus has been involved in developing a number of genetically engineered molecules including factor VM and superoxide dismutase. Tentatively, I would recommend that the Rh Institute be approached with a view to becoming a production site for G-CSF. Once produced, the material could then be available for patients with chronic neutropenia and neutropenia secondary to chemotherapy. The Red Cross model of providing free blood products for Canadians would be ideal and a similar structure would seem appropriate for "the White Cross". Perhaps advisors from Red Cross National would be willing to participate in the development of your program.

If you can successfully have G-CSF developed in Manitoba by the Rh Institute and funded both privately and by government support this model could then be applied to the development of other growth factors such as erythropoietin, GM-CSF, IL-3, interleukin-2 (IL-2) and others.

Without dreams we can never reach the stars.

We sincerely appreciate receiving the many wonderful suggestions. Keep your ideas coming.

Value of Good Public Relations

Excerpt from Winnipeg Free Press

A day doesn't pass without me having to deal with people who know the value of good public relations and those who haven't an inkling. For example, Bill Krawetz, recently retired media communications officer for the Manitoba Telephone System, was one of the few public relations officers who would readily admit, in quite colorful language, if his company had goofed. His successor, June Kirby, is from the same school. "We certainly did screw up," was her blunt response to a runaround MTS had given a local group. A clerk carelessly juxtaposed 'e' and 'u' in the

Neutropenia Support Association's 1993 telephone listing in the white and yellow pages locally, and the group's toll-free and phone book listings in 14 major Canadian cities.

Lorna Stevens, who founded the association with her husband, Lorne, was understandably peeved. This year, the association had planned on expanding its activities across Canada. She said when she called MTS to complain, she was told nothing could be done. Neutropenia is a very rare blood disorder which leaves its victims prone to suffer infections easily and often. Some people are born with it, like the Stevens' nine-year-old. Others develop the disorder after a viral infection and some acquire it after being treated for cancer with chemotherapy drugs. The Winnipeg-based registered charitable organization is the only group in the world devoted to providing support and information to patients and their families.

Kirby said the association will be reimbursed for the cost of the listings and that information services, both locally and in the other Canadian cities, have been alerted to the correct spelling.

... From Mike Ward Wpg Free Press

Along the Support Group Way we have learned:

1. To value relationships.
2. To straighten our priorities.
3. To become less materialistic.
4. To postpone less.
5. The lesson of KINDNESS.
6. To say "I Love You" more often
7. The lesson of strength and courage.
8. To take a lot less for granted.
9. To be more aware of gifts and blessings.
10. The importance of risk.
11. The value of vulnerability.

... From Jeffery's Folks

Canadian Neutropenia Registry Ready to go

We have \$4,000.00 on hand allocated for Dr. Bonnie Cham's SCN Registry

The Canadian Neutropenia Registry is now established and ready to enter patient data. As you may recall, this is a registry being coordinated by myself and Dr. Jon Gerrard at the Manitoba Cancer Treatment and Research Foundation. The goal of the registry is to establish figures for the incidence of severe neutropenia, the morbidity and complications of this disorder, and also to follow the short term and long term effects of treatment with G-CSF or any other form of therapy. In addition, by establishing a central data base regarding these patients, we hope to provide a network to facilitate physician communication. Finally, an International Registry for patients with neutropenia may also be established in the near future and we would be able to contribute to this registry. This project has been approved by the Committee

on the Use of Human Subjects in Research at the University of Manitoba and I have enclosed copies of the consent forms which we will be using locally for your information. Eligibility for the Registry includes severe neutropenia (ANC of < .500) for at least 3 months duration in the absence of any other associated cytopenias. We are interested not only in following patients who are receiving G-CSF but also those who are not. Patients with associated immunodeficiency syndromes or congenital anomalies are not excluded from registration. There is no age limit on registration of patients. Once informed consent has been obtained, the forms can be completed and returned to me at the address on the letter-head. Follow-up questionnaire will be sent on an annual basis and any physician wishing to add a question to the questionnaire is encouraged to contact me.

The registry will collect patient demographics including name and address in order to facilitate long-term tracking of these patients, but each patient will be assigned a unique identifier and the demographics will be kept separately from the main data base.

I am hoping for widespread cooperation with this project. Unfortunately, we have no budget available for reimbursement to centers who complete these forms. The forms themselves are fairly short and I would anticipate should take no more than 10 -15 minutes once a year to complete. With this being a relatively rare disorder I would hope that no one center would be unduly burdened by this. In addition to contacting the Pediatric Hematology departments in Canada, we will be also notifying members of the Neutropenia Support Association, who will then be able to speak to their physician if they are interested in participating. No information will be entered onto the registry however that has not been received from a physician.

Enclosed please find copies of the registration form as well as the consent forms which we will be using locally for both adults and children. I have also enclosed a summary sheet of the project which may be helpful to you in your submission to your local ethics committee. If you require further specifics please feel free to contact me.

Finally, I have enclosed a form which I would appreciate your filling out and returning to me regarding your centre's ability to participate in this project. I hope to hear from all centres in the next month. Thank you for your cooperation.

Manitoba Cancer Foundation/Children's Hospital (HSC)
University of Manitoba
Informed Consent

Title: Canadian Neutropenia Registry

Your child has been diagnosed with neutropenia. This is a disorder of the neutrophils, infection fighting white blood cells, resulting in low numbers of these blood cells. As a result, your child's ability to fight bacterial infection may be decreased. This problem has been diagnosed by your doctor with the help of blood tests, and your child is, being treated as deemed appropriate by your doctor and yourself. Neutropenia is a rare disorder. We would estimate that there are less than 100 patients with neutropenia in Canada. the Division of Pediatric Hematology/Oncology of the Department

of Pediatrics, University of Manitoba, with financial help from AMGEN Canada, is establishing a registry of patients with neutropenia across the country. The goal of this registry is to determine exactly how many people in Canada have neutropenia, what treatment is being used, and information about their quality of life. This will enable your doctor, along with other doctors in Canada, to have more information about this rare disorder. The registry will not have any role in the treatment of your child, other than to make available to your doctor information about other patients with this disease.

The registry will consist of information about the specific type of neutropenia you have, the treatment which has been used, and any complications that have occurred. This information will be collected in the form of a questionnaire which your doctor will fill out. In addition, we plan to periodically sent out questionnaires to update our files as to further treatment and/or complications. The information will be stored on a computer system and doctors across Canada will have access to the information. In addition, should an international neutropenia registry be established, the information collected would be passed on to them. The identity of your child will be kept entirely confidential. Participating in the registry will not require your child to undergo any additional tests and will not involve any treatment other than those which your doctor would normally advise. Participation in this registry is entirely voluntary and you may withdraw at any time.

Informed Consent

I have read the above explanation of the neutropenia registry and have had an opportunity to discuss it in detail with my child's physician. I have had an opportunity to have my questions about my child's illness and the purpose of the registry answered to my satisfaction.

I understand participation is voluntary and refusal or withdrawal from the registry will involve no penalty or loss of benefits to which my child is entitled. I understand that information in the registry will be made available to other physicians and researchers interested in neutropenia but that a policy of strict confidentiality will be followed with respect to my child's identity.

Title : Canadian Neutropenia Registry

If any publications should arise, my child's identity will not be disclosed and confidentiality will be maintained. If I have any questions about this study or my child's rights as a participant, I may contact my child's physician who is

_____ at _____ or Dr.
Bonnie Cham (telephone 787-2198).

My signature below signifies that I have read this document and feel that I am acting in the best interest of my child who is a minor. I have agreed to have my physician enter my child onto the neutropenia registry. I understand that the participation of my child is entirely voluntary and that I am free to withdraw my child from this study at any time without prejudice. The doctors will continue to give the best possible care to my child. I will also be given a copy of this consent form for my information.

Patent

Date

Parent/Guardian

Parent/Guardian

Witness

I certify that I have reviewed the contents of this form with the persons signing above, who in my opinion understand my explanation of the purpose and operation of the registry. Any significant change in the nature of the registry from that described above will be fully explained and permission obtained again.

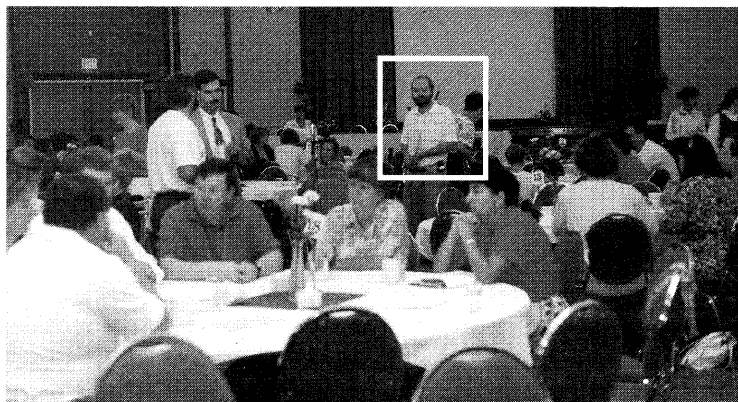
Physician's Signature

Please contact your Physician. This is a recent announcement, so should you require the paperwork, extra packages are available by calling 1-800-6-NEUTRO or in Winnipeg call 489-8454 The Neutropenia Support Assoc. Inc.

Adult Informed Consent forms are also available.

Neutropenia Support Assoc. Inc. Goes to Kingston

The Neutropenia Support Assoc. Inc. was part of the National Children's Cancer Conference



Wayne Greenway, Hotel Dieu Hospital, Child Life.. National Children's Cancer Conference July 30th, 1993

We are indebted to Wayne Greenway, Hotel Dieu Hospital Child Life for his assistance enabling this major event to take place so successfully.

We covered our own expenses at the conference but wish we could have contributed far more as we gained so much. A purpose, a goal, a special project, a focus helps us all to heal and deal with the illness. As our fund raising efforts develop, we shall remember to return the favor for others in the future.

There were many unexpected highlights.

The Hotel Dieu Hospital Tour - Incredible!

Camp Trillium - Moved Us to Tears!

Child Can - Innovative ideas so exciting!

Ben Wolfe - Wow! Wonderful!

Sessions of interest included:

"When there is no cure"

"Support for Grieving Parents"

"Listening to Your Children"

"Pain Management for Parents"

Parent - Health Care Professional:

"How to Make the Interaction Work"

"Evolution of Parent Support Groups"

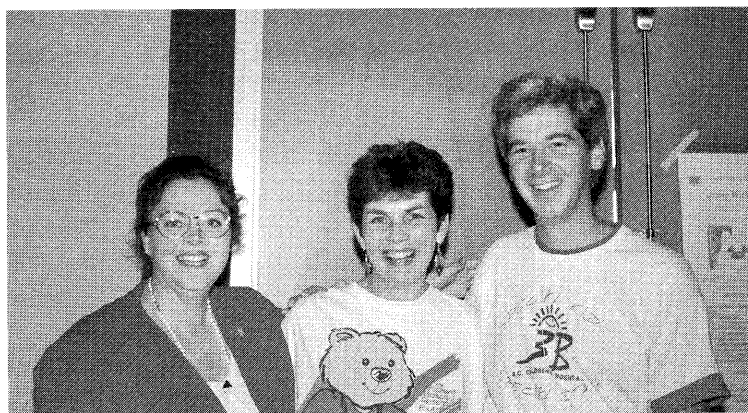
"A Student's Art Gallery in a Hospital" a healing partnership

"The Experience of Being a Parent of a Hospitalized Child"

Parent Workshop

Some comments received from N.S.A.I. families:

Tears in eyes, smiles on faces; Pins and needles at first; no surprises, then reassured; took the fear away; put into perspective; not as afraid; very hopeful; gave them a world of information; answered lots of questions; peace of mind; put mind at ease; the children attending were also well involved and cared for - Thank You!; opened our eyes to innovative fund raising ideas and parental guidance development.



Lorna Stevens , Dawn Kidder MCTRF Child Life, Dr. Kent Stobart

First Neutropenia Info Book Completed

We sincerely appreciate the educational grant from Amgen Canada Inc. enabling the 1st booklet on neutropenia to be developed in English and French. (We are working on a Spanish translation) Neutropenia - Causes, Consequences & Care, Information for Patients and their Families

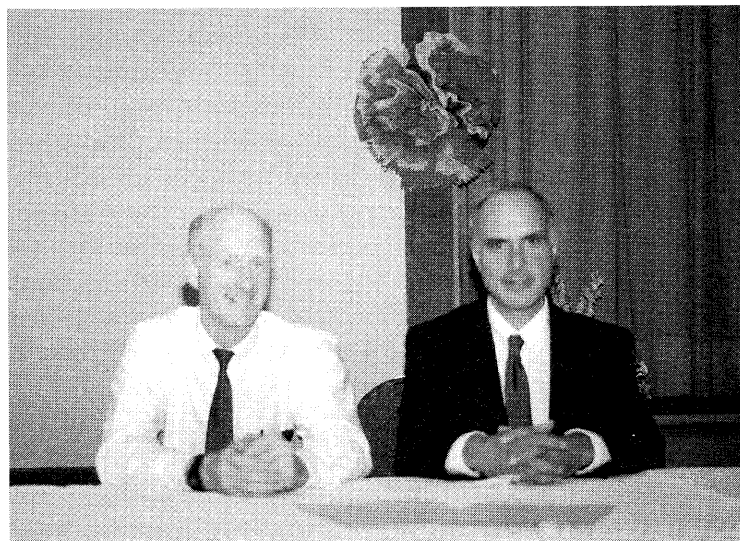
We distributed 800 booklets at the National Children's Cancer Conference 1 in 1000 "Living with Childhood Cancer Can Be Living" held in Kinston Ontario, July 31, 1993.

At this conference we participated in a presentation on "Growth Factors in the Treatment of ChemoTherapy Induced Neutropenia"

Speakers: Dr. David Dale, Dr. Melvin Freedman & Lorna Stevens.

The presentation was well received by child life workers, nurses, physicians and families. Specifically, 18 neutropenia families attended from Manitoba, Ontario, Quebec and Newfoundland, and were also joined by many families touched by cancer, seeking information

Dr. David Dale and **Dr. Melvin Freedman's** presentations at this conference were packed full of slides and interesting information. Their presentations were audio taped and is available by contacting Wayne Greenway, Child Life Program, Hotel Dieu Hospital, Kingston, Ontario, Canada (613) 544-3310, ask for Saturday Session 3:00-4:45, "Growth Factors in the Treatment of ChemoTherapy Induced Neutropenia".



*Dr. David Dale and Dr. Melvin Freedman
National Children's Cancer Conference*

The Ontario Support Group Ready & Rolling !



The Rogers, Corrie & Glen

Corrie and Glen Rogers held the first Ontario Neutropenia Support Assoc. meeting Sept. 19th 1993. This first meeting was just an introductory meeting to get every body together and start the ball rolling. Most of the eastern families were in attendance at the conference in Kingston.

For more information on the Ontario Support Group, Please call them at 416-267-4270. All neutropenia families welcome.

ONTARIO - ELECTRIC CAR!

The Ontario Support Group will be directing their fund-raising efforts to support research, by Dr. M. Freedman at The Hospital for Sick Children. They have many ideas under discussion. They have an electric car to raffle!

CONTACT: Shirley Cox 1-705-424-1285

From Newfoundland - Maritimes

Dianne Cooper of Newfoundland is approximately half-way toward raising the necessary funds to purchase an entertainment centre for use by children in isolation in a local hospital. A very worthwhile project! Well done Dianne!

Dianne can be reached at 1-709-484-3592 and invites all Maritime families to give her a call.



Dianne Cooper

Neutropenia Support Assoc. Inc. Gets down to business with the Ontario Government

Toronto, September 28, 1993.

Excerpts of presentation by Dr. Melvin Freedman, Shirley Cox, Lorna Stevens to: The Ministry of Health Ontario and representatives from the following departments: Scientific Affairs, Special Drugs, Drug Reform Secretariat, Drug Programs Branch. Presentation by Lorna Stevens.

What is the Neutropenia Support Association?

The Neutropenia Support Association was incorporated July 31, 1989 and we became a registered charity on January 31 st, 1990.

All of us are volunteers.

Our main objective in the beginning was to promote the awareness of a disease called severe chronic neutropenia and to accelerate in any way possible the approval process of a drug called G-CSF since some of our members were participating as patients in a large multi-centre trial with G-CSF. The interest that developed from the results of this trial was incredible. (To say the least).

Once the drug was approved in Canada (February 1992), we continued to focus the awareness around neutropenia and severe chronic neutropenia but shifted our attention in this field to helping patients achieve access and financial coverage for the treatment costs.

Our primary mandate is to help and support patients and their families who are suffering from the consequences of neutropenia or severe chronic neutropenia.

Our activities include supporting ongoing neutrophil research. We provide patient support through a Canada wide, toll-free hotline. We are engaged in the development of an international central registry for neutropenia. We provide a periodic newsletter for patients and treating physicians, and distribute our 1993 booklet; "Neutropenia: Causes, Consequences and Care." We also further educate in the field of neutropenia by way of our Health Sciences Centre/Children's Hospital Library established in 1991.

Neutropenia, Severe Chronic Neutropenia & Neupogen:

The general knowledge of neutropenia even among the medical community and nursing staff, because of its rarity, is not as well understood as it could be.

Generally the patients we are involved with in our Neutropenia Support Association can be classified in one of the two following groups:

1. Neutropenia as a consequence of chemo-therapy, and, although this meeting will not deal with these patients, access to Neupogen for these patients is still problematic.

2. The second group is a group of patients who have chronic neutropenia. It could be idiopathic, cyclic or a congenital form of neutropenia.

These patients will have infection after infection due to the fact that they lack an appropriate number of neutrophils which are the primary defense system against bacterial infections.

This is the group that we want to talk about in this meeting today. Patients with severe chronic neutropenia are predominantly children, since without Neupogen treatment, most of them will not reach adulthood because the smallest infection can turn quickly into a life threatening infection.

This brings us to the product Neupogen. It has been reported that it can reduce by 50% the incidence of potentially life-threatening infections in cancer patients treated with chemo-therapy.

This produces a significant cost savings to our health care system by reducing or eliminating the associated costs of hospitalization and infection control.

We also know that Neupogen had won the United Kingdom 1991 Most Outstanding Pharmaceutical Product of the Year Award, the internationally renowned Prix Galien Award.

Dr. Freedman will talk in more detail about what Neupogen can do for patients with severe chronic neutropenia, but I would like

to give you some quotes of some of the patients we are familiar with:

Quotes:

“My daughter also suffers from neutropenia, but through the grace of God became one of the lucky candidates who participated in the research program”.

“I’m sure that there are many families who were not so lucky”.

Excerpts from another letter received:

“Our baby was the joy of our lives ... our joy turned into a nightmare”...

“She became critically ill and during a six week hospital stay she had one setback after another, including rectal abscesses, kidney failure, high blood pressure, seizures and gangrene of the bowel requiring removal of a portion of her small bowel”...

“She required intravenous medications and fluids for many weeks. I can’t stress enough the suffering the parents and family go through, how helpless you feel, watching your child suffering this pain and unable to stop it”.

“Miraculously, with huge doses of antibiotics and cortisone, she made it”.

“The next five years she spent more time in hospitals than at home”...

“I believe she suffered more pain and illness during that period than most people suffer in a lifetime”...

“Her gums were bleeding, sore and swollen most of the time, making it difficult for her to eat”...

“By the age of 10 years old she had severe damage to the roots of her teeth ... and probably the dentist thought, by the time she was a teenager she would lose her teeth”...

“She was angry that she had scars all over her body from the infections”...

“She also had to endure all the testing, probing, needle sticks, etc. which, in itself, is very traumatic”...

“We must not ignore the pain and suffering a child endures with Neutropenia, but we must not ignore the emotional pain and suffering the child and family go through also”...

“Very few people seemed to truly understand the difficulties and hardships caused from this horrible disease”...

“I had prayed for a treatment or cure for this disease, and finally, my prayers had been answered in 1989...”

“She began treatment of daily G-CSF injections under the supervision of a study program”...

“Within one month, we were beginning to see the remarkable effects of this miracle drug”...

“...Her energy level increased”...

“Her gums began to improve”...

“Her loose teeth tightened up”...

“She can now live a full life without frequent pain, suffering and illness”...

“and grateful to be alive”...

“We’re unable to put into words the happiness and relief this has brought to her and our family”...

“She is now a beautiful, caring, sensitive young lady with severe chronic neutropenia, taking a daily dose of G-CSF”...

“Thanks again to all those involved in Neutropenia Research and Support Programs, etc.”

The Neutropenia Support Association Inc. has so many similar letters on file which motivates us to pull together and gain support for people suffering from neutropenia.

We do understand that the treatment with colony-stimulating factors is expensive and that the system has to advocate the appropriate use of these drugs.

That is why we continue to follow the cost savings data and apply this information to support our SCN families’ treatment needs.

Other Quotes:

“My son, by the age of three, had been hospitalized 39 times. The treatment has been lifesaving”.

One of our SCN children, for example, has well over 175 hospitalized days on his file over a period of a few years. The direct comparison of the hospital costs of 175 days provides him with a minimum of 4 - 6 years of treatment. (Just to put “costs” into perspective). Since he started on Neupogen, one injection a day, I will quote his Mom:

“Since going on the G-CSF, he has not had any hospitalizations for 3 years. Seeing him participate in sports with friends and basically being a ‘normal kid’ has made everything worthwhile”.

“Now at the age of 37, after seeing a total of 17 specialists, after a wide assortment of medicines, many of them with harmful and dangerous side effects, I have been given Neupogen”.

“I can now look forward to a relatively healthy lifestyle, one that most people take for granted”.

“The psychological stress, that constant worry of what will be next and when, takes a drastic toll on the quality of life, as well as the fact of being constantly sick”.

“Her problems started when she was two weeks old. It’s been one infection after another. Her lungs, throat, mouth, ears and so on”.

“For years we lived in a nightmare. A small splinter could infect her arm to the elbow within an hour. We were on alert all hours of the night”.

“Living in an outpost, the emergency helicopters to hospitals were an enormous cost to the system. Now, as of last November, it was like a dream come true she received a daily injection. We’re happy she can finally lead a normal life. She hasn’t had problems with sickness of any kind”.

A Quote from a Physician:

“We can cite instances where this drug saves lives”.

The Neutropenia Support Association Inc. realizes this is a treatment, not a cure and long-term follow-up is needed to determine the continued efficiency and safety of this new product. We recognize the pressure the Ontario Drug Reform Secretariat experiences operating an effective drug program within a tight budget.

The Problem and the Suggested Solution:

We have been involved Canada-wide with applications for treatment. In Ontario, the system seems to defeat its own purpose. Rather than creating an efficient vehicle for processing, the system provides more road blocks than bridges.

We have analyzed and tracked the system in Ontario. Costly duplication of services exists.

As you know, the applications for treatment are presently reviewed by the Drug Quality and Therapeutics Committee for O.D.B. coverage. They examine the severity of condition, the degree of disability caused, and the frequency of treatment and "dosage" requirements. The documentation is substantive, the accountability determined, the process is exhaustive with the families being "audited" by two levels of health care; provincial and county level. The application process in itself has upset and frustrated the families and the physicians with errors and omissions, delays and confusion.

The conclusion is that the patients suffering with severe chronic neutropenia in Ontario have had a difficult time getting through the system. There has to be a better way.

What we want to suggest as a possible solution is, the creation of a special fund (a la Gaucher disease) that would cover treatment costs for all severe chronic neutropenia patients that are deemed eligible by a panel of knowledgeable haematologists.

This proposal would assure fair and equal access to all Ontarians for this debilitating disease and would assure proper treatment in a cost-effective way.

Presentation by Shirley Cox:

The changes Neupogen have made to our family's lives, are almost too hard to express. Our daughter, Stacey, died at the age of five, before Neupogen treatment was available. I don't think a person can say very often, a drug has completely turned around their life. In our case, the drug Neupogen, has completely turned around our entire family's life. Simply stating that life is normal means so much to us, yet is hard for others without this experience to understand. Life before Neupogen meant isolation from the world and all the people we knew in it. Life consisted of doctors appointments and praying for encouraging test results. Our life revolved around disinfectants and preventative techniques of avoiding bacterial infection. As well as being isolated, up until about a year ago, we were not aware of any other neutropenic families enduring this stress and worry. Simple chores like grocery shopping were major problems. I could not expose the children and yet, my husband usually wasn't well enough to take care of them. My health was suffering as I was being pulled from all directions day and night, taking care of a chronically ill family. The word worry does not come close to properly expressing the intense fear you have under these circumstances. I called this fear, hysterical fear, that with each crisis would only grow more intense because there was no hope of anything getting better. Now, with Neupogen, we feel we actually can have a life. The baby receives a dose of .06 cc., his brother .2 cc., and their father .7 cc. of Neupogen daily. These doses are far less than what was reported in media coverage when the drug was first released. My husband's infections are under control and he can now be gainfully employed. Since starting Neupogen treatment he hasn't missed a day of work. My two children have had no serious infections or illnesses since their treatment began. Our family life is now relaxed and the stress of neutropenia is all but gone. But we do realize that Neupogen is a treatment and long term research is still necessary. Neupogen has made such a miraculous change in our entire lives that it is nearly impossible to fully comprehend the total impact it has had. For me, I feel I gave birth to my children, but Neupogen

has given them life.

Before our miracle became a reality, I must stress, absolutely no professional direction was given to us. We identified the Handicapped Children's Benefit Program as having the coverage we required. Careful examination of our financial statements which included a complete audit of our family's income then took place. We were granted a Drug Card and are currently reimbursed monthly for extraordinary expenses. This application has to be resubmitted each and every year for approval.

Initially, my husband was referred to a haematologist in Toronto. Once his Section 8 application was submitted, November 1992, we focused on obtaining a Drug Card. We came up against many many brick walls. People simply would not listen to my story. My husband was working, he was not handicapped, and did not, therefore, qualify for Welfare. After fifty or so denials, I started to feel defeated. My pharmacist then enlightened me with the key words "Minimum Benefits ". With this information, we were successful in having telephone calls forwarded to the right official. Eventually, we were visited by the social worker and we were once again audited and repeatedly audited by different levels of Social Services. The application went back and forth to decide whose jurisdiction it fell under and after, I believe about three or more weeks, a decision was made by all to issue an emergency Drug Benefit Card, at county level, on a month by month basis until a permanent system of coverage was established elsewhere. We are still on the emergency month-to-month status! Finally, with the Drug Card obstacle over, we then had to get approval for coverage of the drug from the Ontario Drug Benefit Programs Branch. Ten weeks went by with no word. When I called we are told that the Drug Qualities and Therapeutics Committee had made a positive decision and a letter was being sent immediately to my husband's physician. The letter our physician received was a denial letter dated December 21, 1992 with option to appeal and this was February 9, 1993. In my mind, there had to have been a mistake. My children could have the drug based on high risk, but my husband, who has volumes of documented hospital admissions and life-threatening infection treatments was flat-out denied. Our appeal was supported by the Neutropenia Support Association, the physician, and many Canadian SCN patients receiving treatment sent letters of support. Documentation was resubmitted and sent again, because of lost or misplaced information, delaying the proceedings even further. Meanwhile, my husband's health seemed to be getting worse with only one or two days passing between antibiotic treatments. I felt at that time, tremendous fear as I felt he would succumb to something soon. I constantly worried how I would possibly, financially and emotionally, carry on all by myself, supporting and caring for 3 children all under the age the age of 4. Life insurance was not a commodity we could buy, because of the pre-existing health condition. I relied heavily through this entire experience on the Neutropenia Support Association for guidance and support. We then received a positive outcome, with coverage being granted March 1993. This experience, exhaustive and completely frustrating, was by no means, even close, in comparison to what our daily nightmare would be without this drug. We are eternally grateful, for the drug coverage and for the new life you were all responsible in giving my family. The

groundwork has been laid now and I would like to thank you all.
Shirley Cox. September 28, 1993

General Comments about the meeting

The government made a commitment to take care of the SCN patients through a mechanism that today is still open for change but in the future will be labeled as a special fund for catastrophic drugs.

For now, SCN patients will be processed under the Section 8 provisions and they will be given an emergency drug card if they are not ODB eligible. There was a promise from the government that they would give high priority to Neupogen requests coming through the Section 8 system.

If, in the future the suggested changes as a result of the reform initiative are politically enacted, Neupogen coverage could fall under a special fund that will envelop all with catastrophic drug coverage. There will be a co-pay depending on ability to pay.

There was some strong discussion on the fact that decrease in hospitalizations is not important to the ODB program, because it is coming from another arm of government.

Comments by Dr. Melvin Freedman:

In the context of children with severe congenital neutropenia, we were discussing patients with neutrophil counts of less than $0.5 \times 10^9/L$ (compared to normal neutrophil numbers of greater than $1.5 \times 10^9/L$). When counts are less than 0.5, the risk of severe, life-threatening infection is extremely high and accounts for the recurrent infections complications that plaque affected individuals. With counts between $0.5 - 1.0 \times 10^9/L$, the risk of infection is much less but there is still a predisposition. With counts over $1.0 \times 10^9/L$, life-threatening infections are not seen unless the neutrophils do not function properly, which can also be a problem with some forms of congenital neutropenia.

As we discussed, G-CSF is a specific and powerful treatment for these patients because it reverses the neutropenia and promotes safe levels of neutrophils for as long as the G-CSF is administered. As far as we know, this is for a life-time unless some new therapy which is equally effective is developed. There is good reason to believe that with modern scientific technology we will have alternative therapies in the future. Currently, though, G-CSF is the best treatment.

There is not question that G-CSF is keeping these patients out of the hospital and away from doctors' offices. Because our hospital budget is being reduced every year, we are forced to find ways to meet the budgetary restrictions. Reduction in hospitalization is a major strategy and keeping neutropenic patients out of hospital has had an important impact on cost-savings.

Research Comments by Dr. M Murthy

My laboratory is investigating various mechanisms of neutrophil functions and their relationship to infections and inflammation. We have recently found a unique mechanism by which human neutrophils synthesize a compound called "leukotriene B₄" which is essential for chemotactic functions. These findings may be highly relevant in the treatment of neutropenia by G-CSF and others. In collaboration with Dr. Eileen Murray, Dermatologist, Winnipeg Clinic, Dr. Bonnie Cham, Cancer Research Foundation

and Dr. Ian Chalmers, Section Head, Rheumatology Clinic, Health Sciences Centre, my laboratory is also investigating the effect of flax oil on neutrophil functions and their possible relationship to psoriasis and arthritis.

Submitted by Dr. M. Murthy.

Very Moving ! Poem Submitted by Shirley Cox

MOVING MOUNTAINS

My babies are so sick. To look you would not know. Their sweet and lovely faces. Full of smiles and all aglow.

My babies, they are dying. We've been told, things just aren't right. So we watch them every moment. In total fear and fright.

When I tuck my babies in their bed and kiss them on their cheeks, I hug and kiss them one more time. As tears stream to my feet.

Our Stacey left us years ago. Her picture's all we have. Her hopes and dreams and love of life. Are memories of the past. I know the boys would love her. If they'd ever met. As she touched so many hearts. In her life before she left.

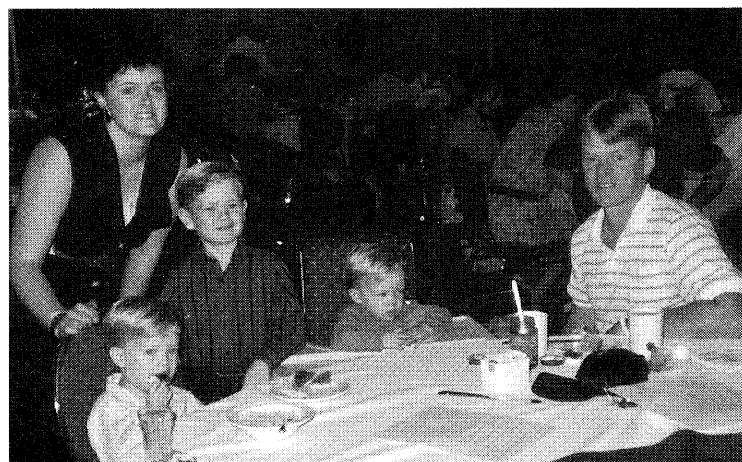
She did not mean to hurt us. Break our hearts and crush our dreams. But she was called to Heaven. And we couldn't intervene. Now Stacey looks from Heaven. And keeps us safe and sound. I think she lent her little hand. For this cure to be finally found.

We know she helped and guided them. We know she knew our pain. The Tragic loss of a precious child. We couldn't endure again.

Our baby's they are healthy now. The sickness gone for good. They laugh, they grow, they jump and sing. I wish they understood.

As parents of sick children. We hope you keep in mind. The faith and promise in our hearts. For the cures we hope you'll find.

For us there's now bright sunlight. For others the darkness looms. But maybe today you'll discover a way. To move mountains and watch flowers bloom.



Shirley Cox and Family At Kingston Conference

Another Info Book under way

Excerpts from the "Information for Cancer Patients and Family" booklet we are facilitating:

Introduction

This booklet is for people of all ages who are receiving chemotherapy for their cancer. It is also for family members or anyone else who is helping the cancer patient. The subject is neutropenia, (NEW TROH PEE NEE AH) a condition where the body does not have enough neutrophils. These are a special type of blood cell which are very important in fighting infection.

Cancer patients often go through periods when they don't have enough neutrophils. In some cases, this is because of the cancer. In others, it is due to the treatment. Either way, the patient is in danger of getting an infection. These infections may make the patient very sick and put them in hospital. They can also cause delays in further treatment of the cancer, and make it harder and take longer to get better.

Avoiding infection is an important goal when fighting cancer. This booklet has been written to provide information about neutropenia and what you can do about it. The more you know about neutropenia and infection, the better able you'll be to help the members of the medical team do their best to help prevent the problem.

This Booklet is in revision #7 and is 16 pages long. Please call for a copy and we will forward one to you upon completion.

American Support Groups

In Los Angeles, Lorna Stevens had the opportunity to meet with a number of physicians and interested professionals considering the development of an international severe chronic neutropenia disease registry. The goals were determined.

Dr. Jon Gerrard and Dr. Bonnie Cham have already communicated with the head of the ethics committee to determine requirements, etc. We consider the development of the M.C.T.R.F. registry to be the pilot project as a catalyst for the international registry. They will communicate with the medical community accordingly. We want to facilitate U.S. support groups as funds and opportunity permits. We have many U.S. families' letters on file with strong interest in helping develop support groups. They have similar issues of concern and the need to participate in their vested future and children's treatment. Please continue to write. Let us know your information needs so we can try to provide in the meantime. We have received many U.S. letters of support and encouragement. Some quotes are as follows:

"Enclosed is my membership form: Thank you for the chance to find out more about Neutropenia and to feel less "alone"

"I've passed the Newsletter along to my doctor and look forward to the next one."

"I received all the info on Neutropenia that you sent me, yesterday, as planned. I want to thank you very much for all the information, as well as for the time you took to speak with me on the phone. The information has been extremely helpful!"

"I would very much like to organize a supportive network in the U.S., or in some way help your organization to be the U.S. counterpart."

"Thanks for sending us your newsletter. We will be happy to speak with anyone who desires."

Small Medical Note

Our Neutropenia Support Association Inc. is aware of the benefit of reducing infection during breast feeding.

Many of our children were diagnosed with neutropenia after breast feeding ended. Infections were experienced during this period, but the mother's "protection" helped considerably in our opinion.

Attention All Support Groups

There will be ongoing media interest.

Please send copies of all human interest stories, newspaper, radio tape, T.V. coverage, etc. to "P.O. Box 243, 905 Corydon Avenue, Winnipeg, Manitoba, R3M 3S7".

This is our "Head Office" address and we want to maintain a central library of information. We will reimburse you for your video/audio tapes - just send us your receipt.

Thank you.

Congratulation to Dr. Jon Gerrard

Congratulations are extended to The Hon. Dr. Jon Gerrard, newly elected M.P. for Portage Interlake, now Sec. of State Science Research & Development.

Canada Volunteer Award

Previously reported in the last newsletter, Lorna Stevens received 'The 125th Anniversary of Canada Commemorative Medal. Mr. & Mrs. J. Benzelock also brought her achievements to the attention of the Canada Volunteer Award Program. The Benzelocks received notification August 17th, 1993 that Mrs. Lorna Stevens had been selected to receive a Canada Volunteer Award Certificate of Merit from The Minister of Health and Welfare, Ottawa, Canada.

The Hon. Dr. Jon Gerrard will present the award.

This certificate is awarded each year to recognize and encourage those who have made valuable voluntary contributions towards improving the health and social well being of their fellow citizens. Formal presentation is being made at 12:30 pm December 12th, 1993 at Westworth United Church. Refreshments will be served. All are invited.

We CAN Too!

One of the contributors talking to Janis Benzelock, asked us for a donation can for his store.

Janis and Jim Benzelock are developing this idea!

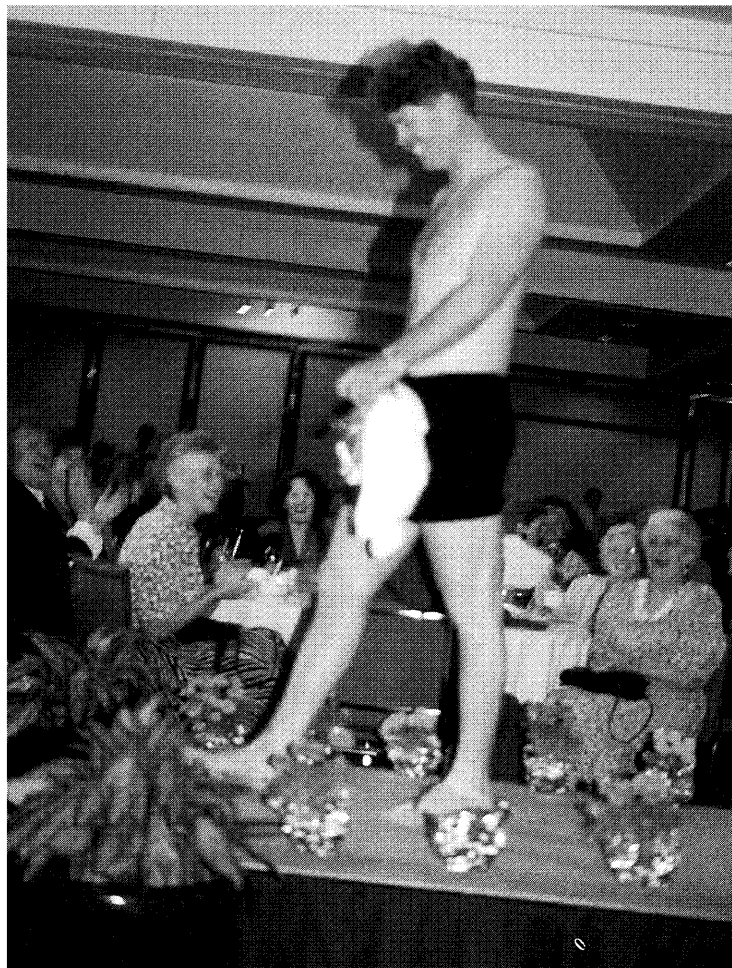
Should you like us to supply your office, bank, or store please call and leave a message on our answering machine at 489-8454.

We will also send out our information booklet.

THANK YOU.



Panache Model with Calendar Firefighter



Lorne Stevens strutting his stuff



*Brian Gamley, Jim & Janis Benzelock
Working hard at the Prize Table for the
Fashion Show!*



Use This Handy Form To Send In Your Tax Deductible Donations

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Send to Neutropenia Support Assoc. Inc. P.O. Box 243, 905 Corydon Ave. Winnipeg, Manitoba R3M 3S7