

The Canadian Society for Mucopolysaccharide & Related Diseases Inc.

2003- 2004 Annual Report

Chairperson's Report 2003-2004



This year has been one of passing the torch. After many dedicated years, Corri Kaweski stepped down as Chairperson of our Society in July 2003. I had the honour of taking over this position, and I have been endeavoring to fill her shoes since that time. In addition, our fearless Executive Director, Lori Di Ilio, told us last September that she was also going to step

down at the end of her contract this summer. The Board then had to try to fill her dedicated shoes as well.

In order to do this, the Board formed a small hiring committee consisting of myself, and fellow board members Karen Hurst and Debbie Braun, and advertised both within the Society and to the general public through Charityvillage.com. We were overwhelmed by the caliber of candidates that applied. We shifted through the sixty-two applications and selected ten to interview - one from within the Society, and nine outside. After a series of lengthy interviews, the committee came to the unanimous conclusion that our own Kirsten Harkins would

be the best choice for the Society. Special thanks to Board member Karen Hurst, whose background in human resources was invaluable, and to Debbie Braun, for undertaking this time-consuming and difficult task.

Both Kirsten and I joined the Board of Directors in the summer of 2002, at our last conference. While we may both be considered novices on the Board, I am confident that with the assistance of the more seasoned board members, and with the continued guidance of Lori and Corri, we will do their legacy proud.

Upon reflecting on her time with the Society, Lori had this to say:

My passion for MPS started 10 years ago when my then 4-year-old son Matthew was diagnosed with MPS III. I recognized my passion when I found myself traveling to various libraries for information, surfing the web, joining the MPS Society and becoming a Board Member. Two years later, I was hired as the Executive Director for The Canadian MPS Society. It has been a constant learning curve from fundraising, to simple letter writing, to planning national conferences. I had no experience in the non-profit sector let alone running a Charity. With the

help and encouragement from former Chair, Corri Kaweski, we essentially ran the organization from our hearts and by what we personally endured through our own experiences having children affected with MPS diseases.

Through trial and error and constant changes we managed to take the Society from a grass roots organization to a viable international society, which is recognized and respected for its dedication and contributions to the MPS Community.

As my job winds down I reflect upon my accomplishments and the heartache I

have endured through the loss of many MPS children and the slow decline of my own MPS child. My focus has been and always will be the families, for without their support and encouragement this organization would not exist.

I would also like to express a special thanks to my husband Rob, my family, and all my colleagues on the Board who have encouraged me over the many years and work so hard with limited resources to do what we all want to do, "make a difference in the lives of children and adults with MPS".

2003-2004

Inside the Annual Report:

Chairperson's Report	1
Society Highlights	2
Partnerships & Research	3
What is MPS? Support	4
Fundraising	5
Review	6
Financial Statements	7
Major Donors	8

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Chairperson's Report—Con't:

I would like to thank Lori Di Ilio and Corri Kaweski for all their hard work this year, and all the years prior. One can just imagine the dedication required, while at the same time trying to make life comfortable for your own MPS children and the rest of your family. Their commitments to our Society will never be forgotten. We would also like to thank their whole families - their husbands, parents, brothers and sisters, who have all done their part to make the Society the success that it is. Most of all, we would like to thank their children, who have given up valuable time with

their mom so that they could help make a better life for not only them, but all the MPS children in Canada.

Words will never be able express our gratitude, and we will miss you very much.



Lori Di Ilio & Corri Kaweski

2003 – 2004 Society Highlights

Much of the past year has been dedicated to planning our 2004 summer conference to celebrate the Society's 20th Anniversary. As well, we continue our advocating efforts for MPS I patients across Canada. In the early part of 2003 we celebrated the approval of Aldurazyme in the U.S.A. and the European Union for use in children affected with MPS I. Approval for Aldurazyme from Health Canada was not forthcoming until the end of May, 2004.

Kirsten and Todd Harkins implemented The MPS Cup Fantasy Hockey Game and Gala held on June 21, 2003, in North Vancouver raising just over \$40,000.00 for the Canadian MPS Society. This exciting event was the first of its kind for the MPS Society generating publicity and an opportunity to bring about more public awareness of MPS Diseases.



The 2003 MPS CUP team.

2003 – 2004 Society Highlights

Through a generous donation from Simon Ibell, The Canadian MPS Society has partnered with The National MPS Society to hire Tracey Halford to write an Everyday Booklet on living with MPS. Questionnaires were sent out across Canada and the US to all families for input on dealing with the very unique needs of MPS children. The booklet is well on its way to completion.

The Society's Jeans Day concept was revised to reflect our new logo and slogan: 'Canadian MPS Jeans Day'. A few Jeans days were held throughout country: It's our hope that more people will embrace this easy and fun fundraiser.

Along with the new Jeans Day design, we developed our new 'Kick Back Week-end Wear' consisting of jean shirts, sweat shirts, t-shirts and three different coloured t-shirts - red, orange, and light blue - for the kids, featuring the Society's logo.

In this past year, our Society also maintained its international presence. Lori Di Ilio, Kirsten Harkins and Tina Hapgood attended the Expert Meeting on Orthopedic Surgery in Manchester, England. The meeting was used as a forum to discuss debate and record the best techniques for managing and treating orthopedic issues in children with MPS. After each discussion a moderator prompted the panel of doctors to reach a consensus as the best approach to treatment. Organizers plan to publish their findings in a future Pediatric Orthopedic Journal.

The launch of our updated website took place in early 2004. We wish to thank Line of Sight and Michael Ball for their endless hours of hard work to bring the completion of the new sight www.mpsociety.ca.



Partnerships & Research 2003 – 2004

With the help of The Sanfilippo Children's Research Foundation the Canadian MPS Society was able to fund two \$4,000 Summer Studentship with Dr. Alexy Pshzhetsky PhD at Hopital Sainte-Justine.

Through the generous donation of the MPS Cup and The Trevor MacDonald Foundation, the Canadian MPS Society awarded \$35,000 to Dr. Lorne Clarke and his team of researchers at the University of B.C. They will use micro array analysis and isotope-coded affinity tag (ICAT) proteomics to investigate the pathogenesis of MPS I utilizing the murine MPS I model.

Ultimately their hope is to identify serum biomarkers of disease that may be useful in the evaluation of disease progression and response to therapy in children with MPS I. In addition, their more exploratory approach will lead to identification of other factors that may underlie the pathophysiology of MPS diseases. ICAT analysis of both immunocompetent and immunocompromised MPS I mice will represent one of the first applications of this type of proteomic analysis to a mouse model of disease.

In early 2004, The Canadian MPS Society became a participating partner in the LSD Research Consortium in order to fund CNS research. This component is a very important objective of our research goals and we are very excited about having pledged \$10,000 U.S. to this collaboration. Visit www.LSDresearch.org for details.

The National Institute of Neurological Disorders and Stroke and the Office of Rare Diseases will partner with the LSD Consortium by issuing a new program announcement (PA) for translational research. The purpose of this PA is to solicit applications from researcher on LSD's, focused on improving central nervous system treatment outcomes; enhancing the effectiveness of delivery and targeting of cells, enzymes drugs and genes into the brain; and developing novel therapeutic modalities. Transitional research is the step beyond basic research and will move the research into the clinical stage.

We would like to thank our members and all of those who have offered their financial support to the Society, permitting us to continue to offer our vital programs of family support, education and research.

Conclusion

I would like to thank my fellow board members who have worked so hard this year to make a difference. Being an MPS parent is challenging enough, but these people have sacrificed what little free time they have to make our Society a better organization. I thank you from the bottom of my heart.

Judy Fowler Byrne
Chairperson

What are Mucopolysaccharide & Related Diseases?

They are rare metabolic diseases referred to as MPS I to IX or more commonly by the name of the doctor who first described the condition.

Mucopolysaccharidoses

- MPS I Hurler, Hurler Scheie, Scheie
- MPS II Hunter syndrome
- MPS III Sanfilippo syndrome A,B,C,D
- MPS IV Morquio syndrome
- MPS V Maroteaux-Lamy syndrome
- MPS VII Sly syndrome
- MPS IX Hyaluronidase deficiency
(MPS V and VIII have been reclassified)

Glycoprotein Disorders

- ML I Mucopolidosis–Sialidosis
- Mannosidosis
- Fucosidosis
- Aspartylglycosaminuria

Multiple Enzyme Deficiencies

- ML II I-cell disease
- ML III Pseudo-Hurler polydystrophy
- IV Mucopolidosis
- Multiple Sulphatase Deficiency

Complex Lipid Storage Disorders

- Tay-Sachs
- Sandoffs Disease
- Niemann-Pick Disease A & B
(Sphingomyelinosis)
- Niemann-Pick Disease C

Providing Support to Families

Founded in 1984, The Canadian MPS Society is a registered not-for-profit organization which represents many children and adults affected with Mucopolysaccharide and related lysosomal storage diseases in Canada, as well as their families, caregivers and professionals. An national organization with programs that work in your community, the Canadian MPS Society is entirely supported by voluntary donations and fundraising.

Smiley Faces on Previous Page

Top Left: Rhys Boyd–MPS 1 Monica Nelis–MPS 1

Middle: Zane & Luke Braun–MPS IV Andrew Lanese MPS II

Bottom: Melissa & Olivier Bilodeau MPS IV

The Societies services include:

Family Referral Directory

Our family referral directory enables families throughout Canada to connect with other families who have similar experiences.

Telephone Helpline

An 800 number has been established allowing families to call the head office free of charge.

Website

Our up-dated website, www.mpssociety.ca keeps members up-to-date on the Society's happenings.

Quarterly Newsletter

The Connection, the Society's quarterly newsletter, is filled with information regarding research, clinical trials, resources, family news, medical matters, helpful hints, advocacy, fundraisers, and other events.

Publications

A wide range of publications and booklets are available on all aspects of disease management

Conferences

Our biennial conferences provide professionals and affected families with vital information on clinical management and treatments, as well as an opportunity to meet each other.

Family Assistance Program

A limited amount of funds are made available to families seeking financial support for equipment & renovations.

Funding Research

The Society funds two summer studentship research grants per year in order to promote research into lysosomal storage diseases, as well as larger grants as funds allow.

Fundraising for MPS 2003 - 2004



M & M. Meats Charity BBQ –Harkins Family



Craft Sale–Meech Family



Jeans Day–Lanese Family



Jeans Day–Cove Cliff Elementary School



Great Lake Walk– Brooke Hosdson, with support from the Nimmo Family



Yard Sale–Bankert Family

To the Members of
The Canadian Society for Mucopolysaccharide
& Related Diseases Inc.

We have reviewed the balance sheet of The Canadian Society for Mucopolysaccharide & Related Diseases Inc as at March 31, 2004 and the statement of revenue expenditures and surplus for the year then ended. Our review was made in accordance with generally accepted standards for review engagements and accordingly consisted primarily of enquiry, analytical procedures and discussion related to information supplied to us by the Society.

A review does not constitute an audit and consequently we do not express an audit opinion on these financial statements.

In common with many charitable organizations, the Society derives part of its revenue from the general public in the form of donations and fundraising income, which are not susceptible to complete review. Our review of revenue from donations and fundraising income was limited to the amounts recorded in the records of the Society. Accordingly, we were unable to determine whether any adjustments to revenue and surplus might be necessary.

Except for the effect of any adjustments, if any, which might have been required had we been able to complete our review of revenue as described in the preceding paragraph, nothing has come to our attention that causes us to believe that these financial statements are not, in all material respects, in accordance with generally accepted accounting principles

BDO Dunwoody LLP
Chartered Accountants
Toronto, Ontario

June 24, 2004

Balance Sheet
(Unaudited)

March 31	2004	2003
Assets		
Cash and short term deposit receipts	96,535	\$73,979
GST receivable	299	236
Accrued interest receivable	378	240
	<u>97,212</u>	<u>\$74,455</u>
Liabilities and Surplus		
Accounts payable	500	500
Surplus	96,712	\$73,955
	<u>97,212</u>	<u>\$74,455</u>

On behalf of the Board of Directors

Lori Di Ilio
Executive Director

Financial Statements for 2003–2004 Year Ending

For year ending March 31	2004	2003
Revenue		
Donations	83,857	\$36,500
Fundraising, net of related expenses	44,838	15,079
Subscriptions	2,430	3,023
Interest	1,688	1,213
Sundry	824	13,183
	<u>133,637</u>	<u>69,048</u>
Expenditures		
Conferences	-	16,461
Consulting Fees	9,000	8,500
Family Assistance donations	3,000	4,500
Meetings	3,404	49
Non-recoverable GST (recovery)	801	904
Office Equipment	2,945	-
Office expenses	1,954	3,752
Professional fees	600	550
Printing & postage	5,196	7,658
Registration fees	30	42
Salaries	34,128	23,968
Telephone and Internet	3,998	2,733
Travel	2,824	-
	<u>67,880</u>	<u>69,117</u>
Grants	<u>43,000</u>	<u>8,000</u>
	<u>110,880</u>	<u>77,117</u>
Excess (Deficiency) of revenue over expenditures for the year	22,757	(8,069)
Surplus, beginning of year	<u>73,955</u>	<u>82,024</u>
Surplus, end of year	<u>96,712</u>	<u>\$73,955</u>

Major Contributors 2003/2004

Cove Cliff Elementary School
Mr. & Mrs. Kaweski
Lorraine & Harry Meech
Biomarin
Trevor MacDonald Foundation
Cambridge Golf Course
Betty & Barry Done
Sanfilippo Children's Research Foundation
Cathy & Ralph Bankert
Leonard & NormaLinton
Genzyme
The Bilodeau Foundation
Milo Bigler
Time Out Source for Sports
Bill Adams
Hagen's Travel & Cruises
HMY Airways
Dollarton Esso
Deep Cove Yacht Club
David Wills and the Dynamics
Grant's Digital Diner

Thanks to all our contributors.
Your donations are greatly appreciated.

Board Of Directors 2003/2004

Chairperson

Corri Kaweski/Judy Byrne

Treasurer

Karen Hurst

Board Members

Barb Boland

Doris Hapgood

Kirsten Harkins

Todd Harkins

Debbie Braun

Executive Director - Lori Di Ilio

Executive Assistant—Tina Hapgood

Office assistants—Rob Di Ilio, Lorraine Harding

Accountant - BDO Dunwoody

Bank - TD Canada Trust

Registered Charity # 12903 0409 RR0001

Advocacy

As we say good-bye and say thank you to Lori Di Ilio for her years of service, we endeavour to continue to develop her vision of a society whose aims are to support individuals and families affected with MPS diseases, educate the general public and medical professionals about MPS, and raise funds for research. The Canadian MPS Society faces an exciting but challenging future: increased education and advocacy will be required for those who may benefit from new treatments, while support must be maintained for

those for whom treatments have yet to be developed.

In the past year, we have worked diligently to ensure Aldurazyme® enzyme replacement therapy would be approved for children with MPS I in a timely manner. Several board members, along with Lori, participated in letter-writing campaigns and a petition was circulated at the 2003 MPS CUP requesting expedited licensing of Aldurazyme, and subsequent funding.

As more treatments become available for MPS diseases, we anticipate that

our role as patient advocates will grow substantially, and we look forward to the day when *all* Canadians with MPS and related diseases will be receiving treatments for their devastating illnesses.

Kirsten Harkins
Board Member
Executive Director (2004/05)

