2008—2009
Annual Report

The Canadian Society for Mucopolysaccharide & Related Diseases Inc.

Conquering MPS: Learn. Live. Cure.

> "Once you choose hope, anything's possible."
>
> -Christopher Reeve

What an exciting year 2008—2009 was for the Canadian MPS Society! Years of hard work culminated in a unique celebration of hope as hundreds of people converged on downtown Vancouver to teach, learn, connect, and inspire. It's difficult to adequately express how meaningful it was for us to host the 10th International Symposium on MPS & Related Diseases, but suffice it to say it was a testament to all those who have supported the Society since its inception that we were in the position to host such a world-class event. Please read more about the Symposium inside the Annual Report, and thank you to everyone who has helped us over the years to get to where we are now.

This fiscal year was one where some patients began life-changing treatments, some received news of upcoming clinical trials (plans for clinical trials for MPS IV, commonly known as Morquio syndrome, were announced just prior to the Symposium), and still other families carried on with the day-to-day challenges of managing diseases for which no treatments are on the horizon....yet. We will continue to believe in a brighter future for *all* Canadians with MPS & related diseases, and thank you for sharing in that vision.

Judy Fowler Byrne
Chairperson

Kirsten Harkins
Executive Director

Inside the Annual Report:

Left: Adele Grenon-LeMaitre & Sarah Byrne (MPS I-H) support Team MPS at the Toronto Waterfront Marathon; Right: Maria Praehofer (MPS IV) & Valerie Wiesbauer at the 10th International Symposium on MPS & Related Diseases

Our Mission: The Canadian Society for Mucopolysaccharide & Related Diseases (The Canadian MPS Society) is committed to supporting families affected with MPS and related diseases, educating medical professionals and the general public about MPS and related diseases, and raising funds for research so that one day there will be cures for all types of MPS and related diseases.

What are MPS and Related Diseases?

Mucopolysaccharide (MPS) and related diseases are lysosomal storage disorders (LSDs) caused by genetically inherited enzyme deficiencies. Because affected individuals lack particular enzymes necessary for normal cell degradation and recycling, substances store throughout their bodies, causing progressive damage to their hearts, bones, joints, respiratory systems and, sometimes, central nervous systems. While babies affected with MPS or related diseases often show no signs of disease, symptoms appear and intensify as storage increases.

HOW ARE MPS AND RELATED DISEASES INHERITED?

Lysosomal storage diseases are usually autosomal recessive disorders, inherited from healthy parents who have no idea they carry a common recessive gene: for carrier parents, there is a one in four chance with every pregnancy that their child will inherit one recessive gene from each parent and suffer from the carried disease. MPS II (Hunter Syndrome) and Fabry Disease are x-linked recessive disorders, meaning they are transmitted by carrier mothers to her sons: for carrier mothers, there is a one in two chance of an x-linked disorder occurring in the birth of a son. Parents of an affected child have the option of prenatal testing to determine if their next child will be affected by the same disease, and should seek genetic counseling before planning to have additional children or to inquire about available carrier testing for their healthy children. The occurrence of MPS in the population is estimated to be one in 25,000 births.

WHAT ARE THE MAJOR CHARACTERISTICS OF MPS?

A wide spectrum of clinical involvement is seen in all MPS and related diseases ranging from onset of symptoms at birth leading to death in early childhood to later onset with a near normal life span. While specific enzyme deficiencies, and effects, vary from syndrome to syndrome, characteristics are often shared by individuals with MPS including: course facial features, short stature, corneal clouding, speech and hearing impairment, chronic runny nose and diarrhea, hernias, heart disease, bone disease, stiff joints, liver and spleen enlargement, hyperactivity, mental retardation, and shortened life expectancy.

TREATMENTS:

Currently there is no cure for MPS or related lysosomal storage disorders and until recently, treatment for MPS and related diseases has been primarily symptomatic, with bone marrow transplantation considered a successful, although high-risk, procedure in some cases. Research in the past decade, however, has led to exciting advancements in gene therapy as well as to the development of enzyme replacement therapies (ERTs). Continued research is necessary in order to find cures for all types of MPS and related diseases.

**A full list of lysosomal storage disorders can be found on our website:*
www.mpssociety.ca

2008—2009 Board of Directors and Medical Advisory Board

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Kirsten Harkins

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Registered Charity # 12903 0409 RR0001

Support for families. Research for a cure.

Conquering MPS: Learn

When the Canadian MPS Society originated in 1984 as a support group for affected families, its founder Sheila Lee undoubtedly did not imagine the Society would end up hosting an international symposium for 856 delegates from 37 countries 25 years later. The 10th International Symposium for MPS & Related Diseases was held June 26-29, 2008, and was a highlight of our Society's existence. It was a thrill to welcome affected families (including 141 children), medical professionals, researchers, industry leaders, and 78 volunteers to Vancouver for a weekend filled with education and inspiration. Not only did Canadian families benefit from the educational content of the meeting, they had a once-in-a-lifetime opportunity to meet other affected families from around the world who truly understood them—no matter what the language—and that was something everyone in attendance will always cherish.

Conquering MPS
Learn. Live. Cure.

10th International Symposium
on Mucopolysaccharide
and Related Diseases

June 26 – 29, 2008
Vancouver, British Columbia Canada

Please visit the homepage of our website to view the children and youth in action while participating in "Camp Canada" and "Camp Adventure." They soared like eagles while tackling the high ropes at Trinity Western University and formed lasting friendships while creating lanterns for the fabulous procession that was the highlight of the gala dinner. The strength and courage of the children—both affected and siblings—were powerful reminders of why we were all gathered together: to find ways to create brighter futures for all children affected with MPS and related genetic diseases.

Kushala Allken (MPS IV A) with her brother Dhavishen at the Vancouver Aquarium during the MPS Symposium.

Several new policies and procedures were developed in advance of the 2008 Symposium, with guidance from the UK MPS Society and with thanks to pro bono legal work provided by McCarthy Tétrault LLC. These policies and procedures are now in place and will help guide us in our future endeavours: Emergency Protocol, Good Hygiene Protocol, Seizure Protocol, Volunteer Carers' Conduct Policy, Child Protection Policy and Procedures, Anti-bullying Policy, Policy on Moving and Handling for Volunteers, and Privacy Policy.

Genèvieve Poulin with her children Olivier & Mélissa Bilodeau (MSPS IV A), and Melissa's dog, in Vancouver.

In addition to offering a Travel Bursary Program to support travel to the symposium for our member families, all symposium family program presentations are available for download on our website, and all scientific presentations are available in video format for viewing at www.goldinfo.org.

For those who were unable to attend the symposium in Vancouver, there were other educational opportunities offered in 2008/2009:

In March 2009, following a workshop on Health Technology Assessment sponsored by Canadian Organization for Rare Disorders, a MPS family gathering was held in Edmonton, Alberta, featuring a talk by Medical Advisory Board member Dr. Robin Casey.

A new MPS I Family Resource Binder was developed and distributed to all families affected with MPS I, with plans to develop similar binders for the other MPS syndromes.

We continued to publish our newsletter *the Connection* to provide updates to our member families on all things related to the MPS community, and we worked to develop our website to make it more informational and up-to-date.

Jordan, Tyler & Riley Miranda (MPS II) with their mother Nicole at the MPS family gathering in Edmonton.

Conquering MPS: Live

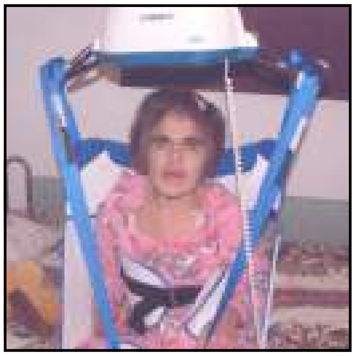
The Canadian MPS Society is committed to helping families affected by MPS & related diseases live their best lives. Without the continued financial support from those who believe in our vision, we wouldn't be able to continue to provide **advocacy and other support** for those who need it.

In 2008/2009, we worked to educate the federal government about the need for Orphan Drug Policy and were thrilled when on May 7, 2008 MP Don Bell's Motion 426 received overwhelming approval in the House of Commons.

At the Symposium, 'Life for MPS' awards were presented to Mark Dant (L)& Dr. John Hopwood (R). Pictured with these two very deserving recipients are Kirsten Harkins & MP Don Bell.

We also worked with provincial governments to ensure patients with MPS received the treatments they needed. In December 2008 the Alberta government announced its Rare Diseases Program, and the Ontario government announced that Elaprase (enzyme replacement therapy for MPS II) would be the pilot drug to move through its new Drugs for Rare Diseases program and patients would soon benefit.

On a practical level, we provided Financial Assistance Program grants to families to help alleviate the costs often associated with raising a child with unique needs, and for the first time, we published an electronic version of our **family referral directory**, to allow our members to connect with each other on a more personal level, even if separated by the vastness of our great country.



Sheena Hodder (MPS III A) in a lift purchased in part by the Society.

We continued to provide **bereavement support** to families grieving the loss of a loved child to MPS or a related disease, and counseled newly-diagnosed families through our 1-800 #.

The following fundraising events, organized by our members and supported by their dedicated circles of family and friends, helped the Society net an amazing $188,151 in fundraising revenue during the 2008-2009 fiscal year. Thanks so much!

Canadian MPS Jeans Days were held at various locations in October 2008, raising both funds & awareness.

The 2008 MPS CUP Fantasy Hockey Game and Gala netted over $160,000 to "help kids raise their arms in victory over MPS." We thank the players, committee members, sponsors, donors & countless volunteers for their commitment to our Society & its members.

Nicklas Harkins (MPS I-H/S) with Mattias Ohlund & Markus Naslund at the 2008 MPS CUP.

Team MPS participated for the second time in the **Scotiabank Toronto Waterfront Marathon** on September 28, 2008. Several Society members and supporters either ran, walked, or wheeled to raise funds and awareness for the Society and over $8,000 was raised thanks to the enthusiastic group effort and the leadership of team captain Judy Byrne. We look forward to Team MPS's continuing participation in this annual event.

Just Singin Round (JSR): The Canadian MPS Society was the featured charity at February 2009's JSR event at the Vancouver Rowing Club. JSR is a monthly singer-songwriter showcase – funds raised are divided equally at the end of the year amongst 12 participating charities. The Society received $2,500 from JSR in December 2008 for February 2008's involvement, and will receive a cheque in December 2009 for our participation in February 2009.

Our third **Annual Fund** campaign took place in December 2008 and resulted in $3180 in revenue.

Thanks to everyone who organized events—even small amounts raised add up in a big way!

Jeans Day at Butler Elementary School in Bedford, Quebec

Conquering MPS: Cure

Thanks to the generous support of our donors in 2008/09, the Society was able to the following RESEARCH GRANTS:

Dr. Lorne Clarke, with Kirsten Harkins and Judy Fowler Byrne, is not only the recipient of our largest grant to date, he was also the Scientific Chair of our Symposium. We are so grateful to have so many brilliant and caring doctors, like Dr. Clarke, working with us toward a cure.

Proteomic studies of skeletal disease in a murine model of MPS I

Principal Investigator:
Dr. Lorne Clarke
Institution: University of British Columbia, Vancouver, BC
Funds allocated:
$100,000 (two year grant—$50,000 per year)

Identification of Pharmacological Chaperones for treatment of MPS I

Principal investigator:
Dr. Allison Kermode
Institution: Simon Fraser University, Burnaby, BC
Funds allocated: $50,000 (one year grant)

A comparison of two biomarkers on treatment outcome in mucopolysaccharide disease

Principal investigator: Dr. Brian Bigger
Institution: University of Manchester, Manchester, UK
Funds allocated: $50,000 (one year grant)

Development of gene expression-targeted isoflavone therapy (GET IT) for Mucopolysaccharidosis type III

Principal Investigator: Grzegorz Wegrzyn
Institution: University of Gdansk, Poland
Amount funded: ~$4,000* (one year grant)
*This grant was funded collaboratively with various international MPS societies.

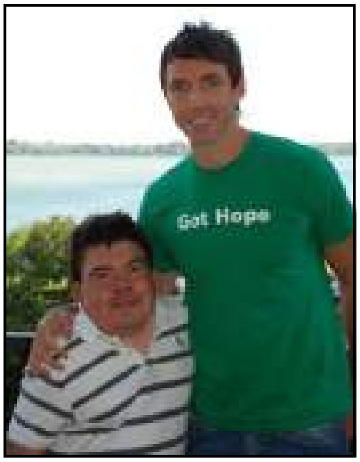
Mac Stiles (MPS I-H)

Summer Studentship Research Grant:

Mucopolysaccharidosis Type IIIB (Sanfilippo syndrome): Affinity purification of human recombinant α-N-acetylglucosaminidase from cultured Sf9 cells for uptake studies and potential therapeutic treatment

Recipient: Sarah Truelson
Institution: University of Victoria, Victoria, BC
Supervisor: Dr. Francis Choy
Amount funded: $4,000.00

In order to achieve our goals, the Society has forged strong partnerships with the following national and international organizations and networks:



Simon Ibell (MPS II) with his friend Steve Nash, who posted a message on youTube encouraging the Ontario Health Ministry to fund ERT for MPS II.

CORD (The Canadian Organization for Rare Diseases):
Our Executive Director sits on the Board of Directors of CORD and our society continues to support CORD in its efforts to have rare disease treatments funded and to have orphan drug policy implemented in Canada.

GOLD (Global Organization for Rare Diseases):
We are affiliate members of this international organization, aimed at improving education and knowledge about LDSs, fostering collaborative research, creating standards for diagnosis and testing for LSDs and fostering coordination amongst existing patient registries.

The International MPS Network:
The Society is a member of the International MPS Network, and hosted its 2008 meeting prior to the International MPS Symposium in Vancouver. Together, we participated in International MPS Awareness Day on May 15, 2008.

The Purcell Family at their second *Tacos for Trey* fundraiser. $62,257 was raised for the MPS II Research Fund in 2008/2009 — "Got Hope?"

*the $59,114 listed as revenue on the financial statement represents the Fund's net revenue, after all expenses.

Financial Statements for the Year Ended March 31, 2009

To the Directors 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, 2009, 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.

Based on our review, nothing has come to our attention that causes us to believe that these Financial Statements are not, in all material respects, in accordance with Canadian generally accepted accounting principles.

Quantum Accounting Services Inc.
Vancouver, BC
July 6, 2009

Balance Sheet as at March 31, 2009
(Unaudited)

March 31	**2009**	**2008**
Assets		
Cash and short term deposits	207,096	91,910
Sales tax receivable	434	1,733
Accrued interest receivable	792	628
Prepaid Expenses	1,500	1,500
Total Current Assets	209,822	95,771
Liabilities		
Accounts payable	1,100	950
Deferred Revenue	-	-
Deferred Revenue**	-	6,395
Total Current Liabilities	1,100	7,345
Surplus	208,722	88,426
	209,822	95,771

**2008 MPS CUP

On behalf of the Board of Directors,

Carrie Nimmo	Judy Fowler Byrne
Treasurer	Chairperson

Notes to Financial Statements:

(Unaudited)

Note 1. Organization

The Canadian Society for Mucopolysaccharide & Related Diseases Inc. is incorporated under the laws of Canada as a not-for-profit organization and is registered under the Income Tax Act as a charitable organization and as such is not subject to income taxes. The Society's aim is to provide support to Canadians affected by lysosomal storage diseases (LSDs), to raise public and professional awareness of LSDs, and to fund research for a cure.

Note 2. Significant Accounting Policies

Revenue Recognition

The Society follows the deferral method of accounting for contributions.

Capital Assets

Capital assets are not recorded on the balance sheet. Expenditures for capital assets in the year are recorded as expenses and disclosed in the statement of operations. The capital assets of the Society consist of office equipment and computer software.

Volunteer Services

The work of the Society is dependent on the efforts of many volunteers. Because these services are not normally purchased by the Society and because of the difficulty of determining their fair value, donated services are not recognized in these financial statements.

Financial Instruments

Unless otherwise noted, it is management's opinion that the Society is not exposed to significant interest rate or credit risks arising from its financial instruments.

Significant cash balances are being held at one major financial institution. The Society has a term deposit totaling $72,435 which bears interest at 3.75%, semi-annually and matures December 2010.

Note 3. Fundraising

Fundraising revenue is comprised of gross revenue in the amount of $213,323 (2008—$126,433) less expenditures in the amount of $25,172 (2008—$24,906).

Financial Statements for the Year Ended March 31, 2009

For year ended March 31, 2009:	2009	2008
Revenue		
Fundraising, net of related expenses (Note 3)	188,151	101,527
Donations	53,526	57,467
2008 Symposium	97,473	10,000
Awareness bracelet sales	42	320
Memberships	3,554	3,089
Interest	2,691	2,372
Sales	2,555	323
Conference Revenue	250	-
MPS II Research Fund (see note on page 5)	59,144	2,363
Miscellaneous Income	3,111	2,283
Total Revenue	410,497	179,744
Expenditures		
Salaries and contract fees	66,610	44,665
2008 Symposium	19,685	19,787
Printing, brochures and postage	7,887	7,279
Awareness bracelets	-	-
Telephone and internet	3,602	3,205
Travel	4,327	9,135
Office lease	4,000	-
Office expense	4,500	4,719
Office equipment	2,439	-
Family assistance donations	7,886	4,724
Computer Software	1,650	1,590
MPS II Fundraising expense	-	1,405
Consulting/Website fees	-	-
Non-recoverable GST	1,070	1,676
Insurance	1,057	493
Professional fees	1,250	1,050
Meetings	3,200	2,192
Conferences	-	-
Membership Dues	855	810
Promotional Items	2,253	-
	132,271	102,730
Research grants	157,930	73,000
Total Expenditures	290,201	175,730
Excess/(Deficiency) of revenue over expenditures for the year	120,296	4,014
Surplus, beginning of year	88,426	84,412
Surplus, end of year	208,722	88,426

We gratefully acknowledge the support of our donors.

Many thanks to *everyone* who made contributions to the Society during our April 2008—March 2009 fiscal year.
Special thanks to the following major sponsors and donors, as well as the organizers of major fundraisers:

DIAMOND (Donations of $10,000 +)

The Jim Pattison Foundation*
Lower Mainland Steel
Genzyme Canada Inc
Dr. Mark Olesen Inc.
Biomarin Pharmaceutical Inc
Matcon Excavation & Shoring Ltd.
Mike & Lisa Hudson
Vidalin Family Foundation
The MPS CUP Fantasy Hockey Game & Gala—The Harkins Family
Tacos for Trey*—The Purcell Family

PLATINUM (Donations of $5,000 +)

Ryan & Deb Purcell*
Shire Human Genetic Therapies
CC Bouchard Ventures Inc.
Southwest Contracting Ltd.
Biomarin Pharmaceutical Inc.(Canada)
Ledcor Construction Limited
Hardwall Construction
The Estate of Joan Mary Warwick
Classic Rock 101
Team MPS at the Scotiabank Toronto Waterfront Marathon—Judy Byrne

GOLD (Donations of $2,500–$4,999)

Bosa Foods
Peter Muirhead
Synergy Foundation
North Shore News
The Sutton Place Hotel
The Destination Slope & Surf Outfitters

SILVER (Donations of $1,000–$2,499)

Tiger Williams & Mendel Vysohlid
North Shore Winter Club
Betty Done
Lehigh Northwest Materials Ltd
NHL Alumni
McCarthy Tetrault Foundation
United Way Ottawa
Gerda Spielmann*
James Adamson
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Linda Jones
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Hans Hamberger
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Robertson Floors
Canadian MPS Jeans Day at Cove Cliff Elementary School
Spring Fling—The Nelis-DuPont Family
The Mount-Royal Family Bike Ride—The Silveri/Duffy Family

BRONZE (Donations of $500–$999)

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Gil & Jessica Rosenfeld
Val Ramsey*
Heather Cehak*
Peter & Charis Kalesnikoff *
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The Great Lake Walk—Brooke Hodson
Walk with Nature—The Nelis-DuPont Family

**indicates MPS II Research Fund donation*