

2007 – 2008 Annual Report



The Canadian Society for
Mucopolysaccharide &
Related Diseases Inc.

Support for families. Research for a cure.

**"We shall draw
from the heart of
suffering itself
the means for
inspiration and
survival."**

–Sir Winston
Churchill

The 2007-2008 fiscal year was an exciting one for The Canadian Society for Mucopolysaccharide & Related Diseases Inc. (The Canadian MPS Society). Preparations went into high gear towards hosting the 10th International Symposium on MPS & Related Diseases, giving researchers, medical professionals, and affected families from around the world an opportunity to gather in Vancouver in June 2008 to share information and experiences, and to be inspired by each other.

In addition to our commitment to educating the public, the medical community and the government about MPS and related diseases, the Society is more committed than ever to providing critical support for affected families, and to funding research for a cure. But we can only do this with your help! On behalf of the Society's Board of Directors and members, thank you for believing in our vision of a future where those with MPS and related diseases are able to grow into healthy adults, live happy and productive lives, and be treated with dignity and respect.

Judy Fowler Byrne
Chairperson

Kirsten Harkins
Executive Director



Inside the Annual Report:

Letter from the Chairperson & Executive Director	1
What are MPS & Related Diseases? Board of Directors, & Medical Advisory Board	2
Family Support	3
Fundraising & Research	4
Partnerships & Collaboration	5
Financial Statements	6
Major Donors	8



Zane & Lucas Braun (MPS IV)

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*

2007-2008 Board of Directors and Medical Advisory Board

EXECUTIVE DIRECTOR:

Kirsten Harkins

BOARD OF DIRECTORS:

Chair—Judy Fowler Byrne
MPS I parent, ON

Vice-Chair—Debbie Braun
MPS IV parent, ON

Treasurer—Delane Terrillon
CGA, BC

Secretary—Carrie Nimmo
MPS I aunt, BC

Barb Boland
MPS III parent, NL

Todd Harkins
MPS I parent, BC

Aubrey Hawton
MPS III parent, ON

Simon Ibell
MPS II adult, ON

Jim LeMaitre
MPS I parent, ON

Jean Linden
MPS III parent, BC

Mary Nelis
MPS I parent, QC
Deb Purcell
MPS II parent, BC
Kathie Stephens
MPS III caregiver, ON

MEDICAL ADVISORY BOARD:

Chair—Lorne Clarke, MD, CM, FRCPC, FCCMG
University of British Columbia, BC
Robin Casey, MD, MSc
Alberta Children's Hospital, AB
Joe Clarke, MD, PhD
Hospital for Sick Children, ON
Cheryl Rockman Greenberg, MD, CM, FRCP
Winnipeg Children's Hospital, MB
Mark Ludman, MD, FRCPC, FCCMG
IWK Health Centre, NS
Serge Melancon, MD
Montreal Children's Hospital, QC

Tony Rugar, BSc, PhD
University of Western Ontario, ON
Sylvia Stockler, MD
BC Children's Hospital, BC



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www.mpssociety.ca

Registered Charity # 12903 0409 RR0001

Support for families:

SUPPORT & EDUCATION:

The Canadian MPS Society originated as a support group for affected families, and thanks to the generous financial support of our committed members, sponsors and donors, we were able to continue to develop our family support programs during the past fiscal year. Some highlights:

We implemented a new **Canadian Family Travel Bursary Program** as part of our Family Assistance Program in order to help Canadian families attend the 10th International Symposium on MPS & Related Diseases in Vancouver. In addition, we funded several Family Assistance Program grants, totaling \$4,724.00, to help families with costs associated with caring for their children with MPS.



A new **website** was launched, with a platform to support online donations, event registration, and membership renewal.

We continued to publish our popular **quarterly newsletters** to allow our members to keep up-to-date on other

affected families, MPS medical news and upcoming events, 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.

We continued to provide **bereavement support** to families grieving the loss of a loved child to MPS or a related disease.

The Society issued a **press release** June 27, 2007 to celebrate Health Canada's **marketing approval of enzyme replacement therapy for MPS II** (Hunter Syndrome), and although a few patients in BC and Alberta were given access to this treatment, the federal Common Drug Review made a negative funding recommendation in December 2007, as did Ontario's Committee to Evaluate Drugs. The Society fought to ensure all Canadian patients needing treatment were given access, and issued another press release on February 29, 2008—the first **International Rare Disease Day**—to raise awareness of the inequity of the funding situation in Canada. To celebrate International Rare Disease Day, Canadians with MPS were featured in a National Post newspaper supplement and at a “newsmakers’ breakfast” in Ottawa. And, on February 28, Society members were part of a group of health advocates who met with 65 Members of Parliament to educate them on the issues relating to those affected with rare disorders in Canada.

As part of our new **Patient Outreach Program**, and in order

to address some of the issues arising for families affected with MPS II in the eastern part of the country, the Society invited affected families—both member families and those who were



not already involved with the Society—to attend an **MPS II patient meeting** in Ontario on February 24, 2008 at Sick Kids Hospital in Toronto.

We also had representation at an **MPS IV family meeting** held in Quebec

by the PQMGO, where we reached out to Quebecois families, many of whom are now new members of the Society. We published our brochures for the first time in French and began adding French content to our newsletters, with hopes of translating our MPS IV booklet into French so that we can better serve our Francophone members.

International MPS Day—May 15, 2007—was a day to recognize, remember, and honour those whose lives are touched by



MPS, and we recognized this day with our international friends by sending out e-cards from the Join the Search campaign, and encouraged our members to mark this day in whatever way was meaningful to them.

In the summer of 2007, we collaborated with Genzyme on an international **MPS I patient survey**, along with several other international MPS societies. Information

collected from the patient surveys was published and is available for viewing at www.mpssociety.org.

Clockwise from left: Sheena Hodder (MPSIII); Nathaniel Phaneuf (MPS II); Trevor MacDonald (MPS I-H) at his 10 year post-transplant tourney.

Research for a cure:

FUNDRAISING:

The Society's initiatives wouldn't be possible without the funds raised by many of our dedicated members and their committed circles of supporters. The Society netted an amazing \$101,527.00 in fundraising revenue during the 2007-2008 fiscal year. Thanks to all involved for your support.

Canadian MPS Jeans Days were held at various locations in October 2007, raising both funds and awareness.



The **2007 MPS CUP Fantasy Hockey Game and Gala** netted over \$80,000.00 to "help kids raise their arms in victory over MPS". We thank the players, committee members, and countless volunteers for their dedication to our Society and its members.



Team MPS participated for the second time in the **Scotiabank Toronto Waterfront Marathon** on September 30, 2007. Several Society members and supporters either ran,



walked, or wheeled to raise funds and awareness for the Society and over \$12,000.00 was raised thanks to the enthusiastic team effort and the leadership of team captain Judy Byrne. Special thanks to Pierre Chayer, who ran the full Marathon and raised several thousand dollars for our cause. Thanks to everyone who took part – we look forward to Team MPS's continuing participation in this annual event.

The Chayer family with Jonathan (MPS III) at the 2008 Scotiabank Toronto Waterfront Marathon; Right: Kyle Turris & Nicklas Harkins (MPS I) at the 2007 MPS CUP.

Just Singin Round (JSR): The Canadian MPS Society was the featured charity at February 2008'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 \$2,500.00 from JSR in December 2007 for February 2007's involvement, and will receive a cheque in December 2008 for our participation in February 2008. We are excited to be on the roster again for 2009.

Annual Fund: Our third Annual Fund campaign took place in December 2007 and resulted in \$3,640.00 in revenue.

Many other local fundraisers also took place. Thanks to everyone for their efforts—even small amounts raised add up!

2007-2008 RESEARCH GRANTS:

Brain-targeted MPS II therapy delivered by microencapsulated cells

Principal Investigator: Dr. Murray Potter
Research conducted at: McMaster University, Hamilton, ON
Funds allocated: March 2007: \$20,000.00
Funds allocated: June 2007: \$20,000.00
(the first installment was issued in the 2007-2008 fiscal year)

A monoclonal Antibody Based ELISA for Heparin Cofactor II- Thrombin Complex

Principal Investigator: Dr. Lorne Clarke
Research conducted at: University of British Columbia
Funds allocated: March 2008: \$40,000.00

NAGLU – Apolipoprotein for treatment of Sanfilippo B syndrome

Principal Investigator: Dr. Patricia Dickson
Research conducted at: Los Angeles Biomedical Research Institute
Funds allocated: March 2008: \$40,000.00

2007 SUMMER STUDENTSHIP RESEARCH GRANTS:

Brain-targeted MPS II therapy delivered by microencapsulated cells

Researcher: Jason Schwindt
Research conducted at: McMaster University Medical Centre
Amount allocated: \$4,000.00

Evaluation & implementation of new fluorometric enzyme assays, for diagnosis & monitoring of patients with lysosomal disorders

Researcher: Patrick Wong
Research conducted at: University of British Columbia
Amount allocated: \$4,000.00

Mucopolysaccharidosis Type III B (Sanfilippo syndrome): Expression & purification of human recombinant α -N-acetylglucosaminidase from cultured Sf9 cells for uptake studies & potential therapeutic treatment.

Researcher: Tasha Kulai
Research conducted at: University of Victoria
Amount allocated: \$4,000.00



We can't do it alone!

PARTNERSHIPS:

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

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. The Society was actively involved in CORD's first-ever International Rare Disease Day initiatives, and in its work with MP Don Bell around his Motion-426 in support of rare disorders.



The Canadian MPS Society's Executive Director Kirsten Harkins with CORD President Durhane Wong-Reiger and North Vancouver MP Don Bell at the "newsmakers' breakfast" held in Ottawa in celebration of the first ever International Rare Disease Day;

Right: Trey Purcell (MPS II) taking some shots at the inaugural Tacos for Trey event, which benefited the MPS II Research Fund.

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 to develop a global resource. GOLD videotaped all scientific sessions at the International Symposium on MPS & Related Diseases in Vancouver and has posted them for viewing on its site: www.goldinfo.org.

The International MPS Network:

The Society is a member of the International MPS Network, and meetings were held in Poland in the spring of 2007 and in Belgium in the fall of 2007 to discuss issues of common concern.

Canadian Lysosomal Storage Disorder Network (CLSDN): CLSDN is made up of leaders of various Canadian lysosomal storage disorder patient groups, representing MPS, Niemann-Pick, Fabry, Gaucher, and Tay-Sachs patients. We look forward to future collaboration with this group as we work toward our common goals.



MPS II Research Fund:

Deb and Ryan Purcell have initiated an **MPS II Research Fund** to fund research into CNS issues relating to MPS II. The Society is administering this fund and facilitating research applications and grant disbursements. On May 5, 2007, the Purcell family held their first fundraiser, *Tacos for Trey*, and raised enough money to fund the second \$20,000.00 instalment of Dr. Murray Potter's 2007 research grant (the Society funded the first instalment of this grant on March 31, 2007). The MPS II Research Fund also contributed \$15,000.00 toward Dr. Clarke's 2008 grant, and \$4,000.00 toward Jason Schwindt's Summer Studentship Research Grant.

Note: The MPS II Research Fund's revenue is reported as its gross revenue (\$45,652) less its expenditures (\$43,289).

Financial Statements for the Year Ended March 31, 2008

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, 2008, 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
June 25, 2008

Balance Sheet as at March 31, 2008 (Unaudited)		
March 31	2008	2007
Assets		
Cash and short term deposits	91,910	86,991
Sales tax receivable	1,733	627
Accrued interest receivable	628	782
Prepaid Expenses	1,500	6,862
Total Current Assets	\$95,771	\$95,262
Liabilities		
Accounts payable	950	850
Deferred Revenue*	-	10,000
Deferred Revenue**	6,395	-
Total Current Liabilities	7,345	10,850
Surplus	88,426	84,412
	\$95,771	\$95,262
*2008 Symposium Sponsorship		
**2008 MPS CUP		

On behalf of the Board of Directors,

Delane Terrillon *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 for families whose children are affected with storage diseases, to bring about more public awareness of lysosomal storage diseases, and to raise funds to further research into storage diseases.

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 \$71,169 which bears interest at 3.55%, semi-annually and matures December 2008.

Note 3. Fundraising

Fundraising revenue is comprised of gross revenue in the amount of \$126,433.25 (2007—\$119,347.74) less expenditures in the amount of \$24,905.57 (2007—\$27,817.10).

Financial Statements for the Year Ended March 31, 2008

For year ended March 31, 2008:	2008	2007
Revenue		
Fundraising, net of related expenses (Note 3)	101,527	91,531
Donations	57,467	57,339
2008 Symposium	10,000	-
Awareness bracelet sales	320	995
Memberships	3,089	3,550
Interest	2,372	2,225
Sales	323	768
Conference Revenue	-	22,204
MPS II Research Fund (see note on page 5)	2,363	700
Miscellaneous Income	2,283	-
Total Revenue	179,744	179,310
Expenditures		
Salaries and contract fees	44,665	37,088
2008 Symposium	19,787	-
Printing, brochures and postage	7,279	7,982
Awareness bracelets	-	754
Telephone and internet	3,205	3,660
Travel	9,135	7,408
Office expense	4,719	3,053
Family assistance donations	4,724	5,495
Computer Software	1,590	11,128
MPS II Fundraising expense	1,405	-
Consulting/Website fees	-	4,178
Non-recoverable GST	1,676	1,813
Insurance	493	1,100
Professional fees	1,050	850
Meetings	2,192	1,728
Conferences	-	39,291
Membership Dues	810	768
Promotional Items	-	2,916
	102,730	129,214
Research grants	73,000	68,000
Total Expenditures	175,730	197,214
Excess/(Deficiency) of revenue over expenditures for the year	4,014	(17,903)
Surplus, beginning of year	84,412	102,315
Surplus, end of year	88,426	84,412

We gratefully acknowledge the following donors:

Many thanks to *everyone* who made donations to the Society during our April 2007–March 2008 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 MPS CUP Fantasy Hockey Game & Gala

Team MPS at the Scotiabank Toronto Waterfront Marathon—Judy Byrne

Genzyme Canada

Shire Human Genetic Therapies

Rock 101/99.3 The Fox

PLATINUM (Donations of \$5,000 +)

AWM Productions*

Steve & Arlene Jungaro

Robert & Judy Hager

John Hardie Mitchell Family Foundation*

GOLD (Donations of \$2,500–\$4,999)

Biomarin Pharmaceutical Inc.

Jacob Heilbron & Heidi Castle

Mike & Mary Ann Nightingale

Hawkeye Gold & Diamond Inc.

Just Singin' Round Foundation

North Shore News

Sutton Place Hotel Vancouver

SILVER (Donations of \$1,000–\$2,499)

Canadian MPS Jeans Day at Cove Cliff Elementary School

The Great Lake Walk—Brooke Hodson

The Rosy Bowl—Jim LeMaitre

Walk with Nature—Mary Nelis and Marcel Dupont

Steve & Lori Bolton

Mrs. Betty Done

McCarthy Tetrault Foundation

Mark & Gill Olesen

United Way Ottawa

Chris Zimmerman & Emily Burch

Orca Bay Sports & Entertainment

TBG The Bicycle Group Inc. (Kona Bicycle Co.)

Ken & Maureen MacDonald

Matcon Excavation & Shoring Ltd.

Gregory & Kelly Wolfe

The Calgary Foundation—The Maxwell Alexander Settari Memorial Fund

North Shore Winter Club

Nancy Johnson*

G.F.H. Enterprise Ltd.*

Rick & Anna Marks

John & Lynda Noble*

Chuck Westgard

Chris & Cindy

Bouchard

Martin Wood*

Bear Mountain Master

Partnership

Renee Chen

Jack Cehak

Linda Jones

Harbour Air Ltd.

Barry Peters*

Ken Bogas

Linda Brown

Foundation For Youth

Little League BC Celebrity Golf Classic

Gayle Purcell*

Robertson Floors

Bernie & Debbie Saywell

Kent Scarborough

Snap Financial

Graphically Speaking

The Dynamics

Vancouver Sharp Imaging

BRONZE (Donations of \$500–\$999)

Tanja Balic*

Bill Adams

Dan & Lena Stefan

Heather Cehak*

Shelly Bryant

Joan & Andy Byrne

Mercer Human Resource Consulting

Todd & Kirsten Harkins

Krista Purcell*

United Way of Winnipeg

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Saginaw Golf Course Ltd.

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Level 10 Fitness

Shirley Anderson*

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Business Objects

AnneMarie Felser*



Elodie Meilleur (MPS IV)

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Catherine & Jim Fowler

Kenny Gibbs*

Ginger Pants Production Inc

Mike & Lisa Hudson

Insight Leadership Development Inc.

International Photographers Guild—669*

Frank & Verle Kaweski

Gary & Marina Lewis

Len & Norma Linton

Peter & Ellen Muirhead

Shayne & Hilda Nyquist

Chris & Debbie Oddliefson

Leroy Olson

Prince Arthur Loyal Orange LOD

Clete Purcell*

Self Storage Depot

Diane Smith*

The Nemetz Foundation

Nancy Trott & Ian Hanomansingh

Michael Urban

West Shore Constructors Ltd.

Elsie Wright*

Mex Y Can

Carter Chevrolet-

Oldsmobile-Chevy Truck

BC Hockey Now

Carrie Marshall Photography

Westview Esso

Okanagan Springs

*indicates MPS II Research Fund donation

The Canadian MPS Society is a registered not-for-profit organization: Charity # 12903 0409 RR0001

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