2009-2010 Annual Report

The Canadian Society for Mucopolysaccharide & Related Diseases Inc.

Aerodynamically, the dragonfly shouldn't be able to fly...

> "When you care, people notice."
>
> -Susane Berger

Every day, kids with MPS and related diseases are overcoming challenges, working hard to be the best they can be, and soaring to new heights. For some, that means sitting up in a wheelchair or leaning in to deliver a kiss that brings indescribable joy to a parent or sibling; for others, it means getting an A on a math test, receiving an MVP award for athletic achievement, or lacing up ballet slippers for a big recital. Adults with MPS and related diseases are making their families proud by enriching family gatherings, living independently, or by passing bar exams. There are no limits on the human spirit and those with MPS display over and over again that they will not be contained by any arbitrary restrictions. In order to live their best lives, though, they need the support of many. In the words of Susane Berger, "When you care, people notice." Every donation made to support the Canadian MPS Society is gratefully received and we honour those who have made commitments to those living with MPS and related diseases.

On behalf of the Society's board of directors, medical advisory board, and members, thank you to everyone who has provided wind for those with MPS to fly...

Kirsten Harkins

Judy Fowler-Byrne
Chairperson

Kirsten Harkins
Executive Director

Inside the Annual Report:

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Left: Trey Purcell (MPS II) receives an award for being named his baseball team's MVP; Right: Sarah Byrne (MPS I H) prepares for her dance recital

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

2009-2010 Board of Directors and Medical Advisory Board

EXECUTIVE DIRECTOR:

Kirsten Harkins
MPS I parent, BC

BOARD OF DIRECTORS:

Chair—Judy Fowler Byrne
MPS I parent, ON
Vice-Chair—Carrie Nimmo
MPS I aunt, BC
Treasurer—Brenda MacLean
Professional, BC
Secretary—Jean Linden
MPS III parent, BC
Barb Boland
MPS III parent, NL
Bernie Geiss
Professional, BC
Todd Harkins
MPS I parent, BC
Aubrey Hawton
MPS III parent, ON
Simon Ibell
MPS II, ON
Jim LeMaitre
MPS I parent, ON
Randall Linton
MPS III parent, ON
Mary Nelis
MPS I parent, QC
Deb Purcell
MPS II parent, BC

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

MPS
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Registered Charity # 12903 0409 RR0001

Support for families. Research for a cure.

...but the dragonfly doesn't care...

Support for families. Research for a Cure. Those are the mandates of The Canadian Society for Mucopolysaccharide & Related Diseasess Inc. (The Canadian MPS Society) and during our 2009-2010 fiscal year, we strove to do both as well as possible.

SUPPORT FOR FAMILIES:

We continued to support families by providing educational materials, such as our website, quarterly newsletters, and disease-specific booklets, and developed a brand-new MPS II Family Resource Binder, filled with information on MPS II (Hunter syndrome) and helpful resources, fact sheets for medical professionals and teachers, and tips on managing the complex care of an individual affected with this particular disease. We also began the process of researching for the production of an educational video as well as a fundraising video – look for both of these in the year to come. We continued in our efforts to connect families, both with our family referral directory and regional family meetings. A very successful regional meeting was held in Toronto in November of 2009, bringing together members and non-members to learn more about each other, new clinical studies and trials, financial resources available, and the rare disease framework in the Ontario Health Ministry. In BC, meanwhile, parents were invited to attend monthly meetings held by the Rare Disease Foundation, on topics from sleep therapy to recreational therapy. We supported several families with direct financial aid, allowing them to modify their homes, hire respite care, and otherwise cope with the demands of raising children with very unique needs. In December of 2009, we provided a travel bursary so that a family could attend a MPS conference held at Disney World in Florida – a "magical" experience to be sure!

Matthew Santos (MPS IVB), a friend, and Verica Gacic (MPS IV A Mom) visit at the Toronto regional meeting

Emma Hardman (MPS I H) with her American friend, who also has MPS I H, at the National MPS Society's Disney Conference.

The Nelis-Dupont family's 6th "Walk with Nature"

The Society partnered with Genzyme by encouraging its members to participate in the Expression of Hope II art competition.

Expression of Hope is a global program featuring works of art by the community touched by lysosomal storage disorders (LSDs). Our vision is that those who experience this art will be inspired and moved by the powerful expressions of human spirit which the pieces will reveal. View the Expression of Hope II gallery at www.expressionofhope.com.

Jansen Harkins—brother of Nicklas Harkins, who has MPS I H/S—was very excited when his painting entitled "Burning Passion" was selected to be part of the exhibit in Boston and to be included in the Expression of Hope Art note cards, coffee table books, and calendars. These products are all available for sale through the Society's online store.

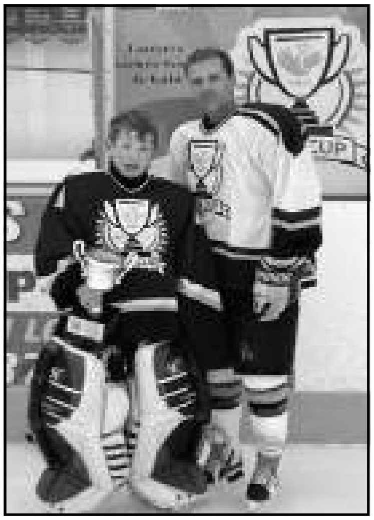
Jansen Harkins with Genzyme CEO Henri Termeer commemorating the launch of the 'Expression of Hope II: Inspiration through Art' exhibit

...so it goes on flying anyway.

In order to provide important services to affected families, the Society raised funds through a number of events and activities and we thank everyone who contributed in any way.

Canadian MPS Jeans Days took place across Canada. Thanks to those who participated and helped us raise funds and awareness.

The 2009 MPS CUP Fantasy Hockey Game and Gala netted over $93,000, and funds raised since the event's inception in 2003 passed the half a million dollar mark—a very exciting accomplishment that wouldn't have been possible without the dedication of a very committed team. The "goal" of the MPS CUP is to "help kids raise their arms in victory over MPS" and we thank the players, committee members, sponsors, donors & countless volunteers for believing in our vision of a brighter future.



Nicklas Harkins (MPS I H/S) and Cliff Ronning at the 2009 MPS CUP

The Canadian MPS Society was the featured charity at April 2010's **JSR (Just Singin' Round)** event at the Vancouver Rowing Club. JSR is a monthly singer-songwriter showcase produced by the Synergy Collective Society. Funds raised through JSR events are divided equally at the end of the year amongst participating charities and charity representatives have a unique opportunity to work together and learn from each other in a supportive and creative environment.

Team MPS participated for the third time in the **Scotiabank Toronto Waterfront Marathon** in September 2009. Several Society members and supporters either ran, walked, or wheeled to raise funds and awareness for the Society and over $4,300 was raised by this energetic group.

Team MPS at the Scotiabank Waterfront Marathon

Our third **Annual Fund** campaign took place in December 2008 and resulted in $5610.00 in revenue.

Time for a Cure, a virtual fundraiser, took place in May 2009 and $13,972 (net) was raised for our MPS II Research Fund throughout the year.

The Purcell Family promote their Time for a Cure fundraiser

The Society was also very fortunate to be the recipient of funds from various foundations and third-party charitable events, and gratefully received proceeds from the BC Hockey Benevolent Association (The Vancouver Canucks Alumni), the Little League BC Celebrity Golf Classic, and Friends of Ferrari, to name a few.

RESEARCH FOR A CURE:

Nicklas and Kirsten Harkins gratefully accept a cheque for $3000 from Friends of Ferrari

Due to the generosity of our many caring donors and sponsors, two new exciting research projects were funded at the end of our 2009-2010 fiscal year, and a second installment was paid out for a two-year grant initiated in the 2008-2009 fiscal year. We offer our heartfelt thanks to everyone who is funding these critical projects through their support of the Society and we pledge to continue to take the stewardship of our donors' funds very seriously. We are proud of our "grassroots" efforts!

MPS II AAV Gene Therapy Research
Principal investigator: Dr. Joseph Muenzer
Institution: The University of North Carolina at Chapel Hill
Funds allocated (from the MPS II Research Fund): $50,000.00

Thanks for helping our children soar!

Comprehensive study of pathogenic glycosaminoglycan storage in a murine model of MPS I
Principal Investigator: Dr. Lorne Clarke
Institution: University of British Columbia, Vancouver, BC
Funds allocated: $100,000.00 (two year grant: $50,000.00 per year for two years—funded first installment in 2009/2010)

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: $100,000.00 (two year grant: $50,000.00 per year for two years—funded second installment in 2009/2010)

In addition to the research grants the Society funded on its own in 2009-2010, and in an effort to engage the federal government in funding research into lysosomal storage disorders, we initiated a partnership with the Canadian Institute of Health Research (CIHR) to fund a **2010 CIHR SHOPP (Small Health Organization Program Partnership) Grant.** The Canadian MPS Society and CIHR announced their intention to jointly fund a 3-year $135,000.00 fellowship for research into Lysosomal Disease Research.


David Mackay (Mannosidosis) enjoys a smore while camping

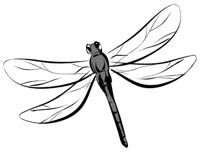
Since we recognize that all the research in the world means nothing if the resulting treatments don't reach the patients who need them, we continued to focus efforts on educating government officials on the need for federal orphan drug policy and on the reasons why funding treatments for those with MPS and related diseases is the right thing to do. We continued to work with the Canadian Organization for Rare Disorders (CORD) to this end by taking part in their *2nd Action Day on the Hill*, which took place in Ottawa April 30th, 2009, followed on May 1 & 2 by the *N of "1": Working Together to Meet the Challenge of Rare Disorders* conference.

We also had the unique opportunity of having one of our members publicly thank the Executive Officer of Ontario's Drugs for Rare Diseases program for not only developing a rare disease framework, but for funding enzyme replacement therapy for MPS II as the program's pilot project.

The Society continues to collaborate with Canadian rare disease groups (in addition to our work with CORD, we also work closely with the newly-formed Rare Disease Foundation), and also has strong ties with North American groups (like the Lysosomal Disease Network's Council of Patient Advocates, or COPA), and international groups (including the International MPS Network). The Society took part in the International MPS Network's annual meeting in Orlando in December 2009 and also participated in *The International Joint Symposia on Rare Diseases in Taiwan: 12th Annual Asia LSD Symposium; 2nd APAC Educational Forum for Families and Caregivers on MPS; & International Conference of National Policies and Actions on Rare Diseases*, hosted by the Taiwan MPS Society in Taipei, Taiwan in October 2009. The Society's executive director, Kirsten Harkins, was asked to sit on the family program planning committee for the 2010 symposium in Adelaide, Australia, and the Society joined the international community to celebrate International MPS Awareness Day on May 15th, 2009 by emailing e-cards to members, medical advisory board members, and other Society supporters, in order to remember those affected with, or by, MPS or a related disease.


Anisa Elder (MPS I H/S)

Together as a community, we can help children with MPS and related diseases fly higher than imaginable. In the words of Maya Angelou, *"Nobody, but nobody, can make it out here alone."*




Members of the International MPS Network

Treatment on the horizon for those with MPS IVA:

BioMarin Pharmaceutical Inc. announced in April 2009 that it was initiating a phase I/II clinical trial for an enzyme replacement therapy (ERT) for the treatment of MPS IVA (Morquio A syndrome). The trial began in the UK in 2009 with the phase III trial beginning in 2010. Also, a cross-sectional natural history study (MorCAP) began in 2009 in several centres, including Montreal Children's Hospital. If approved, this ERT would add to the ERT treatments available currently for MPS I, MPS II, and MPS VI.

Financial Statements for the Year Ended March 31, 2010

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, 2010 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 20, 2010

Balance Sheet as at March 31, 2010
(Unaudited)

March 31	2010	2009
Assets		
Cash and short term deposits	120,276	207,096
Sales tax receivable	423	434
Accrued interest receivable	826	792
Prepaid Expenses	11,910	1,500
Total Current Assets	133,435	209,822
Liabilities		
Accounts payable	1,000	1,100
Total Current Liabilities	1,000	1,100
Surplus (Deficit)	132,435	208,722
	133,435	209,822

On behalf of the Board of Directors,

Brenda Maclean
Treasurer

Judy Fowler Byrne
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 $133,231 (2009–$213,323) less expenditures in the amount of $22,150 (2008–$25,172).

Financial Statements for the Year Ended March 31, 2010

For year ended March 31, 2010:	2010	2009
Revenue		
Fundraising, net of related expenses (Note 3)	111,081	188,151
Donations	56,248	53,526
MPS II Research Fund	13,972	59,144
Memberships	3,630	3,554
Interest	2,750	2,691
Sales	1,197	2,597
Miscellaneous Income	890	3,111
2008 Symposium	-	97,473
Conference Revenue	-	250
Total Revenue	189,768	410,497
Expenditures		
Salaries and contract fees	73,428	66,610
Travel	7,534	4,327
Printing, brochures and postage	6,498	7,887
Office lease	5,655	4,000
Office expense	4,747	4,500
Family assistance donations	4,587	7,886
Telephone and internet	2,995	3,602
Consulting/Website fees	2,870	-
Computer Software	1,750	1,650
Non-recoverable GST	1,225	1,070
Meetings	1,222	3,200
Professional fees	1,175	1,250
Insurance	1,001	1,057
Membership Dues	720	855
Promotional Items	413	2,253
Office equipment	235	2,439
2008 Symposium	-	19,685
	116,055	132,271
Research grants	150,000	157,930
Total Expenditures	266,055	290,201
Excess/(Deficiency) of revenue over expenditures for the year	(76,287)	120,296
Surplus, beginning of year	208,722	88,426
Surplus, end of year	132,435	208,722

Our donors are helping kids with MPS reach new heights!

Many thanks to *everyone* who made contributions to the Society during our April 2009—March 2010 fiscal year. Special thanks to the following major sponsors and donors, as well as the organizers of major fundraisers:

DIAMOND (Donations of $10,000 +)

Shire Human Genetic Therapies (Canada) Inc.
Genzyme Canada Inc
Ivan & Shelley Harmatny
The MPS CUP Fantasy Hockey Game & Gala—The Harkins Family
Time for a Cure*—The Purcell Family

PLATINUM (Donations of $5,000 +)

Biomarin Pharmaceutical Inc. (Canada)
CC Bouchard Ventures Inc.
Ledcor Construction Limited
The Sutton Place Hotel Vancouver
The North Shore News

Shawn Pyl (MPS III)

GOLD (Donations of $2,500–$4,999)

Black Gold Lodge
Betty Done
R. Chen (Friends of Ferrari)
Linda Jones
Peter & Ellen Muirhead
Bill Adams
Team MPS at the Scotiabank Toronto Waterfront Marathon
—The Byrne Family

SILVER (Donations of $1,000–$2,499)

Kent Scarborough & Krista Nickel
TBG The Bicycle Group Inc. (Kona Bicycle Co.)
Synergy Foundation
BC Hockey Benevolent Association
McCarthy Tetrault Foundation
Mondiale Developments (Friends of Ferrari)
North Shore Winter Club
The Destination Slope & Surf Outfitters
Ryan & Deb Purcell*
Robert & Shirley Young
Kaitlyn Bogas
Bear Mountain Master Partnership
The Maxwell Alexander Settari Memorial Fund at The Calgary Foundation
Grenville Pinto
NHLPA (NHL Players' Association)
Shelly Bryant
Lisa & John Stout
Paul DiPasquale (Friends of Ferrari)
Hans Holst*
Leone International Marketing Inc. (Friends of Ferrari)
Robertson Floors
Matcon Excavation & Shoring Ltd.
Little League BC Celebrity Golf Classic
MDE Mechanical (Friends of Ferrari)
Enterprise Holdings Foundation
Ted Newell & Associates (Friends of Ferrari)
David Wills & The Dynamics
Lyle S. Grant Digitially Assisted Design
Vancouver Sharp Imaging
Dearmore Construction Services (Friends of Ferrari)
Canadian MPS Jeans Day at Cove Cliff Elementary
—The Harkins family
Rosy Bowl Volleyball Tournament
—Jim LeMaitre
A Night of Enchantment
—Lara & Rudy Pietrolungo
Spaghetti Supper—The Nelis Family

BRONZE (Donations of $500–$999)

Talee Lumber
Canucks Sports & Entertainment
John Henry Bikes
Rob Thomson & Marianne Stowe-Thomson
PPI Financial Group
Gary & Marina Lewis
Sharon Wyse-Boileau
Gil & Jessica Rosenfeld
Tubac Golf Resort & Spa
Frank & Verle Kaweski
Destination Ski Rentals Ltd.
Kirsten & Todd Harkins
Robert & Lori Di Ilio
Brenda Deseure-Casteels
Nancy Trott & Ian Hanoomansingh

Nathaniel Phaneuf (MPS II)

Harbour Air Seaplanes
Gayle Purcell*
Lori Ann Bradley*
James & Leslie Carter
Jill & Gary Ley
Linda & Graham Brown
Brendan Morrison
Steve & Laura Fryer
Shirley Anderson*
Robert & Judith Hager
Ayasha Valji & Salim Hirji-Lalani*
Ross & Diane Smith*
Arlene Stiles
Carrie Marshall Photography
JC Studio
Sheraton Vancouver Wall Centre Hotel
Broadway Moving Ltd.*
Core Electric Services Ltd.
Vancouver Minor Baseball*
Canadian Building Materials
Bell, Temple
Corintia Developments Limited*
Signature Stone & Tile Inc.
Rotary Women's Association
North Shore Credit Union*
BC Hockey Now
Mex-Y-Can Flowers
Classic Rock 101
The Great Lake Walk
—Brooke & Wilma Hodson
Walk with Nature
—The Nelis-DuPont Family
Inspire: A Benefit Concert for MPS
—Meaghan Grant

**indicates MPS II Research Fund Donation*