2005 – 2006 Annual Report

The Canadian Society for Mucopolysaccharide & Related Diseases Inc.

Believing in a Brighter Future

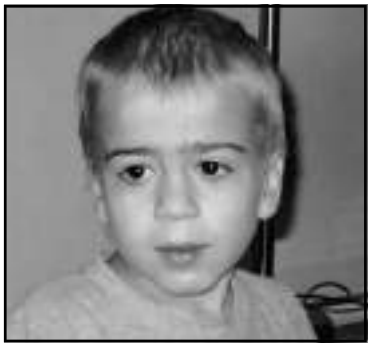
"Never doubt that a group of thoughtful, committed citizens can change the world. Indeed, it is the only thing that ever has."

—Margaret Mead

Margaret Mead's inspirational quote was a favourite of ours during 2005 - 2006: a year in which the Canadian MPS Society did its best to change the world in which Canadians affected with MPS and related diseases reside. Our collective work was driven by our firm belief in a brighter future for all Canadians affected with MPS and related diseases, and has led to triumphs—both large and small—and has increased the quality of life for our members. We are proud of our Society's accomplishments and, on behalf of the Board of Directors of The Canadian Society for Mucopolysaccharide & Related Diseases Inc., we sincerely thank you for your continued support.

Judy Fowler Byrne
Chairperson

Kirsten Harkins
Executive Director



Inside the Annual Report:

From top to bottom: Emma Hardman (MPS I); Jonathan Chayer (MPS III); Members of the Canadian MPS Society rallying outside the October 2005 Federal/Provincial/Territorial Health Ministers' Meeting in Toronto

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.

The Canadian MPS Society is a registered not-for-profit organization: Charity # 12903 0409 RR0001
PO Box 30034, RPO Parkgate, North Vancouver, BC V7H 2Y8
(604) 924-5130/1-800-667-1846 www.mpssociety.ca

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.

LYSOSOMAL STORAGE DISORDERS:

Mucopolysaccharide Storage Diseases:
MPS I – H (HURLER SYNDROME)
MPS I – HS (HURLER-SCHEIE SYNDROME)
MPS I – S (SCHEIE SYNDROME)
MPS II (HUNTER SYNDROME)
MPS IIIA, IIIB, IIIC & IIID (SANFILIPPO SYNDROME)
MPS IVA & IVB (MORQUIO SYNDROME)
MPS VI (MAROTEAUX-LAMY SYNDROME)
MPS VII (SLY SYNDROME)
MPS IX (HYALURONIDASE DEFICIENCY)

Complex Carbohydrate Storage Disorders:
MUCOLIPIDOSES:
- ML I (SIALIDOSIS)
- ML II (I CELL DISEASE)
- ML III (PSEUDO-HURLER POLYDYSTROPHY)
- ML IV (BERMAN SYNDROME)

OLIGOSACCHARIDOSES:
- MANNOSIDOSIS A & B
- FUCOSIDOSIS
- ASPARTYLGLYCOSAMINURIA
- MULTIPLE SULPHATASE DEFICIENCY

SCHINDLER DISEASE
SIALURIA
SIALIC ACID STORAGE
GALACTOSIALIDOSIS
COBALAMIN F MUTATION
GSD II (POMPE DISEASE)

Complex Lipid Storage Disorders:
GLYCOSPHINGOLIPIDOSES:
- GM I GANGLIOSIDOSIS (LANDING'S DISEASE)
- GM 2 GANGLIOSIDOSIS (TAY-SACHS & SANHOFF'S DISEASES)
- FABRY DISEASE (TRIHEXOSYLCERAMIDOSIS)
- GAUCHER DISEASE (GLUCOSYLCERAMIDOSIS)
- NIEMANN-PICK DISEASE A, B & C (SPHINGOMYELINOSIS)
- METACHROMATIC LEUKODYSTROPHY (MLD SULFATIDOSIS)
- KRABBE SYNDROME (GALACTOSYLCERAMIDOSIS)
- FARBER'S DISEASE (LIPOGRANULOMATOSIS)

WOLMAN'S DISEASE
FUCOSIDOSIS
SCHINDLER DISEASE
MULTIPLE SULFATASE DEFICIENCY
SPHINGOLIPID ACTIVATOR DEFICIENCY
CHOLESTEROL ESTER STORAGE DISEASE
GMZ ACTIVATOR DEFICIENCY
CYSTINOSIS

Creating Brighter Futures Through Support and Education

SUPPORT:

The generous financial support of our committed membership, sponsors and donors made it possible for us to continue and further develop our family support programs over the past fiscal year.

- Our **website** was re-launched with a colourful new look in the summer of 2006.
- Our **quarterly newsletter, *the Connection***, continued its role as a valuable resource for our members, helping them stay connected with each other and up-to-date on MPS news and events.
- Our **family referral directory** remained an important link for families wishing to connect with others with the same MPS syndrome or those living in the same geographic region.
- Our **Family Assistance Program** provided almost $2,500 in financial aid to member families to help alleviate the financial burdens often associated with treating and caring for an MPS child.
- **Advocacy support** has continued to be an important element of the Society's work: In July 2005, the Common Drug Review recommended that the provinces and territories NOT fund enzyme replacement therapies (ERT) for MPS I. The Canadian MPS Society rejected that recommendation and in October of 2005, Canadian MPS Society members rallied outside the Federal/Territorial/Provincial Health Ministers' meeting in Toronto to encourage the Ministers to commit to funding ERT for patients with MPS I and Fabry Disease. At the end of the meeting, the Ministers committed to funding both treatments, and although a solution was not in place by the end of this fiscal year, hopefully we will be able to report on the fulfillment of that commitment in next year's Annual Report . A more comprehensive plan for integrating treatments for rare disorders must be implemented in Canada so that rallies like the one held in Toronto won't be necessary each time a new treatment is approved. While Naglazyme, an ERT for MPS VI was approved by the FDA during 2005-2006, it has yet to be approved by Health Canada.

  Of course, our advocacy efforts do not centre entirely around treatment access, but include helping to ensure all our members receive the medical care and services necessary to provide the best quality of life for them and their families.
- Our **bereavement program** continued to provide a series of booklets to bereaved families as they moved through the various stages of grief associated with the devastating death of a child to MPS or a related disorder.

EDUCATION:

The society continues to supply families and professionals with educational resources and society-published booklets relating to MPS and related diseases, and strives to educate the public about MPS and related diseases and important issues relating to them.

- New **posters and brochures** were published in the spring of 2005.
- Together with the National MPS Society (US), we published the booklet **Daily Living with MPS and Related Diseases,** a resource filled with every-day tips from parents for parents.
- We issued a number of **press releases** in the past year, informing the public of important news surrounding MPS. In May of 2005, we announced the date of the 2005 MPS CUP Fantasy Hockey Game and Gala, and in June we were excited to announce that we had raised over $56,000.00 from our third annual event.

  In July, we issued a press release to reject the Common Drug Review's recommendation that the provinces and territories NOT fund ERT for MPS I, and in October we announced the Health Ministers' commitment to fund enzyme replacement therapies for MPS I and Fabry Diseases, and to educate the public about the dire need for an Orphan Drug Policy to deal with integrating rare disease treatments into the Canadian health-care system.
- **Canadian MPS Jeans Days** were held across Canada on our national date, October 14, and on other dates as well, to raise awareness of MPS and raise funds for the Society.
- Along with members from world-wide MPS societies, we celebrated the first **International MPS Awareness Day** on February 25th, 2006—a day to recognize, remember, and honour those whose lives are touched by MPS or a related disease
- **Canadian MPS Society Awareness bracelets** were launched in May 2005, with the inscription **"BELIEVE"** and the Society's website address. Over 2,500 bracelets were sold by March 31, 2006—that's a lot of awareness!

Damien and Natasha (MPS IV) & family
Photo credit: Evan Seal

Fundraising and Research—Hope for a Brighter Future

FUNDRAISING:

Due to the enthusiastic support of several of our member families, many local fundraisers—including golf Pro-Ams, nature walks, volleyball tournaments, and other innovative events–raised thousands of dollars to help fund the society's initiatives.

- New materials were developed for **Canadian MPS Jeans Day** and events were held across the country on the national date and others.
- The third annual **2005 MPS CUP Fantasy Hockey Game and Gala** was another huge success and netted over $56,000 for the society.
- The Canadian MPS Society was the featured charity at February 2006's **JSR (Just Singin' Round)** singer-songwriter showcase at the Vancouver Rowing Club. JSR is a community-based foundation committed to blending art with social responsibility, and we were proud to be one of twelve charities selected to collaborate with JSR in 2006.
- An **Annual Fund** was implemented in December 2005 and resulted in over $4,500 in revenue for the society.

Altogether the society netted over $71,000 in fundraising revenue during the 2005-2006 fiscal year. Thank you for your support.

2005-2006 RESEARCH GRANTS:

Evaluation of Heparin Cofactor II–Thrombin Complex as a Biomarker of MPS I:
Researcher: Dr. Lorne Clarke
Research conducted at: University of British Columbia, Vancouver, BC
Funds allocated, March 2006: $40,000.00 CAD

Research summary:

The mucopolysaccharidoses (MPSs) represent a group of complex progressive multi-system diseases which are caused by metabolic defects in the degradation of glycosaminoglycans (GAGs). The recent introduction of enzyme replacement (ERT) regimes for MPS I and MPS II has brought to light the importance of developing objective methods to evaluate patients. The clinical heterogeneity; manifest as variable age of onset, as well as variable rates of disease, clearly complicate the ability of physicians to accurately prognosticate clinical course for individual patients and leads to significant difficulty in objectively evaluating the effectiveness of treatment regimes. The development of biomarkers that reflect disease severity, disease progression and responsiveness to treatment regimes will be an invaluable tool. Dr. Clarke's laboratory has identified a potential serum biomarker for MPS I and is critically evaluating the efficacy and usefulness of this biomarker in MPS I patients.

Summer Studentship Research Grants:
Due to a lack of high-quality applications, we did not allocate any funding in 2005/2006 for Summer Studentship Research Grants.

Lysosomal Storage Disease Research Consortium (LSDRC):

The following research was funded in 2006, although the Canadian MPS Society's $10,000 USD ($13,302) contribution was made to the LSDRC by in 2004:

Eain M. Cornford, PhD, Professor of Neurology
David Geffen School of Medicine at UCLA, Veterans Affairs Greater Los Angeles Healthcare System
"Gene delivery across the blood-brain barrier in Lafora knockout mice"

Philip E. Dawson, PhD, Assoc. Professor, Cell Biology
Scripps Research Institute, LaJolla, CA
"Potential of chemical chaperones and thioester reactive small molecules as potential therapeutic approaches in the treatment of infantile Batten disease"

Kostantin Dobrenis, PhD, Asst. Professor, Neuroscience, Co-Director, Center for Disorders of Lysosomal Metabolism

Albert Einstein College of Medicine
"GM2 Gangliosidosis Therapy Using Neuronotropic Enzyme"

Angela Gritti, PhD,
Institute for Stem Cell Research (SCRI), Milan, Italy

"Neural Stem Cell Based Therapy for GM2 Gangliosidosis"

Synthia H. Mellon, PhD, Prof, Ob/Gyn. Reproductive Sciences
Univ. of California at San Francisco
"Neurosteroid Therapy for Lysosomal Storage Disorders"

Thomas N Seyfried, PhD, Prof. of Biology
Boston College
"Evaluate MJ-DGJ as a substrate reduction therapy, neural stem cells (NSCs), as a cross-correctional therapy, and caloric restriction (CR) as an anti-inflammatory therapy for ganglioside storage diseases"

Brian W. Soper, PhD, Research Staff Scientist
The Jackson Laboratory
"MPS VII CNS Gene Therapy Using Neuronal Stem Cells"

Partnerships—Working Together for a Brighter Future

PARTNERSHIPS:

Throughout 2005-2006, the Canadian MPS Society continued to develop its working relationships with allied national and international organizations and networks to further its mission. In addition to the LSDRC, the Canadian MPS Society worked with:

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 orphan drug policy implemented in Canada.

GOLD (Global Organization for Rare Diseases) –
We are pleased to be members of this new 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.

The International MPS Network –
A meeting of the International MPS Network was held in Norway in May 2005 to discuss issues of common concern. It was during this meeting that the societies involved decided to implement **"International MPS Awareness Day,"** the first of which was held February 25, 2006.

The Canadian Fabry Association and The Canadian Gaucher Society –
The Canadian MPS Society works closely with these societies toward our common goals, particularly those surrounding access to available treatments.

Expression of Hope

Many of our members took part in **"Expression of Hope,"** an international art exhibit sponsored by Genzyme Corporation in partnership with international MPS societies in order to showcase the creative expression of those whose lives are affected by lysosomal storage diseases. Nicklas Harkins's painting of a dragon was chosen as one of the featured pieces in the traveling exhibit—well done, Nicklas! All submitted pieces of art can be viewed at www.expressionofhope.com.

Nicklas Harkins with Genzyme Corporation's CEO Henri Termeer at the Expression of Hope Art Exhibit.

From top: Nathan Linden (MPS III) & friends at Ron Brent Elementary's Candian MPS Jeans Day; Matt Cooke, Todd & Nicklas Harkins & Brent Sopel at the 2005 MPS CUP; Simon Ibell (MPS II) and Sarah Byrne (MPS I) at the Sarah Byrne Charity Pro-Am for MPS Kids.

Financial Statements for the Year Ended March 31, 2006

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, 2006, 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 12, 2006

Balance Sheet
(Unaudited)

March 31	**2006**	**2005**
Assets		
Cash and short term deposits	**94,095**	**92,551**
Sales tax receivable	**289**	**419**
Accrued interest receivable	**481**	**600**
Prepaid Expenses	**8,200**	**—**
Total Current Assets	**$103,065**	**$93,571**
Liabilities		
Accounts payable	**750**	**500**
Total Current Liabilities	**750**	**500**
Surplus	**102,315**	**93,071**
	$103,065	**$93,571**

On behalf of the Board of Directors,

Delane Terrillon

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 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 only capital assets of the Society consist of office equipment.

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 totalling $71,169 which bears interest at 2.70%, semi-annually and matures December 2007.

Note 3. Fundraising

Fundraising revenue is comprised of gross revenue in the amount of $92,488.34 (2005 - $60,947) less expenditures in the amount of $21,275.96 (2005 - $10,389).

Financial Statements for the Year Ended March 31, 2006

For year ending March 31	2006	2005
Revenue		
Fundraising, net of related expenses (Note 3)	71,212	50,558
Donations	34,144	69,699
Awareness bracelet sales	5,064	—
Subscriptions	3,604	2,727
Interest	1,828	1,353
Sundry	504	624
Conferences	—	1,430
Total Revenue	116,356	126,391
Expenditures		
Salaries and contract fees	34,847	34,197
Printing and postage	10,051	6,280
Awareness bracelets	3,818	—
Telephone and internet	3,335	6,795
Travel	3,166	2,708
Office expense	2,688	2,630
Family assistance donations	2,392	2,885
Office equipment	1,676	—
Consulting fees	1,650	2,197
Non-recoverable GST	1,287	1,477
Insurance	1,100	1,100
Professional fees	750	600
Meetings	352	1,391
Conferences	—	20,441
Registration fees	—	30
	67,112	82,730
Research grants	40,000	47,302
Total Expenditures	107,112	130,032
Excess/(Deficiency) of revenue over expenditures for the year	9,244	(3,641)
Surplus, beginning of year	93,071	96,712
Surplus, end of year	102,315	93,071

Thank You for Believing in a Brighter Future

We sincerely thank *all* donors who contributed toward a brighter future for Canadians with MPS & related diseases during our April 2005—March 2006 fiscal year, and we graciously acknowledge the following major sponsors and donors, as well as the organizers of major fundraisers:

DIAMOND (Donations of 50,000 and higher)
The MPS CUP Fantasy Hockey Game and Gala

PLATINUM (Donations of $5,000 and higher)
Genzyme Canada

GOLD (Donations of $3,000—$4,999)
Sarah Byrne Charity Pro—Am for MPS Kids—Byrne Family

SILVER (Donations of $1,000—$2,999)
Anonymous
Walk with Nature for MPS Kids—Mary Nelis
Rosy Bowl—Jim LeMaitre
BioMarin Pharmaceutical
Betty and Barry Done
Paul and Jaina Eviston
Chelsea Durant Trust Fund
Gerry and Beverley Berkhold
Bow Valley Energy Ltd.
North Shore Winter Club Pee Wee B1 Team
Canadian MPS Jeans Day at Pineview Public School—Heather Kells
Foundation for Youth
Robertson Floors Ltd.
Jane Gunton
The Calgary Foundation—The Maxwell Alexander Settari Memorial Fund
Transkaryotic Therapies Canada Inc.
North Shore News
Sutton Place Hotel, Vancouver
Harmony Airways
Classic Rock 101/Mojo Sports Radio
Carter Chevrolet
North Shore Winter Club
Vancouver Sharp Imaging
Mills Basics Printing & Stationery Co. Ltd.

BRONZE (Donations of $500—$999)
Anonymous
Canadian MPS Jeans Day at Cove Cliff Elementary School
Canadian MPS Jeans Day at Delta Bingo Downsview—Tammy Organ
The Great Lake Walk—Brooke Hodson
West Shore Constructors
Leone
The Destination Slope and Surf Outfitters
Trades Labour Corporation
Rick and Anna Marks
LeRoy Olson
The Nemetz Foundation
Mike and Lisa Hudson
Pacific Towing
Graham Rubber Company
United Way of Ottawa
United Way of Lower Mainland
United Way of Winnipeg
United Way of Greater Toronto
The Electromac Group
Matt and Michelle Cooke
Mercer Human Resource Consulting
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BC Hockey Now
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Molson Canadian
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