

# 2014 annual report



The Canadian Society for  
Mucopolysaccharide &  
Related Diseases Inc.



## Inside the Annual Report

The Canadian MPS Society's  
Vision, Mission and Values,  
Board of Directors &  
Medical Advisory Board

Support for families.

Research for a Cure.

Celebrating 30 years: a tribute  
to our founder Sheila Lee

Financial Statements

Major Donors: we owe our three  
decades of success to you!



Top: Maya Bosdet (MPS I) with Cammi  
Granato at the 2013 MPS CUP; Bottom:  
Matteo Spina (MPS I) celebrating MPS Day

# THE CANADIAN MPS SOCIETY: CELEBRATING 30 YEARS

## Our Vision:

We believe in a brighter future for those affected with and by Mucopolysaccharide (MPS) and related lysosomal diseases. We envision a future where patients no longer die from MPS and related diseases because treatments have been developed, cures have been discovered, and our government has made these treatments and cures accessible to those who need and deserve them. In the future, we envision those affected are living long, healthy, and fulfilling lives — enjoying the company of their friends, children, and grandchildren.

## Our Mission:

Founded in 1984, The Canadian Society for Mucopolysaccharide and Related Diseases Inc. (The Canadian MPS Society) is committed to providing support to individuals and families affected with MPS and related diseases, educating medical professionals and the general public about MPS, and raising funds for research so that one day there will be cures for all types of MPS and related diseases.

## Our Values:

The Canadian MPS Society values:

- community & compassionate support
- health & wellness
- justice & equality
- education & awareness
- research & innovation
- accountability & transparency

## BOARD OF DIRECTORS AND MEDICAL ADVISORY BOARD

### Board of Directors

#### **Chair—Bernie Geiss**

Professional, British Columbia

#### **Vice-Chair—Carrie Nimmo**

MPS I, Aunt, British Columbia

#### **Secretary—Judy Byrne**

MPS I, Parent, Ontario

#### **Treasurer—Brent Nichols**

Professional, British Columbia

#### **Warren Campbell**

Professional, British Columbia

#### **Todd Harkins**

MPS I, Parent, British Columbia

#### **Aubrey Hawton**

MPS III, Parent, Ontario

#### **Terms ended September 22, 2013:**

#### **Jean Linden**

MPS III, Parent, British Columbia

#### **Mary Nelis**

MPS I, Parent, Quebec

#### **Dan Priest**

MPS IVB, Parent, British Columbia

#### **Matthew Santos**

Adult with MPS IVB, Ontario

#### **Term began September 22, 2013:**

#### **Lisa Bosdet**

MPS I, parent, British Columbia

### Medical Advisory Board

#### **Chair—Lorne Clarke**

**M.D., C.M., F.R.C.P.C., F.C.C.M.G.**

University of British Columbia,  
Vancouver, British Columbia

#### **Robin Casey, M.D., M.S.C.**

Alberta Children's Hospital  
Calgary, Alberta

#### **Joe Clarke, M.D., Ph.D.**

Hospital for Sick Children  
Toronto, Ontario

#### **Aneal Khan, M.D.**

Alberta Children's Hospital  
Calgary, Alberta

#### **Eva Mamak, Ph.D.**

**(Neuropsychology)**

Hospital for Sick Children  
Toronto, Ontario

#### **Serge Melancon, M.D.**

Montreal Children's Hospital  
Montreal, Quebec

#### **John Mitchell, M.D.**

Montreal Children's Hospital  
Montreal, Quebec

#### **Cheryl Rockman-Greenberg**

**M.D., C.M., F.R.C.P.**

Winnipeg Children's Hospital  
Winnipeg, Manitoba

#### **Julian Raiman, M.D.**

Hospital for Sick Children  
Toronto, Ontario

#### **Tony Rupar, M.D.**

CPRI

London, Ontario

#### **Sylvia Stockler, M.D.**

BC Children's Hospital

Vancouver, British Columbia

### Staff

#### **Kirsten Harkins, Executive Director**

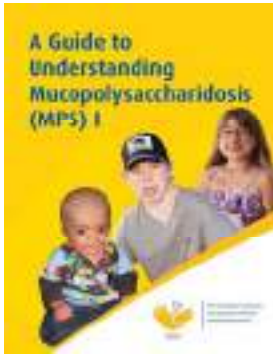
#### **Jill Ley, Executive Assistant**

#### **Karen Saxvik, Executive Assistant**



*The Canadian Society for  
Mucopolysaccharide &  
Related Diseases Inc.*

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Charity # 12903 0409 RR0001



## NEW BOOKLETS PUBLISHED

In December 2013 we published new educational booklets for MPS I, MPS II, MPS III, MPS IV & MPS VI, as well as a new booklet on anesthesia. Thanks so much to the Medical Advisory Board who helped edit the booklets, and to Julie and Craig Taylor and everyone at Renegade Digital Media Inc. for doing the layout and design of our new booklets pro bono (in English and French!). Copies of these booklets are available for download on our website and hard copies can be ordered through our online store. Family Resource Binders for MPS IV were also produced and distributed.

## Help us Join the Search for MPS!

During the summer of 2013, the Society launched a new campaign to raise awareness amongst pediatricians of the early signs and symptoms of MPS. **Join the Search for MPS** brochures were mailed to all pediatricians across Canada, asking them to visit [www.jointhesearch.ca](http://www.jointhesearch.ca) to learn more and “join the search” for MPS. Dr. Lorne Clarke and Dr. Julian Raiman spoke about MPS diagnosis at the Canadian Pediatric Society meeting and a full-page ad ran in the Canadian Pediatric Society’s fall 2013 newsletter. Early diagnosis leads to better patient outcomes. Do you know the early signs and symptoms? Let’s all Join the Search.



## A NEW TREATMENT FOR MPS IV A

Throughout our 2013-2014 fiscal year, several Canadian patients continued their participation in BioMarin’s Phase III enzyme replacement therapy study for the treatment of MPS IV A at three Canadian trial sites.



On December 4, 2013, Vimizim enzyme replacement therapy was submitted to Health Canada for approval and on **February 14, 2014 the US FDA approved Vimizim for the treatment of patients with MPS IV A.** See next year’s Annual Report for news on approval in Canada!

Our sincere gratitude to the Principal Investigators at the Canadian sites— Julian Raiman (Hospital for Sick Children, Toronto), John Mitchell (Montreal Children’s Hospital) and Bruno Maranda (Hopital Fleurimont, Sherbrooke) —and their teams for their hard work and commitment to the patients and the trial,. And to the patients who took part, we can’t thank you enough!

### MPS IV A Family Meeting in Toronto

On October 1, 2013, we hosted a MPS IV family meeting in Toronto, Ontario, which featured presentations by the Society’s executive director Kirsten Harkins, Sick Kids’ Dr. Julian Raiman and advocacy expert Ryan Clarke. Thanks to everyone who attended the meeting, including all the affected families (especially the amazing kids—

pictured above are Jackson Milewski, Zane Braun, Stefan Gacic, Kayla Milewski & Christina Markos).

# THE CANADIAN MPS SOCIETY: CELEBRATING 30 YEARS

**With our donors' support, we were able to fund three research grants during our 2013-2014 fiscal year. Thanks to you we have now funded over \$1,000,000 in research!**

## **2013 Summer Studentship Research Grants:**

### **Sleep Problems in Children with Morquio Syndrome (MPS-IV): A Prospective Cohort Study**

- Recipient: Jason Steinmetz
- Institution: University of McGill
- Supervisor: Dr. John Mitchell
- Amount funded: \$4,000

### **Rescue of mitochondrial dysfunction in Mucopolysaccharidosis IVA disease by treatment with an antioxidant combination**

- Recipient: Hsien Huey Tan
- Institution: University of Victoria
- Supervisor: Dr. Patrick Walter
- Amount funded: \$4,000

## **2014-2 Research Grant:**

### **Somatic and CNS gene delivery for the treatment of Hunter syndrome**

- Recipient: Dr. Douglas McCarty
- Institution: Nationwide Children's Hospital
- Amount funded: \$110,000 (over 2 years); funded through our MPS III Research Fund

## **TEN YEARS OF ENZYME REPLACEMENT THERAPY:**



Because of those patients who participated in the clinical trials, the first treatment for an MPS disease was approved by the FDA ten years ago (in 2003)—Aldurazyme for MPS I.

Thanks to the individuals' (and their families') bold and dedicated participation in the clinical trials, it is estimated that over 1,000 individuals with MPS I in 40 countries are now

receiving Aldurazyme. In addition, the MPS I Aldurazyme clinical trials paved the way for future clinical trials for MPS II, MPS III, MPS IV, MPS VI, and MPS VII.

These milestones were cause for celebration and on October 25, 2013 North American participants and their families were honoured at a luncheon during the National MPS Society's annual conference in San Antonio, Texas. Moving speeches were given by Steve Holland, whose three children participated in the Phase III trial, Dr. Lorne Clarke, who was the principal investigator at the largest North American trial site at BC Children's Hospital, and Ryan Dant, who was one of the first people—as a participant in the Phase I trial—to receive enzyme replacement therapy to treat ANY type of MPS.

On behalf of the board and members of the Canadian MPS Society, we salute those who participated in this trial and those who are continuing to sacrifice to create brighter futures for those with MPS.

## **Dave Charlebois wins first HollisWealth Community Leadership Award!**



Canadian MPS Society Executive Director Kirsten Harkins & Board Chair Bernie Geiss receiving a cheque for \$26,000 from Dave Charlebois, HollisWealth Community Leadership Award recipient, & Mark Neale, RVP Western Canada, HollisWealth.

Dave, a HollisWealth financial advisor, was the bone marrow donor for Sarah Byrne (MPS I) and was inspired to apply for this award on our behalf. Our heartfelt appreciation goes out to Dave and everyone at HollisWealth. Funds received allowed us to print our new booklets, translate them to French, and purchase grant-writing software.

# SUPPORT FOR FAMILIES. RESEARCH FOR A CURE.

For 30 years, families have been organizing events, large and small, to benefit the Canadian MPS Society. Every dollar raised makes a difference. Thanks to everyone who organized, participated in, or contributed to any of the many events that took place during our 2013-2014 fiscal year. Here are some of the highlights:



On April 28th, the inaugural **Manulife Financial Cove Fun 5k & Relay** took place in Deep Cove, BC. Over one hundred participants walked and ran through beautiful Deep Cove and raised over \$14,420 (net) for a great cause - to create a better future for kids with MPS. Thanks to all the sponsors, donors, participants, the enthusiastic committee led by Warren Campbell & Bernie and Christie Geiss, and everyone at Cove Financial & Manulife Financial. It was a fantastic turnout for an inaugural event and we look forward to seeing even more people turn out in years to come! Visit [www.covefunrun.ca](http://www.covefunrun.ca) to view more event information and photos.



## Join our virtual community!



Follow us on twitter (@CanMPSSociety)



Like us on facebook (Canadian MPS Society)



Watch our videos on YouTube.

Join the mailing list for our electronic newsletter, *the e-connection*. to keep up-to-date on Canadian MPS Society news throughout the year!



Hockey players young and old(er) joined forces against MPS on May 4, 2013 at the North Shore Winter Club in North Vancouver, BC. Western Hockey League 1<sup>st</sup> and 2<sup>nd</sup> overall draft picks from 2012, Mathew Barzal and Jansen Harkins, laced up with seasoned NHL greats including Tony Tanti and Dave Babych, Olympian Cammi Granato, and many generous souls who paid for the honour of playing with their hockey heroes.



Trey Purcell (MPS II) with Tony Tanti at the **2013 MPS CUP**

Players and others then gathered at the Sutton Place Hotel for the gala in support of the Society. The 2013 MPS CUP Fantasy Hockey Game & Gala raised a net of over \$66,000. Thanks to everyone who joined our team!

# FINANCIAL STATEMENTS FOR YEAR ENDED MARCH 31, 2013

## 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, 2014, and Statement of Revenue, Expenditures and Surplus for the year then ended.

Our review was made in accordance with Canadian 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 03, 2014

## Balance Sheet as at March 31, 2014 (Unaudited)

| March 31                                  | 2014               | 2013               |
|---|--------------------|--------------------|
| <b>Assets</b>                             |                    |                    |
| Current Assets                            |                    |                    |
| Cash and short term deposits              | 124,029            | 168,881            |
| TD Waterhouse Investment                  | 106,145            | 50,031             |
| Sales tax recoverable                     | 552                | 2,406              |
| Accrued interest receivable               | -                  | 404                |
| Prepaid Expenses                          | 5,000              | 2,500              |
| <b>Total Current Assets</b>               | <b>235,726</b>     | <b>224,222</b>     |
| <b>Liabilities</b>                        |                    |                    |
| Current Liabilities                       |                    |                    |
| Accounts Payable &<br>Accrued Liabilities | 1,100              | 1,100              |
| Deferred Revenue-MPS CUP                  | 3,943              | 100                |
| <b>Total Current Liabilities</b>          | <b>5,043</b>       | <b>1,200</b>       |
| <br><b>Surplus/ (Deficit)</b>             | <br><b>230,683</b> | <br><b>223,022</b> |
|   | <b>235,726</b>     | <b>224,222</b>     |

On behalf of the Board of Directors,

*Brent Nichols*  
Treasurer

*Bernie Geiss*  
Chair

## 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.

### Note 3. Fundraising

Fundraising revenue is comprised of gross revenue in the amount of \$125,427 (2013—\$124,768 ) less expenditures in the amount of \$29,724 (2013—\$33,463 ).

### Note 4. Donor Advised Funds

Donor Advised Fund Revenue is comprised of each fund's gross revenue less expenditures (including a 5% administrative fee per fund).

# FINANCIAL STATEMENTS FOR YEAR ENDED MARCH 31, 2014

| For year ended March 31, 2014  | 2014           | 2013            |
|--|----------------|-----------------|
| <b>Revenue</b>   |                |                 |
| Donations  | 135,443        | 125,276         |
| Fundraising, Net of Related Expenses (Note 3)                        | 95,703         | 91,305          |
| Membership Fees  | 4,330          | 3,395           |
| Interest   | 1,537          | 435             |
| Investment   | 1,115          | -               |
| Sales  | 857            | 957             |
| MPS II Research Fund, Net of Related Expenses (Note 4)               | 41             | 9,762           |
| Miscellaneous Income   | 33             | 1,106           |
| Conference Revenue   | -              | 24,250          |
| MPS IV B Research Fund, Net of Related Expenses (Note 4)             | -              | 10,867          |
|  | <hr/>          | <hr/>           |
| <b>Total Revenue</b>   | <b>239,059</b> | <b>267,353</b>  |
| <b>Expenditures</b>  |                |                 |
| Salaries and Contract Fees   | 77,170         | 82,311          |
| Consulting Fees  | 30,915         | 27,232          |
| Printing and Postage   | 12,365         | 10,804          |
| Computer Software & Computer Expense                                 | 10,905         | 2,018           |
| Office Lease   | 8,400          | 7,530           |
| Travel   | 7,343          | 9,972           |
| Non-recoverable GST/HST  | 4,528          | 5,710           |
| Family Assistance Program  | 4,518          | 4,958           |
| Office Expense   | 4,500          | 6,703           |
| Telephone & Internet   | 2,437          | 2,665           |
| Insurance  | 2,219          | 2,244           |
| Meetings   | 1,450          | 986             |
| Professional Fees  | 1,235          | 1,268           |
| Promotional Items  | 541            | 2,553           |
| Membership Dues  | 205            | 130             |
| Conference Expenses  | -              | 21,591          |
| Office Equipment   | -              | 1,012           |
|  | <hr/>          | <hr/>           |
|  | <b>168,731</b> | <b>189,687</b>  |
| Research Grants  | 62,667         | 90,500          |
| <b>Total Expenditures</b>  | <b>231,398</b> | <b>280,187</b>  |
|  | <hr/>          | <hr/>           |
| <b>Excess/(deficiency) of revenue over expenditures for the year</b> | <b>7,661</b>   | <b>(12,834)</b> |
|  | <hr/>          | <hr/>           |
| <b>Surplus, beginning of year</b>                                    | <b>223,022</b> | <b>235,856</b>  |
|  | <hr/>          | <hr/>           |
| <b>Surplus, end of year</b>  | <b>230,683</b> | <b>223,022</b>  |
|  | <hr/>          | <hr/>           |

# THANKS FOR HELPING US CELEBRATE 30 YEARS!

Thank you so much to *everyone* who made contributions to the Society during our April 2013 - March 2014 fiscal year. Special thanks to the following major sponsors and donors, and to the organizers of major fundraisers:

## DIAMOND DONORS:

**(Donations of \$10,000+)**  
Biomarin Pharmaceutical Inc.  
(Canada)  
CTV  
Genzyme Canada Inc. (a Sanofi Company)  
Shire Human Genetic Therapies Inc. (Canada)  
Team Radio  
**The MPS CUP Fantasy Hockey Game & Gala** -The Harkins Family  
**Manulife Financial Cove Fun 5k & Relay for MPS**—The Geiss Family & Cove Financial

## PLATINUM DONORS:

**(Donations of \$5,000+)**  
Bill Adams  
Cove Financial Planning Ltd.  
Ledcor Group  
Kent Scarborough & Krista Nickel

## GOLD DONORS:

**(Donations of \$2,500 - \$4,999)**  
Air Canada Foundation  
Heidi Castle  
Mike & Lisa Hudson  
McCarthy Tetrault Foundation  
Charles Scarborough  
The Sutton Place Hotel  
Therapure Biopharma Inc.

## SILVER DONORS:

**Donations of \$1000 - \$2499)**  
Luciana Alvarez  
BC Hockey Benevolent Foundation  
Kaitlyn Bogas  
Charles Bois  
Chris & Cindy Bouchard  
Canadian Foundation for Physically Disabled Person  
Dundeewealth - DWM Securities Inc.  
GCIC Ltd.  
Judith Hager  
Linda Jones  
Lianne Potter Law Corporation  
Little League BC Celebrity Golf

Classic  
M.O. Productions  
Scott & Sue-Ann MacCara  
Mackenzie Financial Corporation  
Brent Nichols  
North Shore News  
Pacific Coastal Airlines  
Robertson Floors  
Sharon & Mike Skiba  
Speedbolt Printing Solutions  
Vancouver Canucks  
West Shore Constructors Ltd.  
Y.P. Heung Foundation

## BRONZE DONORS:

**(Donations of \$500 - \$999)**  
A Woman's Touch Housecleaning  
Jeffrey & Lisa Bosdet  
Lisa Burym  
Judy Byrne  
Leslie Carter  
CIBC  
Jenny Cleary  
Fairmont Pacific Rim  
Fraser Fellows  
Joe & Tricia Finn  
Olivia Ford  
Golf BC

Todd & Kirsten Harkins  
Hickey Now  
Ina Ervin Personal Real Estate  
Jill Killeen  
Kitchen Culinaire  
Mex Y Can  
Newport Sports Management  
North Shore Winter Club  
Mark & Gill Olesen  
Nic Petan  
Earl & Barbara Phillips  
PPI Partners  
Pro-formance Goalie Schools  
Rotary Women's Association  
Southwest Contracting Ltd.  
The Vancouver Club  
Tin Wis Resort Lodge  
Nancy Trott & Ian  
Hanoomansingh

## MONTHLY GIVING CLUB:

Dan & Sandi Turner  
Terry Warner  
Stephen Boland  
Judy Byrne  
Bernie & Christie Geiss

*\*You can join our monthly-giving club online or by calling us at 1-800-667-1846.*

## The Society's 2013 Annual Fund Campaign—featuring founder Sheila Lee— raised \$8,225. Thanks to everyone who contributed. And thanks, Sheila, for your inspired vision.

In 1984, Sheila Lee's daughter Brandy was diagnosed with MPS I. She and her husband Loren were told that there was no treatment or cure and that Brandy would die before she turned ten. At that time, they also realized that their eldest daughter Rachelle, who had died in 1979, had also suffered from MPS I but was never diagnosed. Shocked, devastated and desperate for information and support, Sheila reached out but found that no literature existed and there was no organization in Canada dedicated to MPS or its related lysosomal storage diseases. Her desperation to speak to another parent of an affected child fueled her search to find other affected families and The Canadian Society for Mucopolysaccharide & Related Diseases Inc. (Canadian MPS Society) was born from a small Northern Manitoba town.

Sadly, Brandy died in 1991. In 1994, Sheila moved the head office of the Canadian MPS Society to Calgary where she remained its Executive Director until 1998. The Society's head office then relocated to Unionville, Ontario under the leadership of Executive Director Lori Di Ilio and in 2004 it relocated once again to North Vancouver where it remains today.

In order to form an organization Sheila had to work diligently to find other Canadian families affected by MPS and related diseases. The goals of the Society were set early on: family support, awareness and research. Children were dying, so the hope of a treatment for Brandy and the other children kept Sheila pushing on.

Little by little, progress was made and the First National MPS Conference was held in Gimli, Manitoba in 1985. A wonderful success, the conference was the first time Canadian families came together to meet other parents and children affected with MPS and to listen to experts in the field.

30 years after the Society's humble beginnings, it has funded over \$100,000 in family assistance grants, over \$1,000,000 in research, and has assisted hundreds of families on their journeys with MPS and related diseases. In 2008, the Society hosted over 1,000 delegates from 37 countries for the 10th International Symposium on MPS & Related Diseases in Vancouver, BC. There are now four approved enzyme replacement therapies for MPS I, II, IV A and VI and the Society has fought hard to ensure they are accessible to patients who need and deserve them. There's still a long way to go, but the future has never been brighter for those affected by MPS & related diseases.