



IMPSN

International MPS Network
Mucopolysaccharide and Related Diseases

IMPSN Newsletter October 2020

We are all living in uncertain times as a result of the Covid 19 pandemic and all of us have been affected in one way or another. However, the MPS and related disease community, which is already one of the most vulnerable patient communities, has been more affected than many. Isolation, long term shielding, treatment breaks, and delays in diagnosis are just some of the issues facing our community and there has never been a greater need for patient organizations to collaborate more closely with each other and other key stakeholders.

Now that formalization of the IMPSN has been completed, we move on to the next phase of our development with great energy and enthusiasm but we also are very aware of how Covid 19 is putting huge pressures on all our resources to breaking point and beyond. We are all having to spend considerable extra time on our own individual country's support of patients and managing all the negative impacts of this pandemic.

In addition, the Board of the IMPSN is considering the impact Covid 19 is having on our priorities and also how it will affect Barcelona 2021 International Symposium. We are in close communication with our Spanish colleagues and, together, we are investigating all possibilities of how the symposium can take place.

Despite these uncertain times, there is always hope and that hope is built on the dedication of not only the scientific community, and the possibility of vaccines and better treatments for Covid 19, but also on the tireless efforts of the patient organizations that we all belong to or work in collaboration with. In these darkest of hours, the patient organizations reach out to offer comfort, advice, support, and sometimes just a listening ear for our patients and their families. We are all working tirelessly to reduce their isolation, anxiety and to help them cope with their extraordinary daily struggles. However, we must not forget that we need to support each other too, both as individuals and as organizations.

This support and collaboration is the very foundation the IMPSN has been built upon and, by coming together and supporting each other, we in turn are more effective in supporting our global community. This pandemic will pass but the long term effects on our community will linger for many years to come. Only together can we rebuild the resilience and wellbeing, globally, of all those we advocate for.

We hope you enjoy this newsletter with updates from our members around the globe!

Our best wishes
Terri and Bob
IMPSN Co-Chairs.

A Heartfelt Farewell

It's with extreme pride and honor, that the IMPSN Board of Directors announce that Jenny Noble is retiring from here long life work in IMPSN and ISMRD. With the power to handle all the adversity in her way with class and kindness, Jenny Noble changed and improved the lives of hundreds of patients and families with MPS and Related Diseases

Thank you jenny for all the work and friendship! Enjoy retirement!

Love.

Board of Directors.

The IMPSN is Formalized

by Kim Angel

The Board of Directors of the International MPS Network is proud to announce that 2 not-for-profit organizations have been incorporated in Canada, effective April 28, 2020.

The International MPS Network (IMPSN) (Business # 73679 8075 RC0001) was incorporated as a federal not for profit to carry out non charitable objectives of the Society.

IMPSN Mission: The International MPS Network exists to be the primary platform for the MPS and Related Diseases. IMPSN is a global community of informed and empowered organizations, who promote access to medicines, new research, advocacy, and programs that lead to a better quality of lives for patients and those affected by these diseases.

The International MPS Organization (IMPSO) (Business Number: 73647 9130 RC0001) was incorporated as a federal not for profit to carry out charitable objectives.

IMPSO Mission: The International MPS Organization is a global community of informed and empowered organizations, who educate about MPS and related diseases, provide support to patients, caregivers, and those affected by these diseases, to raise and direct funds to support the IMPSO's charitable causes.

A business bank account for the IMPSN has been established at the Toronto Dominion Bank of Canada.

We look forward to working with Network members, our industry partners, and key stakeholders to continue to advance the mission of both the IMPSN and IMPSO forward.

COVID -19 Update

United Kingdom
by Bob Stevens

Like many other areas of the world, the UK is experiencing a second wave of Covid 19. There are different approaches to managing the infection rate of the virus including local and regional lockdowns, reduction of social contacts to no more than six and restricted operating hours within the hospitality sector.

Despite these efforts, the virus is once again spreading with increasing speed and transition back into the over 60s is now once again occurring. The healthcare systems are coping but this is getting increasingly difficult and it may

not be long before no Covid procedures are once again impacted. However, according to scientists, there is light at the end of the tunnel. There are many vaccines in development and improved treatment options. The UK is at the forefront of many of the developments including discovering the first effective treatment options and a lead vaccine candidate. Infection rates thankfully have been rare in our MPS community and we hope this trend continues and the MPS Society is working hard to support all of our patient community during these most challenging times.

Australia **by Vanessa Ede-Scott**

Covid -19 was first confirmed in Australia in January 2020. The outbreak has been managed as a health emergency. Over the past 8 months, many parts of the country have been in various stages of lockdown with a business closing and many people losing their jobs. The government introduced income supplements to keep people in work and raised the pension for those seeking employment.

Our MPS/ML families worked closely with their treating clinicians to manage health needs and continuity of ERT treatments. Hospital appointments were managed through separate access and screening areas to reduce infection risk.

One of the biggest challenges was to have children home from school and a reduced workforce of carers and support for families, but to their credit everyone really banded together to support and problem solve. Our Disability sector was challenged but PPE became available quite a quickly and many tools were provided to train the sector on hygiene, physical distancing and use of PPE. eventually mandatory masks were enforced in Victoria.

The MPS Society held some informal Zoom catch ups to help facilitate shared experience and produced an MPS and Covid -19 information sheet for our community.

Where I live in Melbourne we are coming out of our second wave where we experienced a 3 month full lockdown and we have managed to reduce our covid-19 cases down to single digits. Industry and business are looking to resource big projects to reduce employment. We hope some of this funding flows into the health and research sectors.

Canada **by Kim Angel**

The change in social, medical and economic scenarios following the COVID-19 pandemic has significantly affected the members of the rare disease community – patients, family and caregivers. Changes in accessibility and availability of services owing to the COVID-19 pandemic has had a disproportionate effect on the daily lives of patients with Mucopolysaccharide and related disorders (MPS) and their caregivers. While the situation has created a sense of heightened alertness to remain healthy, it has left MPS patients and caregivers with enhanced financial, physical, emotional and mental distress. With heightened hygiene and health safety measures, there is an increased need for personal protective equipment. Although provincial childcare supports are available, there is difficulty in procuring home support due to the pandemic situation. This has demanded excess care from caregivers with either of the parents staying away from work to take care of their child(ren). The resulting financial deficit has further made it difficult to afford essential needs like healthy food and home-schooling resources. Increased health safety measures and increased concern for COVID-19 transmission has posed difficulty in arranging respite care support for caregivers. To ease the burden and assist our member families through this

demanding situation, the Canadian MPS Society has developed the COVID-19 Extended/ Emergency Member Support and Assistance Program.

The objective of the program is to provide resources and emergency financial assistance to our member families to support health safety measures, childcare, support for homeschooling, respite services for caregivers, mental health support for patients and caregivers. This program involves the distribution of health and hygiene self-care packages for patients and caregivers, resources to support learning from home (laptops, tablets, wheelchair accessible desks etc.), gift cards for procuring nutritious food for rare disease patients, accessibility equipment for maintaining physical fitness while at home and development of navigating through COVID-19 guide for MPS patients and caregivers, education materials.

The Canadian MPS Society is committed to continuing our efforts to ensure our MPS and related LSD disorder families are supported during these unprecedented times.

United States by Terri Klein

United States

The United States continues to struggle with the second wave of the COVID-19 outbreak. One of the largest challenges is that each city within each state is developing protocols best suited for its geographical conditions. Federal messaging remains consistent but has tapered off as we move into the election period in our country.

This week we have recorded more positive cases than we had in the first outbreak, but it is critical to understand that we are testing more patients than we were in the spring. Our pace of testing is sometimes 5:1 than in other countries. We are noting in our country that we are starting to see an increase of attenuated outcomes from the virus. Also, the death toll on the pandemic has decreased significantly. We are at approximately 2.9% of the loss to positive cases reported. No death is acceptable, but we are reassured that physicians and researchers better understand how to treat the virus, and many homes are now stocked with nasal sprays, vitamin A & D, vitamin C, and other over-the-counter proactive measures.

PPE remains elusive in our country. Where I live in the Research Triangle Park, the east coast is fully in PPE mode. Our office is open, and we have the ability to self-isolate in our small office building, and therefore the MPS Society staff is on a need to be at office basis.

Clinical trials have been slightly delayed. Institutions vary in their protocol for MPS clinical trials, but most are continuing with modifications. In general, our MPS patient population is doing well in protecting themselves from the COVID-19. Our social workers and myself are answering many questions to assist patients navigating regular and necessary clinical appointments.

Over 20 clinical trials are taking place in the United States for the COVID-19 testing and we have had the first FDA approval of a therapy. We remain hopeful for the discoveries and clinical trials for a vaccine in the coming months.

The National MPS Society led the rare disease community in response to the Center of Disease Control on the dissemination of a vaccine to our patient community when it becomes available. We received over 200 signatures, and we were successful in assuring MPS patients and other rare diseases will be in

Tier 1, Distribution 2 of a COVID-19 vaccine when it becomes available. The distribution of our comments are included in the final [Framework for Ethical Allocation of the COVID-19 Vaccination](#).

Showing all of us that ADVOCACY matters!

Brazil **by Regina Prospero**

Children with MPS and other rare diseases generally have low immunity, which is why they are more susceptible to contraction of Covid-19, a disease caused by the new coronavirus. To guide patients, families, and their caregivers about the care they should take during this pandemic, the Vidas Raras Institute prepared a simple guideline that provides details of the disease and its symptoms, but also specific care recommendations.

"We received a lot of questions from people with a rare disease and their relatives about how to proceed with the coronavirus - if they can leave the house, go to the hospital, what care should they follow, if they need to wash their hands a lot or wash less, questions like that - that's why we decided to make this booklet ", explains Regina Próspero, vice president of Instituto Vidas Raras and an IMPSN board member.

The guideline's available to download in English, Spanish, and Portuguese here:

<http://www.vidasraras.org.br/site/vidas-raras/noticias/543-orientacoes-gerais-de-cuidados-com-as-pessoas-com-doencas-raras-e-seus-cuidadores-na-pandemia-de-corona-virus>

And to make sure our MPS patients wouldn't stop their treatment, we launched a program that they could do their ERT at home if their physicians believed the kid fit the security criterium to do it. With this program, we decreased the number of patients that contracted the COVID-19 and the number of patients that had their treatment and care on hold.

Charitable Access Programs - A Lifeline !!

by Bob Stevens

Many MPS and related diseases patients and their families, my family included, benefit from the advances in science and receive the ground-breaking treatments and therapies. Not only are we able to have access to these treatments and therapies, but we also are fortunate to have world leading standards of clinical care thanks to the healthcare systems in many of our countries.

Imagine a world where you or a loved one has been diagnosed with an MPS or related condition, knowing there is a treatment or therapy in other parts of the world, but because you live in a certain country, this treatment or therapy is not available. For many families this is a stark reality, a world without hope of treatment and/or little healthcare.

But there is some hope through the Charitable Access Programs. The possibility of gaining access to certain therapies or treatments despite their unavailability through the healthcare system in that particular country.

Two access schemes sponsored by Takeda and Sanofi Genzyme offer a glimmer of hope to a few individuals and their families who meet the treatment criteria. These Charitable Access Programs offer Enzyme Replacement Therapy options for some Lysosomal Storage Diseases including Gaucher, Fabry, Pompe and MPSII. Currently, some of the countries (by no means all) that these treatments are available in on a limited charitable basis are listed below:

Egypt Pakistan Tunisia Palestine Tanzania Botswana

The International MPS Network (IMPSN) is actively involved in the Charitable Access Programs and we are diligently remaining current on other countries where these treatments and therapies will be accessible through the program. The Access Program is about available treatments, having access to basic healthcare, and support through patient organizations. **The goal is to provide a safe and accessible system.** The IMPSN will continue to provide current information and be actively involved in the Charitable Access Programs. Our hope is that more individuals will be commissioned over the coming months and years by additional industries collaborating and finding mechanisms for involvement.

Although these programs are not available for everyone, they demonstrate that it is possible to innovate and give hope. This is a path of true advocacy. A founding principle of all our patient organizations and one we are rightly proud of.

For further information please follow the link below or contact Bob Stevens, Group CEO UK MPS Society at b.stevens@mpsociety.org.uk

For more information visit:

TakedaCAP@directrelief.org.

https://webportalapp.com/sp/login/takeda_cap_application

<https://www.sanofigenzyme.com/en/responsibility/humanitarian-programs/>



One on One with the new IMPSN Board Member

IMPSN believes that together we can achieve improved accessibility of care diagnoses and treatments for the MPS community around the globe, and that information can save lives and help families to have a better quality of life.

With this in mind, we want to share the story of Eda, a mother that gave a kidney to save one of her children, and also a mother of an MPS IVA, who decided to create an organization so she can help not only her own children but others that suffer without the proper care.

We hope her story can inspire others and make all of us feel less lonely in this rare disease world.

1. Hi Eda! How did you become involved with this organization?

I am a mother of four children, our first two children have rare conditions. Our son has an undiagnosed kidney condition and both of his kidneys failed when he was 6 years old. We were fortunate I was his match so I was able to donate one of my kidneys to him. Our daughter was diagnosed with Morquio A (MPS IVA) when she was 3 years old. She is not on ERT due to a lack of funding. She is 10 years old now and wheelchair bound since 2017. Due to the hardships, I experienced with my children I decided to establish a Botswana Organisation for Rare Diseases (BORDIS) in 2015 to serve other families.

2.Can you tell me about the work your organization does and the advocacy or programs you run?

BORDIS is an umbrella organization for all rare diseases in Botswana. Our Vision is working towards having an environment where rare disease patients thrive. Our advocacy involves creating awareness, driving policy changes, ensuring access to quality healthcare and ensuring our patients and families access the support they need for their daily living.

3.What do you think your members would say is the best thing about your organization?

Our patients and their families are most grateful for the support they get from

BORDIS because through our work where possible they are able to achieve things they would not get anywhere else. The diagnosis for some families that had waited for years, getting treatment access to some, facilitating skills development for our healthcare professionals among others.

4. What are your priorities?

My spiritual journey, personal growth, and ensuring my mind is positively tuned to handle anything at any time and yes my kids. I stay up late at night looking into how I can make their lives better now and into the future. Because of my kids, I do not allow myself to crumble and be swallowed by pity.

5. What are the biggest challenges faced?

Living in a country where very little is known about rare diseases, having a healthcare system that does not care much about our patients. The biggest blow when I felt and realized we and our children are treated like second class citizens even inferior to an illegal immigrant.

6. Can you tell us about the people you work with?

I work with different people, patients, their families, health care professionals, people and organizations with an interest in rare diseases. All working in BORDIS volunteer their support as BORDIS has not yet reached a level of paying for this much needed support. I am very much grateful as we are working very hard to reach that level and we are much closer than when we started.

7. You have recently joined the IMPSN Board, what are you hoping to achieve over the next 2 years?

I am looking forward to establishing MPS Africa so that the needs of our patients in the continent are well represented and met. This is very much important because what is known and works in other regions does not always address African issues. Another important thing I will pursue will be to strengthen collaborations among our health professions across the continent and make information sharing easier for the benefit of the patients.

8. What do you enjoy doing outside of work?

I enjoy horse riding and swimming to calm my soul, I love traveling I can't wait for the restrictions to be lifted so I can see places and people. I am an avid reader, anything that makes me a better person and a better mother.

9. Who do you look up to for inspiration?

God, he anchors my soul because most of the time my life seems like I'm standing on a sinking ship.

MPS 2021

16th International Symposium
on MPS and Related Diseases
From 30 July to 1 August, 2021 | Barcelona | Spain

The 16th International Symposium on MPS and Related Diseases will take place from Friday, July 30 till Sunday, August 1, 2021.

Due to the Covid -19 pandemic, the 16th Symposium on MPS and Related Diseases was rescheduled for 2021 in Barcelona, for more information about the symposium, access the site: <http://mps2020.com>

<http://impsnetwork.org/>

