



IMPSN
International MPS Network
Mucopolysaccharide and Related Diseases

INTERNATIONAL MPS NETWORK MEETING

Courtyard by Marriott Belgrade city center, Belgrade, Serbia

Friday July 5th 2019 (Day One)

Attendees

- Argentina: Veronica Alonso
- Austria: Michaela Weigl
- Botswana: Eda Selabato^[L]_[SEP]
- Brazil: Regina Prospero and Amira Awada
- Denmark: Thomas Lange^[L]_[SEP]
- France: Stephane Antolin^[L]_[SEP]
- Germany: Carmen Kunkel and Michaela Giel^[L]_[SEP]
- Greece: Barbara Mamatis^[L]_[SEP]
- Hungary: Monika Demcsik and Zsuzsanna Almassy^[L]_[SEP]
- Ireland: Mary Boushel
- Lithuania: Ramune Sliuozaite
- Netherlands: Hanka Dekker
- Serbia: Dragana Miletic Lajko
- Spain: Ana Maria Mendoza Maestre
- Sweden: Oskar Ahlberg
- Switzerland: Fredi Wiesbauer
- Turkey: Muteber Erodoglu and Tahir Selcuk Kayan
- UK: Bob Stevens^[L]_[SEP]
- USA: Terri Klein and Lisa Todd and^[L]_[SEP]
- MPS Europe: Marija Joldic
- Canada: Kim Angel
- New Zealand: Jenny Noble

On the video call:

-Australia: Vanessa Ede-Scott



Meeting Minutes Apendices: presentations slides

In total 25 representatives from 19 countries, plus MPS Europe participated in person and via video call 3 representatives from 3 countries.

International Network Meeting minutes, San Diego, USA, 2018

Minutes Approved – Motion offered Terri Klein, second Bob Stevens
Ask for full vote – 1 Nay vote, Brazil

Matters Arise from the minutes – This is null

Formalization of the IMPSN proposal - Bob and Terri – opening remarks

Canada Formalization – Kim Angel/Canada Document was sent to everyone through the Newsletter

Operate as an International – Viable with low tax base; economically feasible Does not need to solicit private donations Entity on its own We have had input from Legal counsel from Canada.

Fredi/Switzerland -asked if others presented proposals for the formalization Determined by the Board to vote for Canada.

Bob welcomed and introduced the board of directors. Terri discussed the background of the IMPSN and discussed the process to formalize which was discussed in San Diego and we need to talk today about going forward. Discussed MOU that was agreed upon over many years but has not changed much till San Diego. It was time to form a board and move work forward for accountability. As a look back - no accountability in past with IMPSN - time to make meaningful change happen. Need to be in full understanding - need to move forward It is vital that each country has a voice and determine how we work together. Goals have not changed much - hope that we make impactful changes as we go forward.

Bob - discussed IMPSN priority survey Top - 16- Clinical Trials



priority^[1]2nd - 9 - Public Awareness, NBS, Access for Therapies 3rd -
IMPSN strength and Symposium

Bob - discussed progress that have been done by the Board since last meeting. Work on a way forward - e-newsletter was completed. Ready to start communication with key partners - so they can see what is being accomplished at the network.

MOU has been tweaked - slightly changed but only minor - a good place to begin^[1]2 other main areas - how is the group to be sustainable and communicate. Money is necessary to move forward and it is difficult to obtain funds without structure.

Formalization was reviewed for a business point of view - it was determined by the board to create a formal not for profit in Canada - best legal structure due to constraints in the US.

Bob discussed the vote outcomes from the updated MOU, formalization of IMPSN and formalization of network in Canada - all votes passed by landslide votes.

Kim - discussed reasons why formalization was done in Canada - with tax laws and legal structure it made most sense. We can run the network run internationally and Kim utilized a lawyer in Canada to assist. A document was sent out to the group with the details via email.

All Board members looked at various countries, but Canada was determined to the best option.^[1]Vanessa discussed how the board looked at all options to formalize in their regions.

Bob opened to floor for discussion on formalization.

Netherlands - not against formalization or Canada - what is the plan once formalized? Do we know - what is feasible? Not sure if it's a good idea or not.

Canada - there is an action plan that has been created and



developed that will be presented

Germany - would like to see action plan first before vote of formalization

New Zealand - need one set at a time, first part is MOU and then think about formalized and the board is working on action plans. Need to be prepared to move forward. Agree - we can go through the presentation

US - discussed how board reviewed MOU, updated it, board then determined if formalization was necessary. We agree that the entire network needs a voice. The board has worked significantly on this and are trying to move the work forward. Today is the day to help develop the plan. The vote has already taken to formalize to organization.

UK - the board has worked hard for the proposal and we need to move forward.

Sweden - MOU, location and need to formalization are 3 things. We may not know it all today, but we have a mission and a vision and that is enough to move forward. Believe we have enough and can fill in the detail later. Procedural - the electronic vote - clarified that the vote as already been taken to formalize.

UK - the board has taken time to make recommendations but we need to move forward with the group as a whole.

Brazil - since 2008, this is a very respected group and has seen significant improvement of treatments. Staying together will help other countries that do not have help like Brazil does - agree we need to stay together. We need to think about those that cannot help themselves - we are the strong ones.

Australia agrees with Brazils comments - we are stronger together.

US - the vote has been taken and formalize.

Netherlands - I don't understand the action plans and need to



understand what will be done going forward - without that she is unsure if she will be a part of the group

Switzerland - agrees it's time to move forward. We agreed to move forward as a network - need to move forward.

Sweden - who are current members?

MPS Europe - we have 40 countries in the mailing list- 4 are not active at all. UK - Discussed the process again - Board created structure to move forward.

Business and action plan - call for action forward

SWOT and PEST Analysis presentation

Bob- updated group on SWOT and PEST analysis. Board has worked on a business plan with solid core principles. So now, it is time to build it out.

Actions plan have been identified and Bob presented the items:

1. Operations^[SEP]
2. Treatments^[SEP]
3. Clinical Trials
4. Communication Education, Advocacy and Research
5. International Symposium

Terri - went back to discuss SWOT Analysis. Terri would like focus on weaknesses - need to be focused on lacking representation of countries - language barriers. Need better internal communication. We need a clearer internal path for communication and access to documents. Time is a weakness. Many have lot of duties - but need time management these collaborative efforts. Diversity is a challenge. Website needs to be managed updated. How do we communicate Managing membership expectations/ goals?

These items must be addressed to move forward. Asked the group to really consider these items that need to be overcome.



Opportunities should be acknowledged but focus should be on the Treats to develop a path way forward.

When analyzing a PEST analysis must also be considered which is why the board looked at these items.

The action plans draw us back to the survey completed in San Diego- these action items were based on those priorities. These items are where we are looking to help. We need volunteers and subcommittees to help complete the action items. We want this to be a unified voice - the more help we have the quicker we can get there.

One point Bob mentioned earlier when reviewing the survey - MPS Awareness Day and Education - this is under Public Awareness. These action items were completed by Jenny and Vanessa

Terri pulled up the budget and discussed. This budget was based on past budget for ISMPN meetings. The program budget was built out with charitable levels from pharma partners - buy in/funding from them to fund the organization

Need funds for communication/website and meetings - not looking at any employees. The board has all contributed their expertise to create this budget and newsletter. Kim did a great job on initial communication piece and logo to do first newsletter.

Switzerland - asked how meeting have been funded in the past - US commented that grants were written to fund, all for streamlining costs and being more efficiency with better communication.

Netherlands - still confused on what the network is trying to accomplish and what goals and action steps are.

Terri - went through the Mission and Vision Statements. Discussed the Key achievements in 2018/2019. Bob then presented slide on 2019/2020 objectives

Terri discussed that in January we will be presenting a bidding opportunity for the 2024 Symposium.



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Germany, Carmen- mentioned that the next 2 Symposiums will be in Europe so it may need to be on another continent - Maybe in Asia/Virginia?

Terri - Discussed the need for subcommittees is over to complete the action items - and that help is needed for each item

Netherlands - asking the board to determine simple procedure. The network needs to understand simple procedures - needs education - it used to be for the program Symposium. She would like Board to create specific procedures related to symposium so that people know who is responsible for what.

Terri - agree we need good information.

Switzerland - asked timeline for subcommittees? Terri - timeline for formalization is critical - 3 to 4 months. After that other areas prior to the end of the calendar year.

Anyone can volunteer.

Bob - we have a great starting point and have examples to go on for formalization (MPS Europe as an example)

Bob mentioned to please email Terri for interest in committee work
Terri took hands for those that might be interested in committee work.

Argentina -ask what committee entails

Terri - it will be determined by the committee. Board will be involved in committee work to drive process. Greece - is there a plan to have an adult/patient committee? Consensus that we need to find a way to get move patients involved - how do we get more patient involved? Very important to find ways to get more interested adults.

Terri - how do we get more adults in a group in Barcelona. Bob - we recognize we need an adult resource board.


Switzerland - mentioned there are some communication ideas for



adult resources. Bob - the current adult meetings that have happened that have been very powerful.

Greece - would like a patient/adult advisory board.

The following pharma companies presented their updates:

1. Takeda update and Charitable Access Program (CAP), Daniel Fertek
2. Ultragenyx, Tom Pulles
3. Sanofi update, Barbara Diana and Sanofi Humanitarian Aid Program, Jasmina Ahrens
4. Biomarin, Steve Jadhav
5. Regenxbio, Vivian Fernandez and Paolo Falabella
6. Orchard, Annamarie Dillon and Leslie Meltzer
7. Abeona, Jodie Gillon and Juan Ruiz 

Day concludes at 18:00

Saturday July 6th 2019 (Day Two)

Best practice examples of successful projects - learn from each other

1. Newborn Screening in Brazil - Awareness Campaign to expand the screening – Amira Awada and Regina Prospero, Brazil

Organization has been around for 18 years. Amira went over Brazil's mission, vision and values. They work with Advocacy for public



politics and reimbursement. They use social media campaigns with various hashtags in order to measure engagement NBS, promoting campaign to enlarge NBS and working with government. Launched campaign in 2018 - working with 20 other organizations towards this goal. Presented ad that is being used to raise awareness of importance of NBS. Trying to tell the government that it is less expensive to treat earlier. Current NBS in Brazil includes only 6 diseases. Need to have 50 on NBS. In certain states they are paying for an expanded test but in large areas it is harder to convince the government to cover the cost of the testing. They have created a petition with a goal of 1 million signatures to take to the government to create a law to expand, without a sponsor.

Netherlands- working on expanding for 31 diseases - MPs will be on in 2021. Discussion on protocol for treatment based on NBS. Discussion on psychology impact and if certain diseases with late on set - should they be screened?

2. Therapy week – Michaela Weigl, Austria and Carmen Kunkel, Germany

Started originally in Austria over 20 years ago, 10 years in Germany. Germany: Completed a member survey to see what are the needs of patients. Realized lack of knowledge of physical therapies. Worked with an experienced physical therapist to determine how to structure the program. Created draft of program and got feedback from patients and parents. Determined they needed Physical therapy, working/occupation therapy, Speech, and animal therapy. Determined a 5 day program - with 3-4 programs a day – every year week prior to Easter holiday. Have 15 patients, include parents in therapies to teach them and siblings with childcare and programs for older siblings. Completed in a central location in Germany. Created budget for costs of all these programs and looked for funding from foundations and other grants, hotels, etc. It's a 6 months process to organize. Cost in 2019 for 33 thousand Euros. Have used same location every year. Now have 17-19 patients.

Austria: 50 participants- originally Now - over 200 participants and now includes patients from other countries. 7 days/6 therapy



days^[1]_[SEP] Reuse same hotels to make it easier to plan Programs for parents and siblings to do their own workshops and fun activities. Creative workshops and theme nights are extra programs for families. Photography is available for families and outings are available.

As requested on a e-mail sent by Michaela Weigl, on July 27 of 20, it was included more relevant informations about the Therapy Week , a project that MPS Austria executes for more the 20 years:

“Austria started therapy week 20 years ago with 50 participants and two therapies only. This has grown since over the years to about 200 participants including a minimum of 15 therapists and a range of single therapies from physiotherapy, ergotherapy, different massages, cranio sacral therapy, osteopathy, Rolfing, Feldenkrais, dog assisted therapy and creative therapy to a range of group therapies (mainly for parents and siblings) like Nordic walking, water gymnastics, back gymnastic, progressive muscle relaxation, relaxing, mountain biking, tennis or line dance. Additionally we offer different workshops like Genetics, Nutrition Counselling, Physiotherapy, Mindfulness and Simulation Trainings for Emergency cases. Physiotherapy is a must and you can choose freely from the other offers, so that there is an individual plan for each patient.

In 2019 this is resulted in a total of 562 therapies for 40 patients and their families in 6 days during a stay of 7 nights.

There are 3 to 4 evening programs like concert, surprise-evening, fairy tale evening, talent show or karaoke. Additionally we offer photo shootings for patients which can be booked in advance.

Childcare is guaranteed by at least 10 volunteers who offer a colorful game and handicraft program for mps children and their siblings, but also take care of the severely disabled.

Usually we change hotels to give families also a holiday-feeling and new possibilities to explore the area during our outings, because this week is the only holiday during the year for some of our families. However it makes organisation easier if you reuse hotels, which we



did with the best of them.

We use to be open for participants from other countries to show them our way of doing this, hoping they will be able to do similar events in their own country like Germany already does.”

3. Advocacy in APAC Basin^[SEP] Using social media to boost advocacy campaign – Vanessa Ede-Scott, Australia

Australia- Social Media Campaign

Rare Disease day project completed with video Project AIMS - around rare disease day - produced video of a day with 2 patients, to connect families, worked with PR company and mapped out primary objectives to impact the quality of life of MPS. Target parents, general public and public officials. Aligned with Rare Disease Day - February 28 and then leverage on MPS Awareness Day Used mainly social media as the campaign platform. Cost \$10-15K. Created video - have different lengths on videos. Had 10 days campaign on Facebook - reach 40k in 24 hours. End of 10 day - 252K views. Video became finalist in NY Film Festival Film and Television Awards. Video is available on MPS Australia website.

New Zealand - Communicating with families

2 projects^[SEP] In 2009 - started working with Mistry of Health to communicate via video chat to work with families in rural areas and connect them with care needs etc. System is working in every major hospital in New Zealand - rural families can connect in local hospital to major medical centers.

Social Media - no therapies - working on social media to promote need for therapies. Advocacy work has been consistent to work with government to try to get policy for Rare diseases and action plan - went to Media with stories of families and patients - used change.org for petitions for New Zealand people. Brought families to the capital to



get the petition in front of government committee- petition was tossed out but still working on other petitions with other Diseases - feels they are making progress and using social media can effect change.

Ride for Hope Yee Seng Lee, Malaysia

4. Support for newly diagnosed families – Bob Stevens, UK

Bob Stevens - UK - Support for Newly Diagnosed families Discussed how families become members of UK Societies-through children's services/works in connection with specialists Free membership for UK residents After membership - 9 social workers cover the membership - each specializes in specific disease Also put them in contract with another family Home visits offered to every new member Visits are 2/3 hours

How to strengthen and promote MPS Awareness Day on a global level in the future?

Ideas and discussion

Amira discussed how to promote MPS Awareness day as an international community. She would like all countries to share information that can be put out for MPS Awareness Day in their languages. [L] [SEP] US - is there something we can be doing more to help foster a global message for MPS Awareness Day? Various ideas were discussed for awareness ideas and themes to promote on Awareness day.

International MPS Symposium 2020 in Barcelona, Spain - update

Preliminarily scientific and family program Friday July 31 - Sunday - August 2 - ending midday program still working on to be determined. Volunteers will be needed to help. Registration will open in September on website. International meeting will begin on Wednesday afternoon and also on Thursday. Carmen - suggested



understanding of cost of interpreters.

International MPS Symposium 2022 in Luzerne, Switzerland – update

Access via train from many area airports. Conference will be in KKL. Start is Wednesday evening. Program begins Thursday – Saturday. International network meeting will be on Tuesday/Wednesday.

Discussion and proposals to host the 2021 International MPS Network meeting

Turkey offer^[SEP] Netherlands suggested UK for June/July^[SEP] It was determined that Turkey would submit a proposal to the Network

Challenges with writing grant applications and relationship building with pharma industry

Terri discussed her business background and suggested her presentation will help our organizations raise money and work with industry to partner with them in order to translate to funding. She discussed how to secure a grant.^[SEP] How you make someone feel in the grant process is the most important thing. Grant writing is a process - grant preparedness is important - you must understand your organization to determine if a grant is something that can be executed. It is a 5 step process - understand financials, get board commitment, define grantor, build your case, and grant writing. Terri discussed each step in detail.

Challenges with reimbursement for existing and new treatments – country perspective

There is concern that future and new treatments will not be funded by various governments.

Switzerland- ERT for all MPS available - reimbursements for MPS 4 is not clear - depends on single insurance company - one on one basis so very complicated-70 different insurance companies- the govt



insurance pays until 20 - late diagnosis also creates a problems.

Brazil – MPS 1 & 2 - fully reimbursed by government; MPS1 - no limits; MPS2 - treated until physicians determined if treatment can continue. Takes a month for 1/2 treatment; MPS 4 & 6- approved for reimbursement last year - but government has to still determine how to work with pharmaceutical - its taking a long time- it will be re-evaluated in 3 years - all patients currently treated through a lawsuit- but it is taking 2 years from diagnosis to get treatment. BioMarin will only treat now for patients under the lawsuit - they are not donating drug. Talking now about new drugs/trials to determine how to fund in the future.

Sweden - ERT - MPS 1 & 2 available, not available for 4 - decision in 2015 that is too expensive. Last week approved they would revisit decision. MPS 6- treatment available- struggle for attention for rare disease drugs. Average waiting time is 3-5 months.

US - ERT for all patient receive drug via private insurance or state/government assistance program. In past 2 years, private insurance companies have said they are not going to get the drug anymore. We constantly write letters to support drug to get insurance company. Due to communication issues and red tape at private insurance companies. Families are considered. Access can be in a week when diagnosed.

Netherlands - all ERT with exception of MPS 4a because of the price and not successful trial results. MPS 1- is reimbursed ERT. They have severe type of MPS 1 and they are transplanted and not on ERT. All MPS 1 are diagnosed within first year of life. Big population MPS 3 in Netherlands. Most IIIa - population can live into 60s. Average time for ERT - no waiting time to get drug. But new drugs at least 1 year.

Ireland - MPS1 - Hurler - ERT on diagnosis and then transplant in England- all other MPS 1 - ERT MPS 2 - ERT - over years patient may stop treatment if physician decides. MPS 6- ERT available MPS 4- only 3 - 2 girls on trial - received from BioMarin until December



2017 and government would not fund - eventually with media and

legal campaign got funded - got another one. None of MPS 4 adults have asked for it.

Serbia - MPS 1, 2, 4 ERT reimbursement - MPS4 - only children, adults were denied. No MPS 6 patients. Main problem - atmosphere of doctors not positive. This year 2 MPS children removed from therapy. Medicine is planned by annual budget.

Hungary- treatment for all types - waiting period is 6 months to a year and gaps in treatment due to committee not meeting regularly.

France - MPS ERT - all available - waiting time is a few months

Spain - have approval MPS 1, 2 and 6; MPS 4 & 7 - no approval; 20 patients - MPS 4- apply for treatment - under conditional approval - they are receiving drug-paying Germany price. 4 other on MPS 4 treatment. Gaps in treatment occur to do payment between Spain and Germany a problem for all rare disease. Waiting time for 1-2 months but it depends and sometimes is difficult.

Botswana - no treatment available for MPS.

Argentina - All receive ERT. Takes a long time for correct diagnosis but once determines treatment available quickly but depends on levels of approval can be several months depending on system. Health system in Argentina very fragmented due to segregation of states, cities, etc. Government wants everyone to have access and be universal health care but currently fragmented. 300 patients- 2 girls MPS II.

Denmark - MPS 1, 2, 4, and 6 receive ERT - most waiting time is a month.

Turkey - ERT all treatment available. Waiting time is 1 to 4 months. Currently 65 patients MPS 2. 428 patients - MPS 4. If patient cannot



walk - MPS 4 treatment not available. 153 patients - MPS 6, MPS 7- waiting for approval from scientific panel MPS 7 - 8 patients.

Greece - 8 members in association - all treatments are reimbursed- annual basis approval - sometimes government can say no and then can get approval after 1 year. 80/70 patients in country. MPS 1 - treatment is availability quickly MPS 6 and MPS 4- 3 patients - not approved. To be covered you must have a job - parents must have a job.

Germany - access to all treatments-including type 7 - waiting time is minimal but can depend on the doctor and administration facilities to receive treatment. Every once in a while, a legal battle is necessary for patients that are denied and society assists with this.

Most countries allow home infusions^{[L][SEP]} Greece only hospital^{[L][SEP]} Brazil - only MPS 2 - Takeda has a reimbursement assistant for home infusion.

Clinical trials – how to support families?

Marija - 80% of trials are failing to recruit patients. Presented an insight into the participation of rare disease patients in research.^{[L][SEP]} Country perspective on Clinical trials. Do your patients participate?

Switzerland - patients much travel aboard to participate in trails.

Brazil - one trial for MPS 2 ERT - cannot travel aboard for trails.

Sweden - no Clinical trials- families would like to travel but have not been accepted - due to language barrier.

US - clinical trials- we do significant education to our families - we have patients traveling to other countries for trials.

Netherlands - no clinical trials- very strict ethic requirements so not ideal for companies - had MPS 3- and have families travel to UK - feels drug companies have very strict on criteria for recruitment of



patients and they are had to find an exact match. Drug companies need improvement on their outcome measurements - they need a lot of changes here - would like to see more work completed on end point discussions and contribute to trial design. Patient voice must be included.

Ireland - no trials in the past patients on other trials in the UK Serbia - no trials - trials aboard are difficult due to EU^[L]_[SEP] France - trial for Lysogene to start soon- a new patient ad board

Spain - a few trials - IIIa, IIIb, 609a

Botswana- none

Argentina- no patients qualify for trials, they have a physician that would like to do more research but does not have Human Resources to do so MPS 6 patient is going to Italy.

Denmark - none and not aware of those traveling aboard.

Turkey - first year a clinical trial approved in country - 2 patients - IIIa and IIIb both BioMarin.

Greece - no clinical trials and try to inform patients but not a lot of interest.

Germany - have had trials in past and currently have a few trials though out and others coming soon.

We discussed that we need to educate our patients and ourselves

How do we support patients after trials are dropped? We need to speak up with Pharma for services after.

Terri shared that NIH (National institute of health) webinars are a great source for updated on gene therapy and other education opportunities.

We discussed that our patients must know the consequences for



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entering a trial and if a trial fails the patient may be excluded in the future.

Best practice examples of successful projects - learn from each other: cont. ^L_{SEP} **5. Fundraising – Raising Mission Dollars and Creating Sustainability – Terri Klein, US**

How do you create a legacy and ensure that your organization can maintain itself once it is gone. How do you diversify funding? Terri discussed different funding sources - grants, donors, fundraising, Legacy giving/major giving, and MPS Awareness/use this opportunity to raise money. Think about partnering with other organizations and entities that you and partner with for fundraising. Public awareness - use communication tools to raise awareness and to raise money. Actions create results and sustainability.

Meeting concludes at 17:30, Lisa Todd, United States National MPS Society