A word from the President:

Dear All

The first Newsletter of 2019! Spring is upon us and we are very proud to present you with the latest news from FIN!

We are finalizing the program for our annual Expert Meeting in Barcelona, we look forward to having you at the meeting! I also want to encourage all of you to participate in our photo-contest we are organizing during Fabry Awareness Month. Please do send us pictures and updates of the activities you are organizing during this month and we will be happy to share this within our network. Of course, you will find a lot of other news which I am sure you will find an interesting read. We also invite you to participate in the Japanese Survey. We would like to emphasise the importance of taking part in this initiative. We would also take a moment to express our gratitude to Toni Ellerton, our coordinator of many years, who will move on to new tasks after the FIN headquarters have moved to the new location in Belgium.

Please enjoy reading the FIN newsletter.

Looking forward to seeing you in Barcelona!

Lut, FIN president
The Fabry International Network is pleased to announce that the 7th FIN Fabry Expert Meeting will be held at the Front Air Congress Hotel, Barcelona, Spain. Unlike previous meetings we are planning to extend this annual meeting over two and a half days. The programme will open with focus groups on Friday 24th May followed on Saturday and Sunday morning by a day and a half of presentations from worldwide professionals specializing in Fabry disease.

We have an exciting program lined up.

Accommodation and events will be at Front Air Congress Hotel, Barcelona, Spain: Which offers a free 10 minute airport transfer from Barcelona-El Prat Airport every 30 minutes. Please see website for more information on airport transfers [https://www.hotelfrontaircongressbarcelona.com](https://www.hotelfrontaircongressbarcelona.com).

Every year we like to encourage as many FIN Member Patient Organisation representatives as possible to participate, but unfortunately have a limited budget and can only offer travel bursary to one person from each organisation.

We very much hope that you will be able to attend the FIN Fabry Expert Meeting and should be pleased if you would confirm your place as soon as possible (before April 10th) by completing the booking forms and returning to info@fabrynetwork.org if you have not yet confirmed your attendance.

[Please click here to see the full program]*

* please note that this program is still subject to change

We look forward to seeing you in Barcelona!

Lut de Baere
President
Fabry International Network
International Fabry Women’s Day

Firstly, I would like to introduce our patient association: Fabry Support & Information Group Netherlands (FSIGN) was founded on 9 March 2001 with the aim to unite patients suffering from this condition and look after their interests by giving them a voice.

The FSIGN achieves this by spreading information about the disease and exchanging knowledge regarding the handling and the treatment of the disease. FSIGN works on disease awareness, both for patients, as well as for practitioners and scientists. The association informs its members through a regular newsletter and the FSIGN website: http://www.fabry.nl.

Annually a general members meeting is organized, where patient information is provided by practitioners and scientists. In addition, the Association assists its members where possible by means of a dedicated patient contact person. Members that start on home infusion are provided with an IV pole. Since 2005 FSIGN also organizes an annual “women’s day”, every first Saturday of April.

FSIGN is a member of Fabry International Network (FIN), as such our intention is to collaborate with FIN and leverage their existing network, to maximize the outreach Fabry-awareness and to encourage the establishment of a Female Fabry Day.

In 2011 FSIGN won the first PAL-award. This prize was awarded by Sanofi Genzyme. They launched the (PAL) Awards grants program in 2011 to encourage bold new ideas and programs to support the Lysosomal Storage Disorder (LSD) community worldwide. That year, Fabry Support & Information Group Netherlands was the only Fabry organization worldwide that won the prize with a project written by two female board members of FSIGN (both Fabry-affected).

This project was named: Fabry International Female Initiative (FIFI) An important part was to promote project FIFI internationally by organizing a recurring International Fabry Female Day.....Every first Saturday of April

Motivation of the Project:
Women and girls have always been seen as only carriers of Fabry disease. Over the past few years, it has become common knowledge that also women and girls can develop (serious) symptoms as a result of Fabry disease. This is one of the reasons why FSIGN decided to organize an annual women’s day.

Purpose of this women’s day:
• Peer support contact
• Exchange of experience
• Inventory of needs
• Inventory of problems and ideas that may be of interest to all patients with Fabry disease and other Lysosomal Storage Disorders.
**Goal of this day:**
To increase recognition and support for all women with Fabry Disease and those directly affected by Fabry Disease (partner, mother, family, friends).
Everyone named above, who is also member of our patient organization, is welcome to join us during the Women’s Day. This can also increase your number of memberships!

**Message:**
In 2013 FSIGN sent the below message by email to all Fabry patient organizations we could find on the internet:
(Since then we received several reactions of organizations that organized an International Fabry Women’s Day but we would like to see so much more activities being organized and mark that day on the calendar …..)

We want to let you know that we, as a Dutch Patient organization, we officially declare the 1st Saturday of April as: International Fabry Women’s Day.

For the past years, the Dutch patient organization (FSIGN) has organized an annual national Fabry women’s day for our female Fabry patients and other women and girls directly affected by Fabry Disease. Every year we receive lots of positive feedback for this event. It is an opportunity to come together and share experiences, exchange relevant information, show solidarity and understanding and mostly offer support for each other.

On this International Fabry Women’s Day, we want to increase recognition for Fabry Disease and the impact it has on the lives of the women it affects. Our hope is that on this day, activities will be organised for all Fabry women.
It doesn’t matter if these are big or modest, physically or mentally challenging activities (depending on the resources available) as everything you do matters.

Here are some examples of the activities we organised in the past for Fabry Women’s Day
- A dinner for our female members, where everyone regularly swapped seats to stimulate networking.
- A group dinner to develop an inventory of the most common physical complaints experienced and discuss these together.
- We also organised industry sponsored workshops (don’t hesitate to ask for sponsoring to help you get started!).
- A group graffiti workshop: to express emotions creatively and share with each other what Fabry means to them through the form of art.
- A painting workshop to express feelings through painting.
- During our 10th Fabry Women’s day, we spoiled our ladies with a beauty treatment and many emotional conversations in between.
- High Tea, with an inspirational speaker.
- Meeting with a storyteller/lyricist and photographer. The lyricist taught us how to write down and express our feelings and sorrows. We took pictures of all participants and together with their stories, being used to create a book about Woman and Fabry disease. (Coming soon)
- Other suggestions: sending postcards, create a telephone circle and organise regional meetings …..

Please let us know how you filled in your own International Fabry Women’s Day. We very much look forward to receiving your reports and pictures.

An “International Logo” is developed for this special day.
You can use this logo, to announce your own International Fabry Women's Day activities.
Please let us know if you would like to receive this logo.

Upon request, we can also send you a flyer with the logo, where you can place your own text and use for your activities.

*Erica van de Mheen*
(treasurer@fabrynetwork.org)
**Fabry Awareness Month—Photo Contest**

Each year, during April, we aim to increase the awareness of Fabry Disease by increasing understanding of this rare condition and impact of living with Fabry Disease as a patient and family.

Fabry Awareness Month is all about telling the world about this rare disease. The more people that are aware, the more patients we can help get that early diagnosis. We hope that this year you also join us in sharing details about Fabry Disease with your family, friends and doctors!

**To celebrate Fabry Awareness Month FIN is organising a photo contest!**

**Fabry & My Future**

Send us a picture during the month of April and let us know how you see your future! Share with us how you make your life valuable every day, despite daily pain or other discomforts. Tell us a bit more about yourself and your picture.

A contest without a prize would not be a contest, so we will choose three pictures and announce the winners at the end of April and make sure the winner receives a nice prize!

Make sure the picture you send in, is a high resolution picture as we plan to print the picture and also present it during our annual Expert Meeting in Barcelona!

Send the picture to: info@fabrynetwork.org before the end of April 2019
The Japan Fabry Disease Patients and Family Association (JFA) aims to overcome the disease together with researchers and medical professionals. We also hope to establish the symbiosis society to promote Fabry patients living happily with their own dignity. We wish to be the closest support for spending their everyday life with smiles as much as possible. We planned this survey in hopes that it can help improve the quality of life and improve happiness for Fabry patients and others. We feel extremely honoured that Lut and the entire FIN board have given us this great opportunity.

**Purpose of the survey**
To improve the lives and treatment balance of patients. We believe that it is very important to know about Fabry patients who live outside of Japan for and how they experience treatments and life as a Fabry patient. We believe this could be the perfect opportunity to collect and exchange information and more importantly learn from each other.

**Survey content**
The survey investigates all life aspects “Care-Life-Balance” for Fabry patients and families on a global scale. The survey is divided into 6 topics. (About you / Treatment & Study or Work abroad / Employment / About patient support groups / New born screening / Gene Therapy)

**Survey Respondents**
Any Fabry patients and their family or caregiver(s) regardless of receiving treatment.

**How to complete the survey:**
The survey can be completed through “Google Form” (available in English only)
Please click on the link below (or copy and paste this to your browser)

https://goo.gl/forms/Rtw18DEVfkbAaSKt1

Answers are anonymous. The data will be collected and used only as a reference point during medical meetings. Your email address and/or any other personal data will not be recorded when you complete this survey.

**Survey Deadline**
End of Friday March 29th, 2019 GMT

We would greatly appreciate if you could take the time to complete this online survey. Your opinion is extremely valuable to us. A report will be created once all data is collected. The results will be used as only as a reference point during medical meetings. Also, we are very happy to share the results with you at the upcoming Fabry Expert Meeting in Barcelona.

If you have any questions about this survey, please do not hesitate to send a message to: jfaowl2014@gmail.com

Best regards
Japan Fabry Disease Patients and Family Association (JFA), Hisao Harada, President and the JFA Survey Team
The WORLDSymposium™ is an annual research conference dedicated to lysosomal diseases. WORLD is an acronym for "We’re Organizing Research on Lysosomal Diseases". Since its foundation, as a small group of passionate researchers, in 2002, the WORLD Congress has grown into an international research conference that attracts more than 1,600 participants from over 50 countries around the world.

WORLD is not only a great opportunity to connect with our friends and partners around the world, but also an opportunity to look at how we can work more closely with limited resources to improve the lives of the communities we serve.

This congress was again of a high level and a lot of advances were presented there. Not only the speaker sessions were interesting but also the posters were worth seeing.

Science has come a long way, but it is clear that there is still a long way to go in perfecting new treatment options for rare diseases. Gene therapy is again more complicated to get right than we thought. Many initial reports from surveys were somewhat disappointing, but this is going to happen. We have all become too enthusiastic about gene therapy and think this is the answer and it is already perfected. It can be an answer, but many of the technologies need much more work before we can say it for sure. There are other answers, such as new and existing ERTs. We think they will play a major role in the near future and we know that for many people they are a good treatment option. For us, one of the most exciting, also realistic, short-term developments is oral therapies. We know that for some patients they are a good option to give them more freedom, but there are also psychosocial issues that need to be taken into account.

The Pharma industry and science do good things and there are some setbacks, but even these can be considered progress if we learn more about the diseases each time. We understand too well that time is not our friend in the rare disease community, but let us have faith in those who work on new treatments. We have some of the best scientific minds working on new treatments for rare diseases and we are pleased to be part of their dedication to finding a way to create a better future for the rare disease community.

They also paid a lot of attention to clinical trials. Patients participating in clinical trials are heroes and pioneers. The science that is being developed to make these treatments a reality is at the forefront of what is scientifically possible today and we’re amazed at how many smart scientists and professionals there are working on these treatments. But without the patients being prepared to participate in clinical trials there would be no new potential treatments.

Participating in a study can be a daunting thought - not just about treatment, but also about how it affects our lives as patients and patient families. We understand the problems that families face when they participate in clinical trials. We know how stressful life can be with a rare disease.

Besides attending this conference, we had many meetings with different companies, who wanted to tell their latest advancements and to make arrangements for cooperation projects, such as the Charity program (Takeda) and the Humanitarian Program (Sanofi). In Belgium it can sometimes be very difficult to get reimbursement for an orphan drug. In some countries there is simply no hope to obtain a therapy or ERT for rare diseases. We, and many others, consider it our moral duty to give hope to these people by working together with industry to provide treatment and therapy for the people in these areas.

Unfortunately, only a small percentage of our rare community can benefit from these programs because it is simply not possible to offer everyone free treatment, but it is a small step in the right direction. These humanitarian programs can only take place through cooperation together with our nonprofit partners from other countries and innovative programs from industry partners such as Sanofi and Takeda. We are proud to be involved, because in the end it is all about you - rare but not alone.
Following the WorldSymposium™ there was the annual national meeting of the Fabry Support & Information Group (FSIG). Jack Johnson, Executive Director, FSIG and Vice President for FIN US, invited some of the FIN board members to attend this event.

The members’ meeting was opened by Jack.

During the meetings, the companies (5) were given the opportunity to give an update to the attendees. In Europe, it is not allowed for companies to have direct contact with patients, but the US has different rules.

There was an update about Fabry disease by Dr. Robert Hopkin. He did this based on the different abstracts and posters that were presented at WORLD, this was a good summary of some of the new information that was presented.

Dr. Lau then gave a presentation about the neurological items and Nadia Ali, PhD presented ‘Fabry fog, what is that?’ ‘Patient empowerment’ was again a very interesting topic brought by Dawn Laney, MS, CGC, CCRC.

Day 1 one was a very full day of presentations and we learned a lot. The day ended with a joint dinner, preceded by the welcoming of K.C. Wolf, the mascot of an American football team. During the dinner, Dan Meers gave a presentation about his life after his accident in the stadium. Dan planned to do a bungee jump from a zip line attached to the lighting tower of the stadium as an act at the start of a football game. The accident happened while rehearsing the jump. The jump was partly successful, but he ended up hitting the seats and was catapulted back into the air, and then had to ride the zip line down before being put on the stretcher. He was seriously injured, but recovered after a long rehabilitation. He told the story about his experience in a motivating and humorous way, which inspired many of us. The moral of the story was that people always have choices in life, just by being positive, you can make your life valuable every day, despite daily pain or other discomforts. You should make valuable use of opportunities you are given in life.

The second day was much more informal. Personal stories were told and tips and tricks were exchanged. It even became very emotional occasionally. But it was good to be able to share this with others.

A few examples:
Students can request an adapted program so that they can process the course material at their own pace. Another example was that some children receive their infusion in the classroom (the nurse stays with them in the classroom), which is not heard of in Europe. One person stated the needle piercing is painless when you stretch or warm the skin. Some patients have a modified infusion schedule (e.g. the full dose every week or every 10 days).

We were very happy to have been part of this meeting.
We noticed that there are many differences between the American and European systems. Especially the fact that there are different types of health insurance policies in America, which means some patient are helped better than other patient. In America everyone can choose their Fabry doctor, but that does not always mean that they know what is happening in this field. Also, the patient is not always heard properly, in other words, doctors do not really listen to the complaints and sometimes refuse to give the best available care or refuse to enroll people in clinical studies, even if they ask for it themselves.
GREETINGS FROM FINLAND!

The Finnish Fabry Association held its annual meeting this year in the oldest town of Finland called Hämeenlinna close to a medieval Häme castle which was built in the late 13th century. The meeting was held in mid-March and there were around 40 participants. This time the lectures covered topics like nutrition for a Fabry patient, living and coping with chronic pain and learning about a new phone app to self-monitor disease symptoms and to fill in the required annual information. The head of the Fabry learning center in Finland, professor Ilkka Kantola, gave a talk about the current situation in Fabry disease in Finland and what can be expected in the future. And yes, there were free time activities as well. From swimming at the hotel spa to outdoor activities and last but not least the insightful discussions between the participants which are always peer support at its best. Laughter and tears, serious conversations and jokes, old members and newcomers, sharing the good and the not-so-good. Those are the things that a successful patient meeting is made of.

Anna Meriluoto
(photos by Juha Meriluoto)
**Rare Disease Day 2019**

28 February 2019 was the twelfth international Rare Disease Day coordinated by EURORDIS. On and around this day hundreds of patient organisations from countries and regions all over the world held awareness-raising activities. The theme for Rare Disease Day 2019 was 'Bridging health and social care'. Focusing on bridging the gaps in the coordination between medical, social and support services in order to tackle the challenges that people living with a rare disease and their families around the world face every day.

Rare Disease Day 2019 is an opportunity to be part of a global call on policy makers, healthcare professionals, and care services to better coordinate all aspects of care for people living with a rare disease.

Here are some of the amazing activities our members organised:

**Fabry Australia** sold temporary tattoos for Rare Disease Day ‘Be Rare. Be YOU’.
They received permission from the Canadian Fabry Association to roll out the campaign in Australia. They started the campaign in Canada in 2018. They are still getting feedback now from members who sold them at schools and work places to raise awareness of Fabry Disease and raise money for Fabry Australia.

One boy living with Fabry Disease read the ‘Faber the Dragon’ story book which we published to his school friends to explain the condition he and his brother and family members have. His school dressed in plain clothes (no school uniform) to support Rare Disease Day and made donations to Fabry Australia and purchasing the tattoos.

**Rare Diseases South Africa** arranged a Denim Walk at the Walter Sisulu Botanical Gardens on Saturday 23rd February. They had a massive media drive which assisted them in reaching over 19 million people in South Africa on Rare Diseases – please view their media report here. They had a multi-sectoral stakeholder meeting (more info). They presented at the United Nations on inclusion of rare diseases within the context of UHC (more info).

We are all RARE in our own way. The CFA set out this Rare Disease Day to raise both awareness and empowerment with their Be Rare Be You Tattoos. The message is to embrace what makes you rare! Their campaign led to lots of media coverage as well as curiosity, discussion, and the magnitude of those who live with rare diseases. Their tattoos reached 16 countries, they sold 1000’s, and 2 people even made the tattoo permanent!

Other initiatives:
- Toronto CN Tower lit up in blue.
- Nova Scotia, Thornhill, and Toronto all proclaimed Feb. 28th as RDD.

UFA dedicated an entire seminar to Rare Disease Day 2019. Topics such as the diagnose and treatment of the disease where discussed.

The seminar was attended by representatives from the Ministry of Health and Public Organisations, doctors, students, patients and their families.
Thank you Toni!

The FIN board would like to give their warmest thanks to our coordinator of many years, Toni Ellerton, who will move on to new tasks after the FIN headquarters have moved to the new location in Belgium. Thank you, Toni, for keeping the board organised through several big and small meetings, patiently taking care of most of the background work to help the board and always serving our membership with a smile. You helped the FIN board navigate through a very tough period in its history and always kept us informed about the things that we needed to know.

Last but not least we thank you for your ongoing commitment to the field of rare diseases and we wish you all the best in the future.

Warmest Regards

The FIN Board
Dear 

My name is Charlotte Wauters, and I’m the new appointed coordinator for FIN. It’s very exciting to have been given the opportunity to work with the other, very skilled board members of FIN. I’m really looking forward to getting up to speed in this new position—I know I have some big shoes to fill!

I also very much look forward to working with the FIN board to provide help to the Fabry Community.

So, please don’t hesitate to reach out with anything you need, I’m happy to help!

Thank you

Kind Regards
Charlotte
charlotte@fabrynetwork.org

Follow The Fabry International Network on Facebook!
We will be posting regular updates and share the latest information from FIN with you!