



**FABRY**  
International Network

FACILITATE | SUPPORT | ENABLE

[www.fabrynetwork.org](http://www.fabrynetwork.org)

\*This meeting will be recorded

# 2019 was a year full of changes for FIN

- *FIN welcomed Fabry Korea association as a new member to the FIN, a delegation of FIN visited the association and presented at their first patient meeting*
- *SSIEM 2019 - FIN Booth - the FIN Board attended SSIEM in Rotterdam where we also had our very first FIN Booth*
- *FIN celebrated Fabry International Women's Day (every first Saturday in April)*
- *FIN published 4 newsletters with lots of interesting articles and a few new additions such as Interview with patient leaders around the world.*
- *For Fabry awareness Month, FIN organised a contest where we asked the community to send a picture that represents their future, which was a success.*
- *FIN partnered with myTomorrows. The myTomorrows platform offers information and facilitates access to clinical trials and expanded access programs available worldwide. They are dedicated to helping patients who have exhausted their regular treatment options. (clinical trials page on the FIN website)*
- *The FIN expert meeting 2019 was a huge success with more than 60 attendees (patients and patient advocates), we organised workshops for the first time and this was very well received by the community. We also streamed the meeting which we intend to do for future meetings*



# Looking back on 2019



myTomorrows



Having a psychologist facilitating the session, raised some very interesting topics and discussions!

Excellent way of having attendees interact with one another to get introduced! Good to share experiences!



Fabry International Network

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## FIN FABRY EXPERT MEETING

24<sup>th</sup> – 26<sup>th</sup> May 2019

Continue this very good work! Keep on educating by sharing knowledge to Fabry patients!



Every year the Fabry International Network organizes the Fabry Expert Meeting. This year it was held from May 24th to 26th in Spain – in the capital of Catalonia – beautiful Barcelona and was hosted by the president of the society for Mucopolysaccharide Diseases in Spain, Jordi Cruz, who also gave a presentation about the association he represents. During the meeting we had 78 attendees and among them there were patient organisations' leaders, physicians who are real professionals and have a tremendous expertise level in the field of Fabry disease and representatives of the pharmacy companies. This team led us all to experience interesting presentations, discussions and casual conversations not only during the meetings but also during the dinners.



This year we had two patients speaking – Francisco Albiol who is a Fabry patient from Spain and Wojciech Nadolski – a Fabry patient from Poland. Francisco told us his personal story of Fabry disease and what he had to experience during his journey to diagnosis and treatment. Wojciech guided us through the difficulties they face in Poland where reimbursement for treatment is not provided and this leads many people to develop severe symptoms. He also told us about very interesting initiatives they do to raise awareness about the disease among the government officials.

Doctor Nadia Ali told us about the psychological impact of Fabry disease and about Fabry fog. She also gave us a glimpse to the cognitive function examination procedures and informed that she is coordinating a 12 hour-long cognitive testing for Fabry patients.



Professor Dominique Germain gave a really clear and understandable description of X chromosome inactivation so to say the scientific basis of the Fabry woman. He also gave a presentation about family planning when one of the partners have Fabry Disease.

A representative from the Japanese association presented the results of their survey related to Fabry disease. We were also updated on kidney, heart, pain issues and other interesting topics.

Great experience to learn!

## FIN FABRY EXPERT MEETING

24<sup>th</sup> – 26<sup>th</sup> May 2019

This year's Fabry Expert Meeting also had a new initiative – a day dedicated to workshops. We had topics such as "Understanding the psychological needs of Fabry disease", "Symptom management", "Let's talk Fabry men" and "Let's talk Fabry women". All the patients and their caregivers were divided into smaller groups to discuss one particular topic and each participant could choose three workshops to participate in. FIN is working on a summary report based on the outcome of the workshops and will share this in the next newsletter. Participant feedback highlighted the desire for continuing the workshop program at future meetings.



As during all past meetings, the annual general meeting of the membership was held to discuss Fabry International Network matters and also re-elected 3 directors of the board for the three-year term. Of course the FIN board and pharmacy companies meetings prior to the Fabry Expert Meeting are to be mentioned. During these we received updates concerning what is happening in the area of treatment and discussed how the industry partners could help us accomplish FIN's mission – "a world where every single person affected by Fabry disease has the best quality of life possible through early diagnosis, treatment and cure". We will share that information among the membership through our newsletter, website and future meetings.

Thank you for your attendance and valuable contributions to the meeting!

**We look forward to welcoming you in Amsterdam to  
the FIN Fabry Expert Meeting on  
April 24th-26th, 2020!**





## Online Survey from JFA

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 an questions about this survey, please do not hesitate to send a message to:  
[jfaowl2014@gmail.com](mailto: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**.



## A glance of the annual symposium 2019 of the Society for the Study of Inborn Errors of Metabolism



Fabry International Network's vision is of a world where every single person affected by Fabry disease has the best quality of life possible through early diagnosis, treatment and cure. This can be done by organizing and helping to organize various initiatives, Expert Meetings and publishing a quarterly newsletter, also getting into direct contact with other people from the Fabry disease community: patients, family members, caregivers and other stakeholders. We also see gathering knowledge in order to provide the latest information to the membership as a crucial activity for our board. Therefore we attend scientific conferences related to Fabry disease and this time I want to give you a glance of the SSIEM (stands for - Society for the Study of Inborn Errors of Metabolism) annual symposium 2019 in Rotterdam, the Netherlands.



President Lut de Baere and four members of the board – Erica van de Mheen, Anna Meriluoto, Mary Pavlou and Martynas Davidonis attended the congress. We were very happy to present the FIN booth at SSIEM. For us this was a very successful event where we met with several new and known people to speak about FIN and its mission and how we could support each other. We are very grateful for this opportunity and will continue to share information through congresses such as SSIEM as FIN aims to facilitate, support and enable the Fabry community.

We also attended lectures and I might say that the future for all patients with inborn errors of metabolism as well as for Fabry patients looks very promising and science is moving forward quickly. Nowadays we might start looking forward to finally having a cure (what was just a theoretical possibility 20 years ago). The speakers at SSIEM were very strong and gave wonderful insights of what they do every day to work on a better quality of life for patients. Some of the lectures were easy to comprehend and some required specific knowledge or a medical background.

There were very few lectures about Fabry disease but there were more than 10 of them which dealt with the matters of novel treatments in general and keeping in mind that these diseases at some point have similar mechanisms this might be applied for Fabry disease at a later stage.





The FIN board members had chance meet with the pharmaceutical partners: Takeda, Sanofi-Genzyme and Amicus Therapeutics where we were informed about what is happening regarding the Fabry disease treatment and we also discussed our future collaborations. We also took the opportunity to have a board meeting after the sessions and meetings where we could discuss important issues that needed to be addressed.



Wrapping up this article I would like to express my happiness about the new medication which will hopefully be registered and could be used for multiple diseases as one of them is Fabry disease! Here we see a new concept of multi-disease treatment. Stay tuned for more details and news in the near future!

Martynas Davidonis



## Summary Reports workshops Fabry Expert Meeting 2019

This year's Fabry Expert Meeting also had a new initiative – a day dedicated to workshops. We had topics such as “Understanding the psychological needs of Fabry disease”, “Symptom management”, “Let's talk Fabry men” and “Let's talk Fabry women”. All the patients and their caregivers were divided in to smaller groups to discuss one particular topic and each participant could choose three workshops to participate in. We have summarized the outcome of the workshops to share this with you. Rest assured we have taken note of your feedback and will be organizing workshops again at the Expert Meeting in Amsterdam next year with similar and new topics!



### “Let's talk Fabry Men”

*Moderators: Martynas Davidonis and Jack Johnson*

As board members of the Fabry International Network (FIN) Martynas Davidonis and Jack Johnson were very eager to facilitate the Let's talk Fabry Men workshops and go over anything that is related to being a Fabry male. This is a topic we have wanted to address with the members of FIN for a long time and during the last Fabry Expert meeting we finally got the chance to do so. Of course we must understand Fabry men not only from the perspective as a man who has Fabry disease, but also from the perspective of a man without Fabry who has met a woman with Fabry disease. It is important to recognize it does not really matter at what level she may be affected because they could have children one day that are affected differently and this would impact both of them.

***“A man must have a boy, plant a tree and build a house”.***

We prepared a simple so called agenda for our workshop and it was based on a proverb which is common in many countries: “A man must have a boy, plant a tree and build a house”. This very briefly explains pretty much everything required to be a man: to be strong mentally and physically, be able to make decisions, enjoy successes and cope with the unhappy things as well as be able to enjoy life with a woman keeping in mind all these important aspects.

Despite our agenda we didn't strictly stick to it and discussed many additional topics. The discussions were dynamic, unique to each group and frequently full of positive emotion. These very fluid conversations reinforced the importance of open and frank discussions because we have many similar experiences providing a strong foundation for understanding, but we also bring our own distinctive points of view that allows others the ability to see things in a new and enriching light. By sharing both our similar and diverse experiences we are able to forge greater understanding between patients and caregivers as well as build ourselves and our community into stronger survivors.

# FIN Workshop: Symptom Management/sharing experiences

*Moderators: Mary Pavlou and Lut De Baere*

Lut and Mary led the Symptom Management/sharing experiences workshop in 4 different groups of 12 – 15 people each. At the beginning of each workshop we made sure confidentiality rules were well understood by every one and we agreed to share only the overall outcome of the workshops and no names or personal stories.

Every one shared their personal story and even though every story was unique there were also similarities. We talked about the age of diagnosis which for the majority was at the age of puberty and most commonly after the diagnosis of a sibling or parent. The latest diagnosis was at the age of 24 years old.



The experiences when growing up were common for all. The majority experienced problems with activities and sports in general and discomfort while exercising outdoors during warm weather. Pain, GI problems and hypohidrosis were all common symptoms. In some cases parents were advocates for their children during school time to ask for permission to go to the restroom or to be excused from gym and sports. Additionally alterations in diet were made by the majority to avoid GI problems. The most common was to avoid foods that you know you had problems with, eat gluten and lactose free foods, keeping a food and symptom diary etc. An interesting one was to eliminate all food and slowly start adding a new group of food (like fruits, proteins, dairy products, etc) every other week.

In order to deal with pain, other than painkillers or tegretol which is commonly used by Fabry patients most patients are doing acupuncture, TENS machine, dancing, ice patches, yoga or even CBD oil. Some using an app to monitor the pain in between doctor's visits. Weather always makes pain and discomfort more difficult as many report "always being freezing". Hot weather is also a problem. For migraine and headaches using a TENS machine, meditation, diet alteration and medicines seem to help.

For insomnia, tinnitus and anxiety that some reported, having melatonin pills (insomnia) and practising yoga helps a lot to assist walking or even steroids injections in the ear for tinnitus. Many experience hearing loss and for that reason they are using hearing aids.

High blood pressure, cardiac and respiratory problems are most likely to be treated with pharmaceutical medicines and machines (pacemaker). Some reported to have more Fabry Patients with a kind of asthma or cough in their countries.

Fatigue is another common symptom among Fabry patients that affects work, and decreases motivation for sports and exercise. Some are working part time, work from home, need more rest days than others or need more days to recover and/or take B12/magnesium pills.

Mental health is a big issue because everybody needs to be "normal" but the body doesn't allow it. Also some felt guilty because the diagnosis was only made after the elder sibling was already affected a lot by Fabry.

As for monitoring and doctors visits some can talk to their physician every 2 weeks before, during or after ERT. They all reported being well monitored but due to lack of time by the doctor in depth discussion of the symptoms do not take place. In some countries the visit is once or twice a year undergoing annual tests (ECG, hearing tests, eyes, kidneys, heart etc). Home treatment is available in many countries. In Taiwan a heart biopsy is an obligation for non-classic Fabry patients in order to receive ERT. Group therapy with psychologist is not common but it helps.



It's not new that not many doctors are aware of FD. There are not so many experts in the field and that's the main reason for delay in diagnosis or misdiagnosis as a psychological problem.

Overall the workshop was a great experience that everybody enjoyed very much and wants repeat next year.

# “Understanding the psychological needs of Fabry disease”

*Moderators: Nadia Ali, Ph.D. Health Psychologist, Emory University, Atlanta, GA, USA and Anna Meriluoto*

The FIN annual meeting in Barcelona this May was amazing! Many thanks to the Board for inviting me and so many thanks to everyone who participated in the Members Workshop “Understanding the psychological needs of Fabry disease.”

Twenty-five members participated in the workshop, in three separate groups led by myself and FIN Board member, Anna Meriluoto. We started each group by going over the rules of confidentiality (i.e. no one may share another person’s story outside of the group). Then members introduced themselves and shared a fun fact with everyone.

Next, each group came up with a list of psychosocial issues relevant to living with Fabry disease. We also contributed as a group to a list of coping skills for dealing with life with Fabry disease!

Anna and I promised that we would share the combined lists from all the groups with everyone in the next FIN newsletter, so here we go! (Of note, while not everyone with Fabry experiences ALL of these issues, someone with Fabry has experienced each one of them and we respect them.)

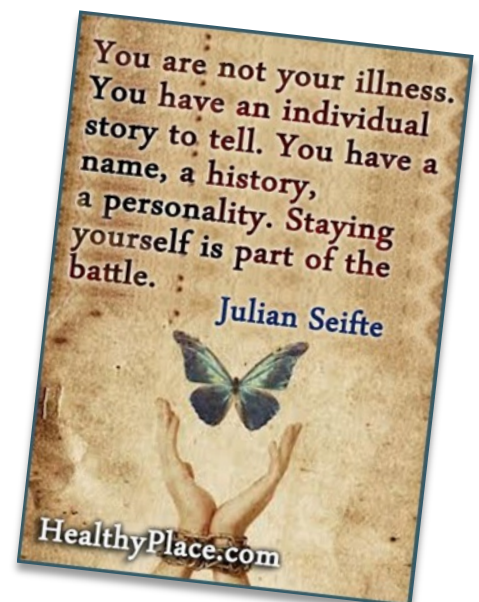
## Psychosocial issues relevant to living with Fabry

- Desire to be understood
- Guilt at passing Fabry on to your children
- How to tell family (extended family)
- Shock at learning diagnosis
- Uncertainty regarding disease progression
- Shame at being sick
- Being labelled a victim
- Career complexities
- Injustice (Is it fair that I’m sick and everyone else is healthy?!)
- Dating (When do you tell someone you have Fabry?)
- Deciding whether or not to have children
- How to parent children equally (between those who have Fabry and those who don’t)
- Teenage rebellion & transitions
- Denial (unable to accept limitations, overworking ourselves)
- Closed in your own thoughts / Over-thinking
- Invisible disease (you don’t look sick, so people don’t believe you have a chronic disease)
- Depression
- Anxiety & Fear
- Being blamed by other people (financial cost, societal cost)
- Loneliness, Isolation
- Desperate, feeling like giving up

## Coping Skills for dealing with life with Fabry

- Building community and connection with other people with Fabry
- Hope for the future / Faith
- Advocacy work
- Compassion / Relative adversity (Empathy for others and the idea that there’s always someone worse-off than you are)
- Reframing your identity (from that of a sick person to a strong person who happens to have Fabry)
- Finding your own passion (and engaging in it!)
- Humor / Laughter
- Therapy in all its many forms (Counseling, Art therapy, music therapy, pet therapy, nature therapy, sports therapy)
- Mindfulness and meditation (Clearing your brain)
- Compartmentalizing Your Pity (give yourself permission to feel it intensely for certain amounts of time and then put it away again)
- Learning to say ‘no’
- Napping
- Quality vacation time away from daily life
- Knowledge is Power
- Invisible disease (don’t have to tell people you have Fabry unless you want to tell them)

We sincerely hope you’ll find the list helpful and encourage you to try out different coping skills to find the ones that work for you!





# "Let's talk Fabry Women"

*Moderators: Anne Grimsbo & Erica van de Mheen*

Anne and Erica have put together a list of topics that were discussed during each of the workshops

## **Workshop/Group 1**

- Thorough personal introduction of the participants
- Different solutions in different countries. Even changes from region to region
- Descriptions of different ways of living with Fabry
- Personal reflections about being a patient, being a mother for children with Fabry
- Holiday and treatment. Your treatment should fit your life – your life should not fit treatment
- Relations to your family, caregivers, etc
- Corporation with your spouse to keep the family going

## **Workshop/Group 2**

- Introductions
- Being a mother of children with Fabry
- Options in prenatal diagnosis
- Women's right to treatment
- Difference between classical Fabry and non-classical
- Trouble with authorizations
- Organizational challenges
- Information leaflets and material
- Visible disease versus non-visible disease
- Women symptoms in particular
- Skin challenges

## **Workshop/Group 3**

- Introductions
- Family pattern
- Challenges at work
- GP's vary in knowledge in Fabry
- Differences between regions in countries.
- Home infusions.
- Criteria for treatment.
- Diversity of disease.





FIN recently started a collaboration with myTomorrows. myTomorrows helps patients with Fabry disease navigate the world of pre-approval medicines. The myTomorrows platform offers information and facilitates access to Clinical Trials and Expanded Access Programs available worldwide. They are dedicated to helping patients who have exhausted their regular treatment options.

myTomorrows' medical team is helping patients and their physicians. myTomorrows is an independent, globally operating organization based in the Netherlands. They provide patients with an unmet need, and their doctors, information about treatment options worldwide and facilitate access to medicines in development.

FIN and mytomorrows believe that everyone should have access to suitable treatment options.

Their goal is to ensure that patients and physicians don't miss out on treatment options due to lack of information, understanding and administrative hurdles.

[Click Here](#) to go the Clinical Trials Page on our website

Click on your country to get an overview of treatment options in development.



# Fabry Findings

FIN launched Fabry Findings - a scientific publication that is being translated into lay language which we share with the community and encourage to have this translated in to local languages.



# Fabry Findings



In this issue, we looked at the relationship between: objective cognitive impairment, subjective cognitive complaints and depressive symptoms

This issue is based on the publication:  
Predictors of objective cognitive impairment and subjective cognitive complaints in patients with Fabry disease  
Simon Körver 1, Gert J. Geurtsen2, Carla E. M. Hollak1, Ivo N. van Schaik3, Maria G. Published in scientific reports



In this issue, we explore pregnancy and Fabry disease, and present the latest findings on the use of ERT during pregnancy

This issue is based on the publication:  
Enzyme Replacement Therapy During Pregnancy in Fabry Patients : Review of Published Cases of Live Births and a New Case of a Severely Affected Female with Fabry Disease and Pre-eclampsia Complicating Pregnancy.  
Madsen CV1, Christensen EI2, Nielsen R2, Mogensen H3, Rasmussen ÅK1, Feldt-Rasmussen U4. Published in JIMD Reports



In this issue we focus on depression, its impact on Fabry symptoms, how to identify the signs and what treatments are available

This issue is based on the publication:  
Treatment of Depression in Adults with Fabry Disease.  
Nadia Ali, Scott Gillespie, Dawn Laney Published in JIMD Reports



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# FIN Projects



Quarterly newsletter  
> 450 subscriptions



FIN Website



Social Media Channels



International Fabry Women's Day



Collaboration & Communication



Fabry Awareness Month



Fabry International Network

[info@fabrynetwork.org](mailto:info@fabrynetwork.org)

[www.fabrynetwork.org](http://www.fabrynetwork.org)

# FIN Projects

- Newsletter – will be published quarterly in 2020 – first one was March, next is June – until date we have more than 450 subscriptions
  - Powerful tool to share information and connect patients worldwide
  - Plan on growing the number subscriptions and we will continue to gather as much relevant info as we can to be published in the newsletter
- Social Media Channels
  - Instagram/Facebook/YouTube – FIN has taken it's first steps into the world of social media, we will continue to reach out to the community through these channels
  - We will provide content to our members and industry partners that can be shared
- FIN Website
  - We will be working on updating the FIN, more accessible, more up to date and fresh look and feel
- Fabry International Women's Day – every first Saturday in April
  - Continue to raise awareness and supporting local initiatives
  - Content on social media / #InternationalFabryWomensday
- Collaboration and communication
  - FIN continues to support new and emerging associations
  - Monthly Board Meetings
  - Actively looking for new members to expand our network
- Fabry Awareness Month
  - 'Let's get Fabry Creative' Contest – winner was published in June Newsletter



## 15th Fabry Women's Day in the Netherlands!

Saturday, April 6th Fabry Support and Information Group Netherlands (FSIGN), celebrated its 15th Fabry Women's Day!!

FSIGN organized a workshop at 'cakes by SUUS' in the exact middle of the country, so everyone was able to attend.. (the longest travel is a two hour drive).

Another location in this town we also use for our Annual Membership Meeting every year.

The workshop 'decorate cakes' was a very big success!



Every woman took a very tasteful cake home with her, to celebrate our special Fabry Women's Day at home with her family.

This special day we celebrate every year on the first Saturday of April and meets a need that we could not imagine...

After all these years we got to know each other better, we have shared each other's joys and sorrows. We can comfort each other after bad results and support each other in difficult decisions. This is invaluable!

I would like to share a few experiences of participants:

*"What a great day it was. The 'cakes by SUUS' were delicious and so good to see you all again, it really has something special, it feels a bit like family... So much fun to talk with each other again and see how it goes. After the workshop we went for dinner. Was delicious and cosy. We sat at a nice table with a new member, so fun and very clever! When we are together, we feel strong!"*

*"Have you missed this party or are you still unsure if you should come?  
I would say: do it, the more the merrier...."*

*"We love to be every now and then, in a company of people who, like no other, understand what you're talking about if you tell about how Fabry takes up space in your life. And after all these years of membership, those people are just a little 'family'. We think it's very nice to see and speak to each other now and then. And so we met again, last April 6th, cosy with a large group of chatting ladies at 'cakes by SUUS' in Almere, on the annual international women's day!"*



As one of the two female board members of FSIGN, I hope that other patient organizations are also able to bring together women involved with Fabry, as there are Fabry-affected, mothers of Fabry-affected, wives of Fabry-affected, daughters of Fabry-affected, sisters of Fabry-affected, friends of Fabry-affected, so we can support each other!

Our only stipulation is that you need to become a member of our patient organization...

This is one of the reasons our patient organization in our small country (17 million inhabitants) has almost 150 members, of which 53% are Fabry-affected.

Good luck with organizing your own International Fabry Women's Day, April 4, 2020!

Erica van de Mheen

## Fabry Awareness Month 2019

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!

Here are some of the events that were held by our members during this month:



Fabry Canada is continuing to travel across Canada holding their Patient Empowerment Meetings and bringing both awareness and education. Through the month of April they shared patient stories, gene therapy experiences, and a female perspective with media across the country.

MPS Turk published this [video](#) on their social media.

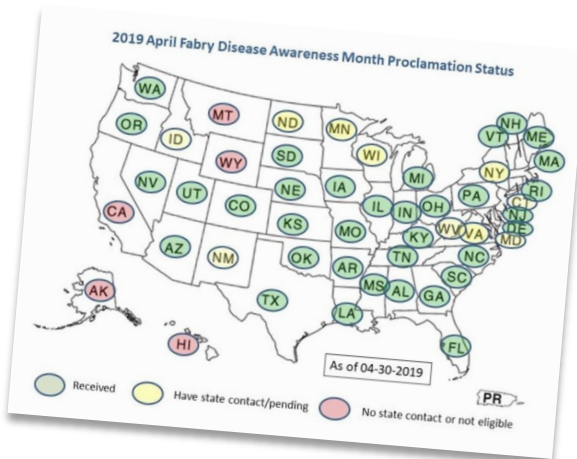
The U.S. National Fabry Disease Foundation (NFDF) coordinated with Fabry community members across the country to request state governor's proclamations for April 2019 Fabry Disease Awareness Month. Of the 50 states in the U.S., 35 state governor's proclamations were received this year as highlighted in green on the map.

The states highlighted in yellow on the map are states where proclamations were possible but attempts this year were unsuccessful for various reasons. The states highlighted in red have requirements that are difficult to meet or the state does not participate in a governor's proclamation program. Each year the NFDF and Fabry community state representatives work together to obtain as many proclamations as possible to maximize Fabry disease awareness.

In addition to obtaining formal governor's proclamations, the NFDF published a daily Fabry disease awareness post on their face book page at [www.facebook.com/FabryDisease](http://www.facebook.com/FabryDisease). The daily posts addressed a wide range of Fabry disease awareness topics to help people better understand, manage, and cope with Fabry disease and to better understand Fabry community programs.

The number of people who viewed the daily awareness posts in April ranged from about 1,300 to over 11,000. The number of people who follow the NFDF face book page is over 3,200 to date and growing.

You can see photos of many governor's proclamations and participating community members in the "2019 Fabry Disease Awareness Month Photo Album" at [www.facebook.com/FabryDisease](http://www.facebook.com/FabryDisease).



The 2019 Chinese Fabry Patient Conference, cosponsored by the Shanghai Four leaf Herb Family Care Center (formerly known as the Chinese Organization of Rare Disease, CORD) and the Chinese Fabry Patient Community, was successfully held in Beijing. Nearly 100 people including Fabry experts, representatives of pharmaceutical companies, rare disease genetic testing institutions, social welfare organizations, patient organizations and patient groups attended.

In this conference, the Fabry Patient Community released the status of the domestic Fabry group. Many patients shared their stories living with Fabry. Experts shared the diagnosis and treatment of Fabry Disease and the status of Fabry drugs. An intense discussion was carried out at the conference. During the meeting, Beijing 301 Hospital, Beijing Dongzhimen Hospital, Beijing Anzhen Hospital, and Beijing Tongren Hospital also conducted a free onsite clinic for the Fabry patients who participated in the conference.



## 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](mailto: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**



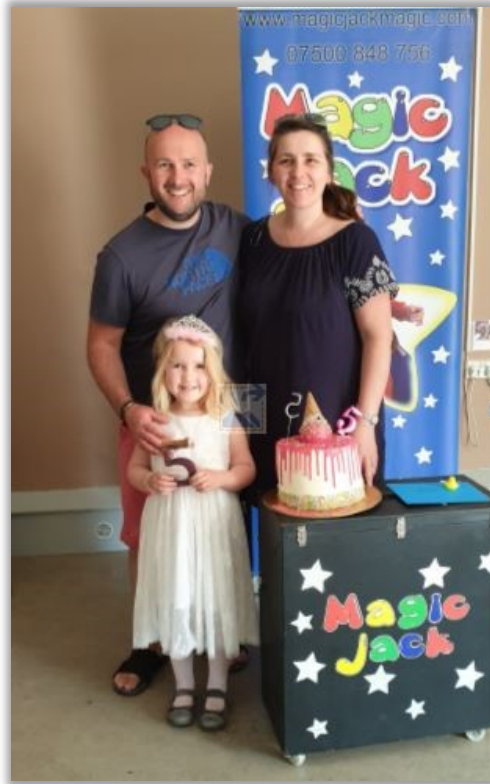
Send us a picture during the month of April and let us know how you see your future! Share with us how you make your life valuable every day, despite daily pain or other discomforts. Tell us a bit more about yourself and your picture.

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](mailto:info@fabrynetwork.org) before the end of April 2019

## Fabry Awareness Month 2019 - Photo Contest Winner



I was diagnosed back in 2011, a week before I started IVF. I really nearly pulled out of the procedure as I was so scared about the diagnosis and what it might mean for any should we be so lucky to have children.

Goodness me, after a whole lot of heart searching and deciding there are some illnesses that can never be picked up, had I been able to fall naturally I never would have terminated so I went ahead. Three cycles later, many losses along the way, our little Blossom is now 5.

She's put the sunshine back in our tummies, she is a constant delight and whatever the future holds, as true as the sun will rise, and fall every day she brings optimism and happiness into our little corner of the world.

The photo is me, my husband and Blossom at her magical birthday party last week. Every day with people who love you for being you, look out for you and you love back makes you the richest person in the world. I am content and happy with what I have. I try not to worry about the future, it's still 50:50 whether B has inherited the faulty gene from me. I think she has as she sometimes says her hands and feet are hot and itchy. We've been advised to not test her until she's older unless of course the symptoms take over.

My Fabry did not affect my medical when we were going through the adoption process before B arrived. I'm hopeful that one day soon we will be able to start the adoption journey again and we will be a family of four.

We are a vegetarian household and I'm sure my diet over the past 24 years will have helped my lysosomal storage disease. I'm not defined by Fabry, I'm defined as a wife and a mummy to Blossom, who is my end and my beginning.

That's how I start my every day.

Thanks for reading

Georgina Cartwright

Southampton UK

***On behalf of the entire FIN board, we would like to thank you and congratulate you Georgina, for sharing your story and sending in this beautiful picture of your family! We wish you and your family all the best for the future!***



# Membership

FIN is connected to over 50 countries and 62 Patient Associations around the world in 2019. Membership is free and open to any National Patient Organisation in which Fabry patients are represented.



New members in 2019:



Fabry International Network

[info@fabrynetwork.org](mailto:info@fabrynetwork.org)

[www.fabrynetwork.org](http://www.fabrynetwork.org)



FIN was invited to attend the first Fabry Korea Patient Meeting hosted in Seoul, Korea. It was our pleasure to present the Fabry International Network to them. We would like to thank the Songsuk Welfare Foundation and Fabry Korea for being such a wonderful host and all of the members for welcoming us to the meeting.



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# Industry Partner Meetings

In 2019 the FIN Board met face to face in individual meetings with Sanofi Genzyme, Shire Takeda, Amicus, Idorsia, Protalix, Freeline, Chiesi, AvroBio, GreenOvation. These meetings were held under Company CDAs (Confidentiality Disclosure Agreements) to enable the FIN Directors and the Company representatives to discuss matters not yet in the public domain. These meetings took place prior to the FIN Expert meeting in Barcelona.

FIN also met with industry partners at WorldSymposium™ 2019 in Orlando, Florida, USA and at SSIEM 2019 in Rotterdam, the Netherlands.



Fabry International Network

[info@fabrynetwork.org](mailto:info@fabrynetwork.org)

[www.fabrynetwork.org](http://www.fabrynetwork.org)

# Governance

At the 2019 AGM Lut De Baere was voted to continue as President, Jack Johnson - Vice President of Americas/Global, Anna Meriluoto - Vice President Europe/Russia. In January 2019 FIN moved its official address to Alice Nahonlaan 7, 9120 Melsele, Belgium.

## FIN Board:

- President: Lut De Baere
- Vice President Americas /Global: Jack Johnson
- Vice President Europe / Russia: Anna Meriluoto
- Director / Treasurer: Erica Van de Mheen
- Director / Secretary: Mary Pavlou
- Director: Anne Grimsbo
- Director: Martynas Davidonis

The FIN Board have met every two months during 2019 by teleconference and met face to face in February 2019 in Orlando, FL, USA and again in May 2019 Barcelona, Spain before and after the FIN Expert Meeting to take forward the business of FIN.

## FIN Coordinator

Charlotte Wauters was appointed coordinator for FIN in January 2019. She is based in Belgium.



Fabry International Network

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# Election Of Directors

- Mary Pavlou to be re-elected as FIN Secretary

Mary Pavlou was up for election in 2020

This year Mary Pavlou, member of the FIN Board and appointed secretary was retiring by rotation in accordance with FIN's constitution. There were no objections and she was re-elected as member of the FIN Board and appointed secretary.

- Resignation Anne Grimsbo & Martynas Davidonis

The FIN Board regrets to inform you that there are 2 board members resigning in 2020. Anne Grimsbo and Martynas Davidonis have submitted their letter of resignation and as of tomorrow they will no longer be part of the FIN Board. The FIN articles of association will be updated accordingly. Anne and Martynas were thanked for their efforts and valuable contributions, FIN wishes them all the best.

No new applications have been received so far. For the time being the FIN Board will move forward in 2020 with 5 Board Members. Applications to become a FIN Board Member are welcome at any time and encouraged anyone interested to contact a current FIN board member to discuss further.





# Financial review 2019

The closing bank balance 2019 was 184.253,24 EUR

Total income in 2019 was 142.801,40 EUR

Total expenditure in 2019 was 145.374,32 EUR resulting. The shortage of income over expenditure for 2019 is therefore 2.572,92 EUR and this results in equivalent lower equity.

The Fabry International Network is most grateful to Sanofi Genzyme, Amicus, Takeda, Idorsia, Chiesi, Avrobio, Freeline , Sangamo and Eurordis for the unrestricted educational grants and charitable contributions they have made to FIN in 2019.

The Year ending 31 December 2019 accounts were audited by BHKK BV (Belgium) and the Auditor's letter signed by senior accountant Hans Blockx.



# Funds received in 2019

Funder

Percentage of overall budget %

Industry

- Sanofi Genzyme 32.68
- Takeda 30.87
- Amicus 5.72
- Freeline 5.72
- Idorsia 5.72
- Avrobio 5.72
- Chiesi 6.15
- Sangamo 5.72

Non Industry

- Eurordis 1.7



Fabry International Network

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# What can FIN do for You and Your organisation?

Please do not hesitate to reach out to us, should you have any questions or suggestions – emails can be send to [info@fabrynetwork.org](mailto:info@fabrynetwork.org) or directly addressed to our coordinator Charlotte.



Fabry International Network

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