

Newsletter



March 2020

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A word from the President:

Dear All



FIN stands in solidarity with everyone affected by the COVID-19 pandemic. Our thoughts are with all of you who are affected by the virus. Please do not hesitate to reach out if there is any way that our community can support you and your patients during this difficult and uncertain time.

In the context of the current COVID-19 pandemic and in line with WHO guidelines, the upcoming FIN Expert meeting has been cancelled until further notice.

In this first Newsletter of 2020 we continue to share a lot of information and announce a vacancy for the FIN Board. Most of the FIN board members attended WORLDSymposium™ and we gladly share with you what we learned. The 3rd issue of Fabry Findings is also available now and we look forward to hearing your feedback about this.

FIN recently added a page dedicated to clinical trials worldwide on the website. Make sure you don't miss it! Of course, you will find a lot of other news which I am sure you will find an interesting read.

In order to make this newsletter as informative as possible we always welcome news and information from you as a caregiver, patient, patient organization and patient representative. Please send it to info@fabrynetwork.org

Stay safe and healthy!

A handwritten signature in black ink, appearing to read "Lut", written over a light blue circular stamp.

Lut, FIN president



FIN EXPERT MEETING 2020

In the context of the current COVID-19 pandemic and in line with WHO guidelines, the upcoming FIN Expert meeting has been cancelled until further notice.

The health and safety of all stakeholders participating in the Expert Meeting, including people living with a rare disease and their carers, is our primary concern.

We are currently looking into options to postpone the meeting. We will keep you updated when we are able to reschedule this event.

Please do not hesitate to reach out to us, should you have any questions.

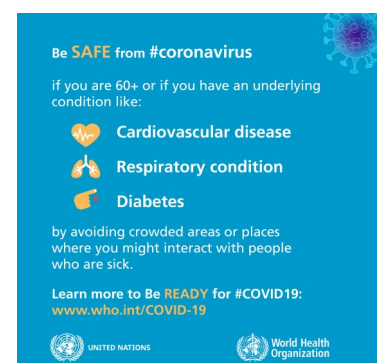
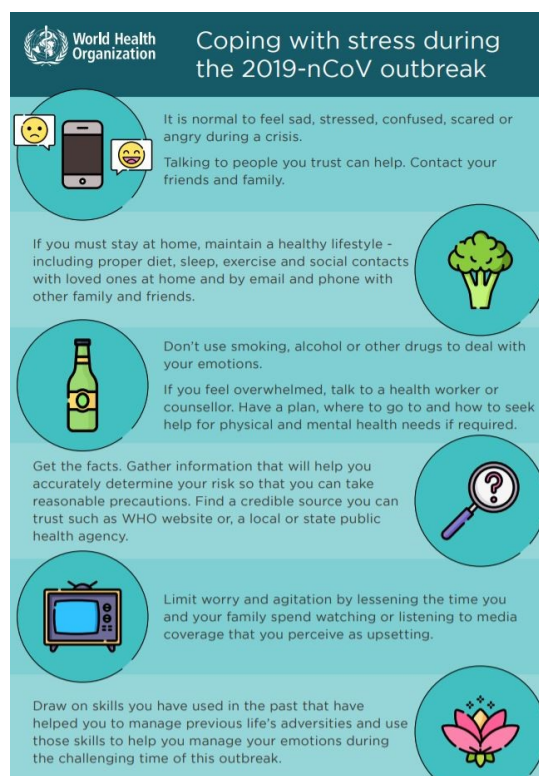
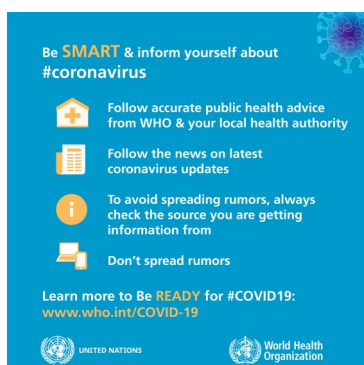
We hope that everyone will stay healthy and safe in these uncertain times.

Covid-19

To prevent the spread of the virus and in support of healthcare professionals, we encourage you to follow the guidelines given by the [World Health Organisation](https://www.who.int/) and your own national authorities.

Stay aware of the latest information on the COVID-19 outbreak, available on the WHO website and through your national and local public health authority. Most people who become infected experience mild illness and recover, but it can be more severe for others. Take care of your health and protect others by doing the following:

- Wash your hands frequently. Regularly and thoroughly clean your hands with an alcohol-based hand rub or wash them with soap and water.
- Maintain social distancing: Maintain at least 1 metre (3 feet) distance between yourself and anyone who is coughing or sneezing.
- Avoid touching eyes, nose and mouth
- Practice respiratory hygiene: Make sure you, and the people around you, follow good respiratory hygiene. This means covering your mouth and nose with your bent elbow or tissue when you cough or sneeze. Then dispose of the used tissue immediately.
- Stay home if you feel unwell. If you have a fever, cough and difficulty breathing, seek medical attention and call in advance. Follow the directions of your local health authority.
- Stay informed on the latest developments about COVID-19. Follow advice given by your healthcare provider, your national and local public health authority or your employer on how to protect yourself and others from COVID-19.



FABRY INTERNATIONAL NETWORK BOARD MEMBER

NOMINATION PROCESS

There is currently one vacancy at the FIN Board. Nominations for a new Board Member are now open and an election process is herewith given together with the nomination form. If you wish to put your name forward for the selection process to become a FIN Board Member, could you please complete the nomination form, remembering that you need one supporter. Please return your completed form to FIN.

Nomination Criteria

Nominations will be considered from people who meet the following criteria:

- Have an active interest in the Fabry International Network and are prepared to invest the necessary time and effort to fulfil the duties attached to the appointment.
- Belong to a Fabry Patient Organisation Board.
- Have the support of at least one colleague.
- Are comfortable in the use of the English language.

Nomination and selection procedure

- The nomination and selection procedure leading up to the election at the AGM is set out below:
- Nominations must be returned to the FIN Office
- Nominations must be supported by at least one colleague.
- Nominees will attend phone or in person interviews with members of the current FIN Board
- A selection of suited candidates will be presented at the annual AGM where the new board member will be appointed by the AGM attendees.
- All nominees will be advised of the outcome.

Role of the Board Member

- The role of the Board Member is to support the entire organisation through the process of governance.
- The Board plans FIN's future mission and priorities, it monitors performance and measures outcomes.
- The Board is responsible for all strategic planning and monitoring tasks and actions undertaken.
- Board members are individually responsible for participating in online and F2F Board Meeting. The language of these meetings is English and the appropriate expenses will be reimbursed (F2F).

[Click here for the nomination form](#)



Most of the FIN board travelled to the WORLDSymposium™ in Orlando in February. The WORLDSymposium™ Annual Meeting is the largest lysosomal disease meeting and exposition in the world. This year's meeting was attended by over 1,900 scientific attendees from more than 50 different countries, plus almost 40 exhibiting companies. The meeting provides a forum for the presentation and discussion of cutting-edge science in all areas of lysosomal disease research and lysosomal storage disease research.

The major objectives for the program are identifying key diagnostic features for lysosomal diseases, comparing the relative advantages and disadvantages for each therapy currently available, identifying unmet needs for these diseases and formulating new research ideas for increased knowledge. The objectives for the FIN board this year were 1. raising awareness for its global patient advocacy work by having a booth at the exhibition hall for the first time, 2. holding face-to-face meetings with different pharmaceutical companies (some for the first time) to build a "bigger picture" of the field of therapy and therapeutic advances possibly available in the future, 3. liaising with other patient groups, global and regional, to pinpoint and discuss common advances, challenges, strategies and finally 4. participating in the general discussion about the most significant unmet needs for patients both world-wide as well as in the specific countries FIN represents.



The FIN booth in the exhibition hall was a success and it helped provide the board with lots of new contacts among the attendees. Being present at the booth the board members were easily available for the audience to find. The leaflets about the Fabry Findings –series seemed very popular and were the topic and starting point of many interesting discussions. Despite the high number of meetings the FIN board members were able to divide and take in most of what was available at the symposium including: interesting presentations and posters about scientific research from across the globe.

Our take home messages from World are similar to the ones from last year. Research on Fabry disease is making many advances. For instance in the field of gene therapy there are different vector types being researched. Some research is at clinical stage and some pre-clinical. After beginning in Canada, trials have now begun in Europe, the United States and Australia. More information on the ongoing trials can be found on [our website](#), clinicaltrials.gov direct links for the [Avrobio](#), [Freeline](#) and [Sangamo](#) trials. One has to keep in mind, however, that clinical trials are a slow process where the patients' safety comes first and efficacy second so patience is required. There are also many more "traditional" therapies available and new ones advancing which provide the patients help in their everyday lives.



One of the other topics of great interest is the research on Fabry in females. It is quite commonly acknowledged nowadays that Fabry disease is much more variable in female patients. It was suggested that women should be tested for lyso GL3 as well as enzyme activity in order to get more accurate results on the severity of their condition. There still seems to exist a number of unmet needs in discussing the female patients' disease because of its heterogeneity.

On top of the vitamin D deficiency for Fabry patients that we've known about for years, Fabry patients seem to also suffer from vitamin B12 deficiency. We feel that this area is definitely something that needs more research. What is the reason for this? Is this one of the underlying reasons for the Fabry fatigue? What about calcium levels, especially for women, in order to protect them from osteoporosis? And finally, what would help? Are there any ideas regarding taking vitamin supplements if the body cannot absorb and utilize them?

One of the other topics that we would like to see much more research on is the topic of neurologic and psychological issues with Fabry disease. Topics affecting the patients are e.g. chronic disease burden and stigma, guilt, depression, sleep disturbances and problems with sexual functions, not to mention reproductive issues for some Fabry patients. One can only hope that with the new therapies for Fabry disease we gain more knowledge on stroke risk, white matter disease burden, peripheral nerve involvement including pain and dysautonomic functions.

There was some discussion on Fabry pain. Pain in Fabry disease is difficult to measure and evaluate because there are so many different tools being used and obviously because pain is always extremely subjective and individual pain threshold differs from individual to individual. It was stated at one of the satellite symposiums that late onset Fabry patients don't suffer from neuropathic pain. We feel this is something that should be looked into more closely because there seem to be late onset patients with a lot of pain in our societies.

One of the current topics of interest are different diets for Fabry patients and their impact on the gastrointestinal issues. Should there be dietary recommendations for Fabry patients? One study suggests that male patients should increase water consumption to reduce gastrointestinal symptom frequency. The impact of Fabry on gastro-intestinal symptoms is acknowledged, but whether diet can make a difference or not is a question we know many patients would like answered. These symptoms can vary widely from person to person and even at different times for a single person. It can be very subjective and is it possible to show a link between diet and symptoms? If diet changes don't make much difference can treatment? These are areas we believe more research is needed.

All in all, attending the WORLDsymposium™ proved yet again to be very beneficial for the FIN board. Even though a lot of the scientific content is difficult to understand, it is of utmost importance to know which topics are being researched and which unmet needs are yet to be addressed by scientists. A Fabry patient is often the best expert in his/her own disease symptoms and it is our common task to make the patients' voice heard worldwide.



FIN is pleased to introduce you the talented Barbara Mamatis from Greece. Barbara is a certified Therapeutic Refined Pilates instructor and a rare patient as well. She will explain the Pilates method to us all, as this an excellent type of exercise for Fabry patients. Enjoy!

Therapeutic Pilates advice from Barbara

Pilates is a conscious, intelligent way of exercising invented by German physical trainer Joseph Pilates and targets mostly the core but works other areas of the body as well.

It's a low-impact exercise for the joints that aims to strengthen muscles resulting in improved posture, alignment, flexibility, coordination, balance and range of motion. It can also help alleviate pain and discomfort becoming nowadays a very popular approach in most physiotherapy and rehabilitation centers.



As the Pilates technique has been over the years commercialized and several approaches have been created, resulting in losing its authenticity, it's important to bear in mind that it should place great emphasis on breathing, lengthening, visualising and on the fine details of how to perform each movement in a slow, precise manner.

That is why in a true Pilates class beginners should be taught by a certified Pilates instructor how to utilize the true power of their breathing which accompanies every movement including all postural fundamentals in order to truly, deeply benefit from this method and above all train in a safe way.

In Pilates, depending on each participant's level and needs, experienced Pilates instructors give customized and alternative exercises which are performed within a small group or a private lesson with the use of mats, equipment or both. It's therefore a great holistic, therapeutic approach not only for strong athletes, sensitive pregnant women, seniors but also for a variety of more demanding and complicated conditions such as Fabry and other related metabolic diseases with muscular, skeletal, neurological and cardiopulmonary dysfunctions.

The low-impact, static and dynamic stretching nature of Pilates combined with a variety of different level-exercises and rich breaths, can help Fabry patients who have a decreased ability to sweat and cannot tolerate strenuous types of exercise, train in a controlled, moderate manner either by standing, sitting or lying down in the comfort of their own place or at a specialized studio and eventually find relief in symptoms such as burning or tingling pain in their hands and feet, dizziness, headaches, heat intolerance and general fatigue.

As Pilates is a body-mind approach it's a great way for Fabry patients to decrease their overall stress levels caused not only by the complexity and the high demands of the disease but also by it's intense pain crises. Pilates can help Fabry patients built a stronger, healthier body resulting in the increase of their self-confidence as it helps them function and do everything better by moving in a "lighter", balanced, conscious and more efficient way.

If this sounds like your type of exercise, make sure you find an experienced Pilates practitioner you trust who can understand your needs, wear a form-fitting outfit, a pair of socks, avoid big meals and off you go to your customized body-mind routine!

Barbara Mamatis



Barbara is a certified Therapeutic Refined Pilates instructor, graduate of Jenny Colebourne's Illium Center of Light, and an elected member of the B.O.D. of the Greek Lysosomal Association "Solidarity".

Born with MPS I Scheie, Barbara faced the need from a young age to train her body and listen to its needs, resulting in 2017 in graduating with honors from her 3 year professional training in Pilates after completing a minimum of 1000 hours of training and teaching (mat work and equipment).

Barbara by now has almost a 10 year experience with the Pilates method and teaches through online and face to face trainings part time therapeutic Pilates to small groups, gives private sessions in Greek and English and gives therapeutic Pilates Workshops.



Saturday 29 February was the 13th edition of Rare Disease Day coordinated by Eurordis. The campaign continues to grow each year since it was first launched in 2008. Thousands of events were organised over 100 countries and regions to mark the occasion.

On Rare Disease Day we called for increased equity for people living with a rare diseases and their families, including greater social inclusion and equity in access to diagnosis, treatment and care.

The community joined together across borders and across diseases to show that

Rare is many. Rare is strong. Rare is proud!

Here are some of the amazing activities our members organised :



Fabry Australia continued the 'Be Rare Be YOU' campaign for 2020. Many more individuals, families, other rare disease groups, companies, schools and businesses got behind this years' campaign raising awareness for rare disease day and all funds raised from sales of the tattoos went to Fabry Australia.

"Thank you to Julia and Donna of the Canadian Fabry Association for allowing Fabry Australia to collaborate and roll out the campaign in Australia" says Megan Fookes, Director Fabry Australia

The Hungarian Fabry Foundation attended the Hungarian Rare Disease Day event where they shared the flyers they developed. This flyer aims to raise awareness and to inform the public about Fabry Disease.

Have a look at the English version of the flyer [here](#)



As Poland received reimbursement in September 2019, their activities this year are focused on helping all Fabry patients in Poland to get diagnosed and start treatment. They would like to share a photo from the public announcement of reimbursement for Fabry treatment. Additionally, on Rare Disease Day 2020 they, as an Association, received an award from the Hungarian Chapter of the Rare Disease Day for their work and effort during the reimbursement process.



South Africa had a few interesting things taking place.

On Social Media they ran a #ForFactsSake media campaign where they asked patients to reframe rare with a personal video sharing a daily fact. This had tremendous reach, with over 30,000 shares of the daily videos, which was great (They are available on their [YouTube Channel](#))

They also had quite a successful media engagement campaign, which resulted in 43 media interviews across digital, radio, print and tv media. The reach of this was 5 million south Africans.

Their events included a blood drive at a busy centre in Cape Town, as well as their #DenimWalk by night event, which put the spotlight on rare diseases. This event was held in Johannesburg, and was thoroughly enjoyed by the patient community as well as members of the public.

They have created a wrap up video of their events, [watch it here](#)

Kelly Du Plessis, CEO & patient advocate says: "Well done to all for the events and awareness, as exhausting as February can be for our community, to see the local and global communities come together, makes it all worth it"



In Italy, the Colosseum and the Tower of Pisa were illuminated with the colours of the Rare Disease Day on February 29th, 2020.

We are all RARE, embrace it. The CFA ran their BE RARE BE YOU Campaign again this year for RDD and proud to report the positive response. School groups, sports teams, politicians, and 1000's of others were educated, and shared the message of empowerment.

"What a powerful day!" says Julia Alton, Executive Director Canadian Fabry Association



FABRY FINDINGS

Scientific publications that are translated into lay language made available to
the Fabry Community;

A new article will be announced in the FIN newsletter.

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



[Click here](http://www.fabrynetwork.org/fabry-findings/) to read the third issue of Fabry Findings

The Fabry Findings articles are published on the FIN website

<http://www.fabrynetwork.org/fabry-findings/>

We encourage you to translate

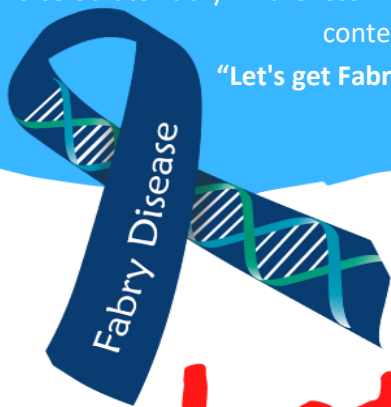
Each year, during April, we aim to increase the awareness of Fabry Disease by increasing understanding of this rare condition and impact of living with Fabry Disease as a patient and family.

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 again organising a contest:

“Let's get Fabry Creative”



Let's get Fabry creative

During Fabry Awareness Month, send us a picture or a video of your artwork, poem, video, sculpture, drawing, painting, song, dance or any creative project.

This is an opportunity to creatively express what it means to live with a rare disease and to share your story with the Fabry community and beyond.

The contest is open to all nationalities and ages from the Fabry community!

A contest without a prize would not be a contest: a gift card of €100 will be sent to the winner!

The winner will be announced and published in our June newsletter!

Send your picture or video to: info@fabrynetwork.org before April 30th, 2020

FABRY AWARENESS MONTH
APRIL 2020





FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Stefania Tobaldini from Associazione Italiana Anderson- Fabry Onlus



When did you join your national patient association?

I joined the Fabry Association in 2004. At that time in Italy there were two national patient associations. This was a very strange thing for a rare disease such as Fabry!

What was the reason for joining?

Initially I only attended the annual meetings organized by one of the two associations. At the annual meetings it was possible to receive up-to-date scientific information from the experts and it was also possible to meet other patients. I attended these meetings because I wanted information about treatments and the complications tied up to the disease since I also had a small child. I wasn't very active but just participated to listen. Then, with time, I decided to try and become a member of the Board in order to do something useful not only for me but for everybody. As the years went by, the two associations became close and started organizing yearly meetings together with the patients. We understood that united we could be stronger and feel less alone, so in 2016 we founded one association which is AIAF Onlus. I became president of the new association that year.

What is the vision and mission of your association?

Our mission is to support the Fabry patients and their families, helping them to overcome the isolation felt especially when the disease is first diagnosed and to help them stay in contact with the community of patients. The Association, together with its Scientific Committee works to promote the best practice for the diagnosis and treatment of the disease. Finally, A.I.A.F. Onlus helps the Fabry patients to face the problems of their disease. A.I.A.F. Onlus believes that each Fabry patient must be quickly diagnosed, monitored and treated so as to have the best possible quality of life.

How many members are there?

We are in contact with many, many Italian patients by email, Facebook (we have 400 people enrolled on Facebook), although not everybody registers with the Association. Since 2016 AIAF now has 170 members, but I believe we could reach out to more members by trying to help the patients understand the importance of belonging to the Association, so that we can be stronger and better heard with the Institutions.

What do you consider to be the major achievements or activities you are proud of?

First of all, uniting two associations into one association was a very important step for which we are very proud: we have overcome our differences and together have become stronger. We have established a very good collaboration with our Scientific Committee (made up of experts on Fabry Disease from all of Italy) and this has allowed us to contribute to scientific research and finance a project about the dosage of the Lyso Gb-3, which has just been concluded. In a few months there will be a scientific publication about this topic. This would have never been possible without the collaboration of the experts and the Italian hospitals. In the last few years we have given the patients more chances to meet: aside from our annual national conference, each year we organize two meetings for experts and patients in the various Italian Regions. In this way the patients have the possibility to meet other patients and families from their own Region so as to feel less alone. One of the most important things we do at our meetings is to have the children and teenagers with Fabry Disease get together. We organize special activities of play and confrontation, with the help of psychologists. This meeting is a real success because on average of 170 people are present. The Fabry children have the chance to share thoughts, feelings, difficulties "amongst equals". In recent years the number of children participating has grown considerably, they've become friends and stay in touch with one another through the various social networks. They are always so happy to meet up again at our meetings. Since 2019 we have also given this possibility for our young people (18-25 yrs. of age) to exchange experiences amongst themselves at our meetings. They would like to organize an European meeting dedicated to young Fabry patients.

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Stefania Tobaldini

from Associazione Italiana Anderson-Fabry Onlus

Can you name some challenges that your association is currently facing?

We worked to have the Anderson-Fabry disease included as obligatory in the new born screening, writing letters of appeal to the Institutions. In Italy new born screening already exists for 40 metabolic diseases, but within the end of 2020, also thanks to our work, new born screening for lysosomal disorders will become obligatory. It will finally be possible to have a precocious diagnosis for Fabry disease. Another challenge we are taking forward concerns home therapy for patients on treatment with ERT. Unfortunately, in Italy there still 5 Regions who refuse to allow home therapy and this is a cause of discrimination between patients. We would like all patients to be entitled to choose home therapy if they wish to do so. For this reason, we have started working together with other patient associations (Gaucher, Mucopolysaccharidosis and Pompe). United, we hope the Institutions will pay more attention to us. Unfortunately the Institutions do not understand the problems patients who work face: having to go to hospital for treatment means losing 26 working days per year and often the patients need to take days from their holidays to go to the hospital otherwise they are at risk of losing their jobs because of all the days lost from work. For many patients treatment at times can become a big problem and this isn't fair.



Can you name some future goals or plans

First of all, we are working on a project to enable better care of patients, in order to reduce the number of times patients have to go to the hospital for periodic monitoring. The objective is to collaborate with the hospitals to be able to concentrate all the exams and tests all in one day. Secondly, we would like the Fabry patients be given an invalid recognition because of their disease. The Italian law allows patients with very severe diseases to have 3 days off from work for treatment (3 days each month paid for). Unfortunately, Fabry disease is not included in the list of diseases entitled to this recognition. Finally, psychological support is very important for Fabry patients. It cannot just be limited to the moment of diagnosis, but should be available throughout the various phases of the patient's life. Not all patients need it, but it is important that this possibility is available when the patient feels the need for it. Hospitals should have psychological support for all patients with a rare and/or chronic illness.

Has your association had issues with the national health system or insurance problems?

I must say that we are quite lucky here in Italy because our Health System offers assistance and treatments free of charge to all Italian patients and therefore also Fabry patients. A big problem is represented by the Regional management of health: this inevitably creates differences between patients because each Region is different. This is why some Regions allow home therapy and others do not.

How would you describe the current treatment situation in your country?

Presently in Italy, there are 2 ERT treatments and 1 oral treatment, all registered. Some large hospitals are conducting experiments with new drugs. This means that in a few years we will have more treatments available for the patients. A screening is being carried out to select patients who could be suitable for gene therapy. It's still a long and winding road, but we are optimistic.

What are the major issues with Fabry disease in your country?

The main issue is the diagnosis, which still takes a long time and thus worsens the quality of life of the patients. Furthermore, patients with Fabry disease are often in the hospital for treatment (if they can't do it at home) and for their check-ups. It is important that the hospitals are well organized in order to concentrate the visits in just one day so as not to create problems for the patient, but unfortunately not all hospitals are so well organized. Having to go to the hospital many times to carry out exams and check-ups, means that the patients always feels sick. The more you are in the hospital the sicker you feel. We would rather not feel sick. The Healthcare System should also pay greater attention to the transition of patients from paediatric to adulthood. It is a delicate transformation step that is often underestimated. It should be managed gradually.

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Stefania Tobaldini from Associazione Italiana Anderson-Fabry Onlus

How would you further raise global awareness for Fabry disease?

I think that only if you try and imagine what it is like to have a rare/chronic disease, you could, perhaps, begin to understand what it's like. According to me it is important to talk about the daily lives of the patients (even without mentioning names). People living with Fabry disease are often not understood: these patients live with asthenia, pain, stomach cramps, tachycardias, kidney problems and sometimes depression. All of this is invisible, but it is real. Sometimes society does not believe we are really sick (especially at work).

What are your suggestions for future projects for FIN?

Two things:

- *It would be great to enable the young Fabry patients to meet up with each other on a European basis so that all their wishes, their needs and their hopes could emerge. Young people are our future.*
- *It might be useful to organize webinars to share up-to-date information on the latest scientific reports (clinical studies, scientific publications, new drugs, etc.)*

What would you like to explain to other people / doctors / nurses / decisionmakers about Fabry disease?

A person living with a rare disease, such as Fabry, will have a life conditioned by treatments, medical visits and all the difficulties caused by the disease. We didn't ask to have this disease and we would like to live a life as "normal" as possible. This is why it is so important to listen to the needs of the patients in order to try and solve them.

What kind of information are we still missing about Fabry disease?

It could be useful that from time to time, with the help of the experts, scientific information from publications are translated into lay language so that we are able to understand that information better.

What is your most effective means of taking your mind off Fabry disease?

Each of us should have some kind of hobby. I love dogs and whenever I can, I spend time outside with my doggy, Toby. I also love cultivating flowers: during the summer my house is an explosion of coloured flowers, which I care for personally. Everybody admires my flowers.

Is there anything else you would like to add?

Thank you FIN!

Thank you for your time!



Stefania Tobaldini hands the pen to Mary Pavlou from the Greek Lysosomal Association

Follow A.I.A.F Onlus on



Dear Fabry Community:

Sigilon Therapeutics™ was honored to have met with Fabry International Network's leadership board at WORLDSymposium™ this year. During this time, we listened to stories of living with and caring for those with Fabry disease. We learned about the day to day difficulties of managing this chronic condition and what the current treatment options are. We realized how strong of a support system FIN is to so many diagnosed families across the world and are in awe of how tight knit your community is. Now, we want to introduce Sigilon to you.

Who is Sigilon?

Sigilon Therapeutics™ is a pre-clinical stage company based in Cambridge, MA, USA. We are developing cell therapies for lysosomal disorders such as Fabry and other rare genetic disorders through our Shielded Living Therapeutics™ platform.

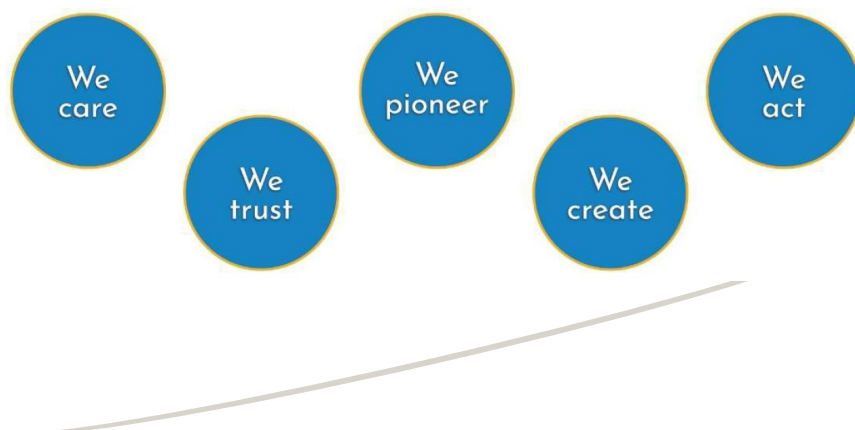
What is the Shielded Living Therapeutics™ Platform?

Sigilon's Shielded Living Therapeutics™ platform is comprised of two elements. First, we engineer human cell lines to produce high levels of the protein we want. For example, in developing a potential treatment for Fabry disease, the cells would produce a-Gal A. Second, we encapsulate these cells with our breakthrough Afibromer™ matrix that is designed to shield the cells from the body's immune response (e.g., rejection and fibrosis). Finally, we place the cells into the body to provide potential functional cures for chronic diseases.

Our Core Values:

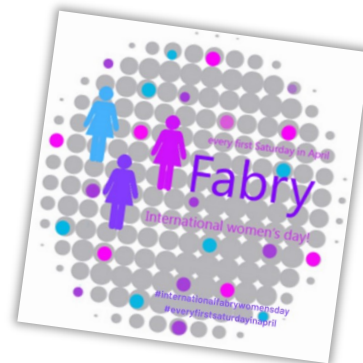
We pride ourselves in our core values and put patients and caregivers at the center of everything we do. We are working hard every day to build your trust and to bring new therapies to the clinic.

Please visit www.sigilon.com to learn more about our technology and platform. We hope to have the opportunity to meet you soon!



International Fabry Women's day

Every first Saturday in April



In 2013, the Dutch patient organisation FSIGN (Fabry Support & Information Group Netherlands) declared every first Saturday of April International Fabry Women's day.

A number of countries have already followed their example over the years: America, Canada, Belgium and Hungary.

The first Dutch Fabry Women's Day was organised in **2005**, and the impact on the female members was huge.

The International Fabry Women's day was established to raise awareness about women and girls affected by Fabry Disease and how females with Fabry are not just "carriers".

For FSIGN, this was one of the main reasons to organize an annual Fabry women's day. There is now a growing community of attendees and after a few years they got to know each other better and better and shared each other's joys and sorrows. They comfort each other after bad results and support each other in difficult times.. This is invaluable! Some of the FSIGN board members participated in a workshop that addressed the topic 'how valuable is contact with fellow sufferers?' Two women who wrote a thesis about this topic presented at the workshop. The outcome was both special and logical:

Peer support is a valuable addition to professional help. Healthcare professionals should recommend it more often because it works!

In the Netherlands they are lucky that they receive funding from the government as a patient organisation - so they can easily organise a meeting like this.

They started off simple: having a coffee or lunch together, organising a round table
When the membership gets more and more enthusiastic, do something creative, provide a workshop.
And you will notice more and more people will hook up, that's how FSIGN reached more young adults.

Everyone is welcome: Fabry-affected women, mothers, sisters, female friends, female partners of Fabry affected.
There is only one requirement: you must be a member of the patient organisation.

As mentioned in last year's newsletter, we celebrate this special day every year on the first Saturday of April and the need for this awareness was much greater than we ever imagined.

Purpose of the international women's day:

- ◆ peer support
- ◆ finally understanding
- ◆ exchange of experiences
- ◆ inventory of needs
- ◆ enjoying and sharing each other's stories

We encourage you to share your activities and projects on social media whilst using the following hashtags:

#Internationalfabrywomensday and **#everyfirstsaturdayinapril**

Have a beautiful Fabry Women's Day!

PS: Use the Facebook Frame on International Fabry Women's Day!



Fabry Outcome Survey Annual Report 2018

Based on data collected within FOS from 5 October 2001 to 7 January 2019

Welcome to the 2018 Annual Report of the Fabry Outcome Survey (FOS), which presents an overview of FOS as of 7 January 2019. As well as providing a summary of the activities of the FOS Steering Committee (SC) during 2018, this report describes the demographics of patients enrolled in the registry and gives details of the data analyses currently in development. FOS (ClinicalTrials.gov identifier: NCT03289065) is a large, global, multicentre, longitudinal, observational registry, sponsored by Takeda, for patients with Fabry disease. Since its initiation in 2001, FOS has collected real-world data from patients treated with agalsidase alfa and also from untreated patients. These data have contributed to our understanding of the natural history of the disease and the long-term safety and effectiveness of treatment with agalsidase alfa. Enrolment of patients who have received another approved treatment for Fabry disease (agalsidase beta or migalastat) started in 2016, and it is hoped that the collection of data from these patients will reveal further insights into Fabry disease and its management.

Key highlights from FOS, as of January 2019

- ◆ As of January 2019, there were 3855 patients enrolled in FOS, which is an increase of 10% compared with January 2018. Patients have now been enrolled at 142 centres in 26 countries (Figure 1).
- ◆ FOS continues to contain more females than males; in January 2019, 58% of the patient population were female. Children (defined as < 18 years old at enrolment) comprised 12% of the patient population.
- ◆ Overall, 2286 patients (59%) had received at least one approved therapy for Fabry disease: 2214 patients had received agalsidase alfa, 97 had received agalsidase beta and 81 had received migalastat (some patients had received more than one approved treatment). The relatively low numbers of patients who have been treated with agalsidase beta and migalastat may reflect the fact that FOS started enrolment of these patients in 2016, whereas patients treated with agalsidase alfa have been enrolled since registry initiation in 2001.
- ◆ During 2018, five posters based on FOS data were presented at three international scientific congresses, in North America, Asia and Australia.

Takeda would like to thank all of the patients and families who have participated in FOS, and also the physicians and their staff who have collected data for the registry.

[Click here](#) for the full report



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.



The next FIN Newsletter will be out at the end of June 2020. Please send your articles and contributions by email to Charlotte no later than June 10th, 2020. We look forward to publishing and sharing your latest news with our International Fabry Community!



The STAAR Study is Recruiting Patients Now

Fabry disease is caused by shortage of an enzyme called alpha-galactosidase A (α -Gal A). This shortage happens when the GLA gene, which provides the body with instructions for making α -Gal A, is not working correctly. A new phase I/II clinical study has been designed to investigate the safety and tolerability of an investigational gene therapy called ST-920 to treat Fabry disease. ST-920 aims to deliver a healthy copy of the GLA gene to the liver. It is hoped that the liver should then be able to produce the α -Gal A enzyme and secrete it via the blood stream to the rest of the body.



The STAAR Study is now recruiting men aged 18 or over who have been diagnosed with Fabry disease. Visit the STAAR Study website (www.staarclinicalstudy.com) where you can see if you qualify. You can also discuss this further with the study team, who are more than happy to help. Contact details: clinicaltrials@sangamo.com

www.staarclinicalstudy.com



FABRY
International Network

FACILITATE | SUPPORT | ENABLE

www.fabrynetwork.org



Fabry International Network

Registered address Alice Nahonlaan 7, 9120 Melsele - BE
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Charity Registration Number 04080030 The Chamber of Commerce Drenthe The Netherlands



Newsletter



June 2020

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A word from the President:

Dear All



We proudly present the latest FIN newsletter.

On June 10th, we held our annual general members meeting, although we would have loved to meet in person, we were very happy to meet many of you online.

In 2020 (and beyond) FIN will focus on projects and initiatives that bring patients together. Of course, this is part of FIN's mission. We feel now more than ever, there is a need for patients to be part of a wider community. We aim to do so through sharing best practices, facilitating connections between Fabry patients to help combat isolation and supporting local initiatives. In case you missed the AGM, we have included most of the information in this newsletter.

The new dates for the FIN Expert meeting in 2021 are now confirmed. Make sure you save the date!

In order to make this newsletter as informative as possible we always welcome news and information from you as a caregiver, patient, patient organization and patient representative. Please send it to info@fabrynetwork.org

Stay safe and healthy!

Lut, FIN president

THE 10th EUROPEAN CONFERENCE ON RARE DISEASES & ORPHAN PRODUCTS



ONLINE on 14-15 May **2020**

THE JOURNEY OF LIVING WITH A RARE DISEASE IN 2030

Anna Meriluoto

In mid-May The European Organisation for Rare Diseases *Eurordis* organized its annual conference on rare diseases and orphan products. The conference was originally planned to take place in Stockholm, Sweden but due to the Covid-19 pandemic it was swiftly moved to be held completely online. It was a magnificent effort from Eurordis with more than 1500 participants from 57 countries attending the 2-day event and yet things were running very smoothly. The online platform was clear and simple to use and provided a lot of easy-access information and several interactive networking opportunities.

The ECRD is recognized globally as the largest, patient-led rare disease event in which collaborative dialogue, learning and conversation takes place, forming the groundwork to shape future rare disease policies. The backbone of the conference agenda was the Rare2030 –project led by Eurordis. Rare2030 is a foresight study that gathers the input of a large group of patients, practitioners and key opinion leaders to propose policy recommendations that will lead to improved policy and a better future for people living with a rare disease in Europe (www.rare2030.eu). It is of utmost importance for us all to identify the major trends and drivers that shape the future for rare diseases.

What are the major trends in the field of rare diseases in Europe then? First of all there is a rise of the pan-European multi-stakeholder networks to advance diagnostics, treatment and care. There is an unprecedented potential in the European reference networks that will definitely shape the future of rare diseases. The success will, however, depend on the activity of the member states as well as different stakeholders. The patients will get an earlier diagnosis, newborn, prenatal and pre-symptomatic, so the numbers of rare disease patients will grow. That will make the member states face new kinds of issues and problems, e.g. add strains on the health care budgets and the emergence of new care delivery models. New healthcare technologies will provide many beneficial developments such as telemedicine, virtual consultations and monitoring diseases with different health apps. However, access to new digital technologies will be slow and limited. They will also bring us new challenges and questions about data governance, ownership of our own data and what happens to it once it's been fed into the new technologies.

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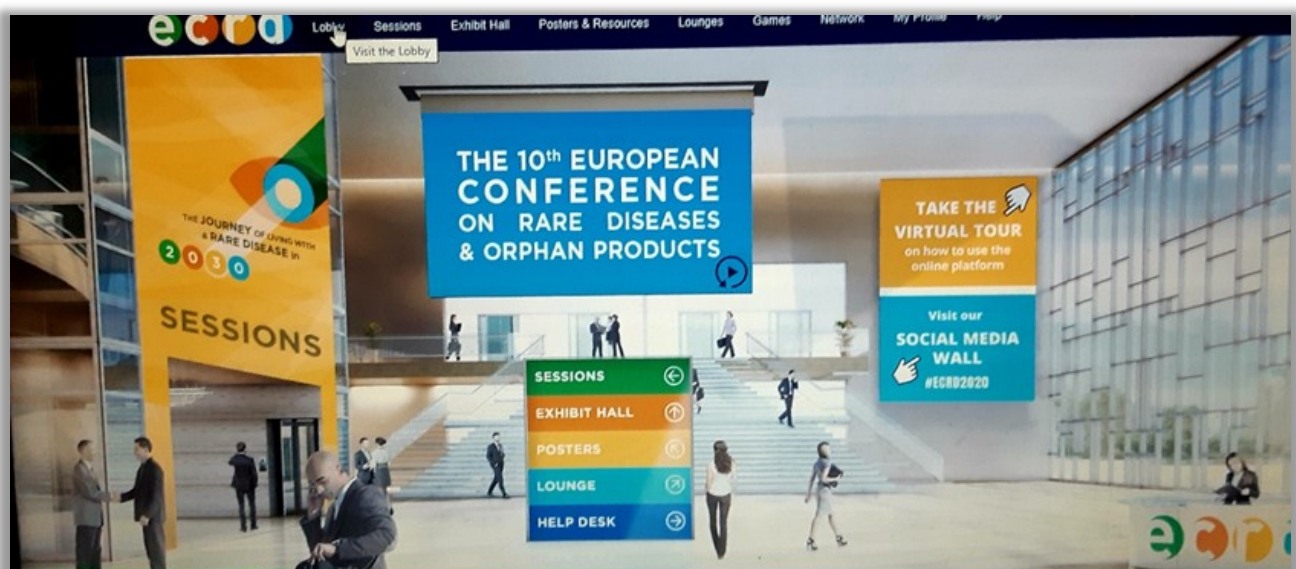
The future will bring us a change in the demographics of rare disease patients as all European countries are faced with a rapidly aging population. That will lead to greater co-morbidities and complexities including those for rare disease patients. This will strain the member states financially and we might see a greater variation in access to treatments and care resulting in more inequality for people with rare diseases. This might also lead to a system of prioritization. Keeping in mind that only 5% of rare diseases have an existing treatment today, we need to work towards creating a paradigm of inclusion, solidarity and fairness. We also need to make sure that the rare disease patients and patient groups are empowered and fully involved in all aspects of their disease management.

What should be the take home –messages from this conference? There are a lot of innovations in healthcare which promise positive develop for a diagnosis. Patients provide even more valuable input to all public sectors. Transformations in care for rare diseases provide better standards for medical and social care. Patients have an opportunity to receive better therapeutic development.

We are witnessing the beginning of the era of digital health technology to improve standards of care. And we have an action plan of accessible, affordable and available treatment of people with rare diseases by 2030.

As Benjamin Franklin pointed out: "If you fail to plan, you plan to fail." I will finish with a quote from the Eurordis Chief Executive Officer Yann Le Cam: "We are preparing for the next decade. We are bringing forward solutions, created at the margins of society but becoming more mainstream. Rare2030 was the backbone of ECRD 2020. The outcome of the conference is a new impetus to initiate a new legislative framework for rare diseases."

The recording of all the conference sessions is available online until May 2021 for a small attendance fee. Find out more at <https://www.rare-diseases.eu/ecrd2020-all-sessions-available-one-year/> If you are interested in viewing the conference posters, they are available at <https://www.rare-diseases.eu/posters/>





FIN EXPERT MEETING 2021
SAVE THE DATE

April 23rd - April 25th, 2021
Amsterdam, The Netherlands

The meeting will take place at the [Steigenberger Hotel](#) near Schiphol Amsterdam

ERT HOME INFUSION FROM MY PERSPECTIVE

Erica van de Mheen

Hi, I'm a 63-year-old Dutch woman diagnosed with classical Fabry disease. I have two daughters who are also Fabry affected and we live in the North of the Netherlands.

In January 2003 I started ERT to make sure that no more damage was done by Fabry disease. At the beginning I already made the decision to do it at home and do the infusion myself. I was so happy that this was possible in the Netherlands already then.



Frisian wooden shoes for a child, on my fence door

Before starting the home infusions the physicians had to be sure, of course, that there would be no infusion reactions and that you would be doing fine after the infusion so you always need to have your first infusions at the hospital. You also have to learn how to apply the infusion materials and to use the needle properly.

(Note: in the Netherlands it is possible to do the infusion completely by yourself, without any help from a trained nurse, but with help from a partner or a friend!)

It is very important that your workplace is clean and safe. At my home, we do it at the dining table, with a nice white clean towel beneath. I prepare the infusion myself and I stick myself at the back of my hand with a small butterfly needle with help of a friend or my daughter to fix the IV. My youngest daughter started at sixteen and she also took her infusion at home, sometimes on the same day.



My daughter puncturing herself.



The nurse uses an IV-needle



Completely fixed so I can use my hand

Since 2015 I live alone, my daughters (36 and 28 now) have their own houses and since then a nurse comes to my home to help me with the infusion.

I get all the required materials delivered at home. My medication for two infusions is also delivered once a month. The medication needs to be kept in the refrigerator to stay cool.

As a member of the Dutch Patient Organisation you receive a mobile IV-pole free of charge when you start ERT. FSIGN has done this since 2003 and it is much appreciated, because insurance does not provide this needed instrument for free. You can take this with you everywhere, even camping or on a boat trip. I like to sit in the garden or go to my sisters' or my daughters' home so they can assist me when the nurse is on vacation or otherwise not available. Yes.... all of this is possible over here.



It is my opinion that there are only positive sides to home infusion:

- You can do it at home in your own environment. You don't have to go to a hospital, which might not be near your home. Some people have to drive far and for a long time.
- You can sit on your couch, watch a movie or your favourite series, sit at the table playing a board game with friends or family, sit in your garden when the weather is beautiful and so on.
- There is no need for a hospital bed, so you don't feel like a patient.
- You have no extra cost e.g. fuel and/or use of car, bus, train etc. and parking money.
- You can choose your own preferred time to infuse, even in the evening, during the weekend or some other time when the hospital is not open for day care....very important!
- Without the hospital visit the cost for home infusion versus day care is much lower. In The Netherlands, the treatment cost is approximately 8 times lower than getting treatment in a hospital, even with a nurse present at home.
- No need to worry about hospital bacteria.
- You don't have to miss work, school or other important things, which you might if you had to go to the hospital every two weeks!

As you can see.....I am a really big **FAN** of home infusion!

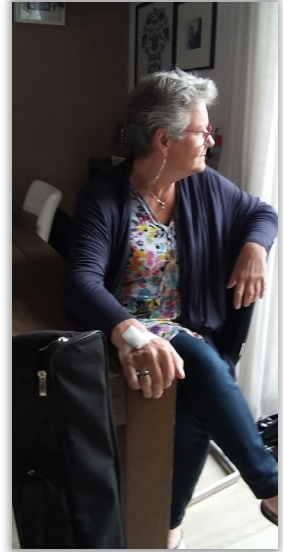
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In my garden



On the sofa



All done

I would like to promote the option of home treatment for all Fabry patients worldwide and let everyone know how much it improves my quality of life. We are all aware that **Quality of Life** is so important for people living with a chronic disease.

We already have to sacrifice so much, lets make this one thing easier for all of us.

I encourage you to translate this report into your own language to share with your community as an example of what can be achieved.

Always willing to provide more info!

Yours sincerely

Erica van de Mheen
treasurer@fabrynetwork.org

FIN AWARD



FIN wants to encourage the membership to organise new activities and initiate projects by contributing financially and giving a platform to share with the wider community.

FIN will award a patient (association) led initiative that informs and educates about Fabry and helps raise awareness by providing a financial grant*

The winning project will be announced at the Expert meeting in 2021

The activity or initiative should be:

- ◇ Educational and raising awareness
- ◇ Bringing patients together (face to face or virtually)
 - ◇ Providing peer support

We encourage all out of the box ideas!

[Download the application form here](#)

**Terms & Conditions are stated in the application form*

COVID-19 and Fabry Disease

Since we wanted to keep our community informed during these uncertain times, we hosted our very first webinar in April, which was very successful. A second webinar took place in May. We were very happy to have our expert panel which consisted of Prof Germain, Prof Eyskens, Dr Ali and Dr Ortiz presenting valuable information to our members.

The recordings are available on our [YouTube channel](#).



Dr. Nadia Ali, Ph.D Emory University, USA

[Click here to listen to the presentation](#)



Prof. dr. Francois Eyskens UZA, Belgium

[Click here to listen to the presentation](#)



Prof. Alberto Ortiz MD, Ph.D, Health Research Institute of the Jiménez Díaz Foundation Madrid, Spain

[Click here to listen to the presentation](#)



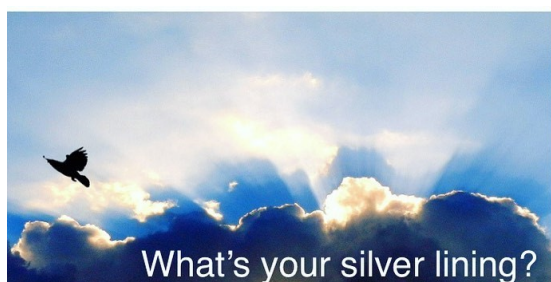
Prof. Dominique P. Germain, MD Ph.D National Center for Fabry disease, France

[Click here to listen to the Q&A](#)

Over the course of 2020 we will organise additional webinars with topics from the expert meeting program in close collaboration with Fabry experts. We will also focus on topics where we see patients as more than just Fabry.

COVID-19 and Fabry Disease

Many of our members organised webinars of their own to inform the community along with additional heart-warming initiatives .



Every cloud has a silver lining means that even the worst events or situations have some positive aspect. Sometimes it is hard to find the positive but it is at these times when it is most important to do so.

We were forced inside to look inward... we asked our community to send in their silver linings through COVID-19 and you can feel a shared sense of togetherness, strength, and resilience.



From making noise for front line workers, reinventing creativity, and taking time to slow down and be present. Adversity shows us what we're capable of and magnifies our strengths. [Watch the video here](#) to bring some inspiration to your day.—*Julia Alton*

A card with a heartwarming message

For the Dutch patient association, one of our members put together a special card, that we sent out to all our members as we were not able to meet face-to-face.

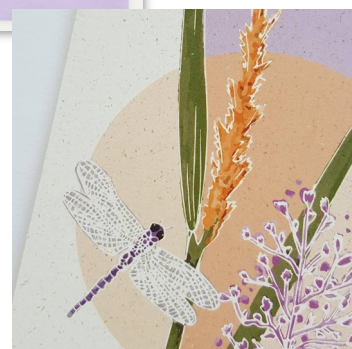
It is a design from her own collection 'Plukgeluk' (Choose luck) The colours are completely adapted in the colours of our own corporate identity.

The design on the back is completely handwritten and designed by Francine, just like the personal postage stamp, with a small piece of art on the address label, which is reflected on every lavender coloured envelope. This completes the image and creates a unique experience.

A special project for people with unusual challenges to encourage them. Printed on paper with a subtle fibre, stylish and environmentally friendly from Dutch soil, sent in an environmentally friendly envelope.

The Dutch text translates into English:
Wishing you lots of strength during this difficult time, we are thinking of you.

Given the responses, the gesture was much appreciated.
Small gesture, big impact - *Erica van de Mheen*



The COVID-19 health emergency was a very complex moment for all of us. Many of us had to spend a long time in solitary confinement without having contact with other people. Medical checks in hospitals had also been canceled. For this reason AIAF has created the "AIAF IN CONNECTION" project: a virtual space with professional support. It has been an opportunity for sharing and talking with patients and families involved in Anderson-Fabry disease to feel less alone and stay in touch with each other. Since April we have organized five webmeetings with patients.

We worked with a psychologist, a lawyer and the AIAF Scientific Committee in order to discuss patients' needs related to the COVID-19 emergency, provided information about the absence from work due to COVID-19 risks and updated them on the scientific news related to Fabry disease and answer their questions. Thanks to this project AIAF has been in contact with about 40 Italian families during the COVID-19 pandemic.



During the COVID-19 pandemic, AIAF also made recommendations for the therapy management available to patients in collaboration with the scientific committee.

[The Italian version is available here](#)

Stefania Tobaldini, President of AIAF Onlus



Fabry Suisse organised a webinar for their members as they could not meet in person.

Mrs. Faiza Kaddour-Gysi Specialist Psychologist for Psychotherapy FSP, Solievo.net - interdisciplinary centre for mental health, spoke about "Accepting the disease and dealing better with the disease". PD Dr. Albina Nowak, Senior Physician, Endocrinology Clinic Rare diseases, University Hospital Zurich presented "New horizons in therapy for Fabry disease" - *Beate Krenn*

On May 4, we started a bi-weekly cycle of conferences, in collaboration with Sanofi Genzyme, a cycle of virtual conferences on Lysosomal Storage Disorders. Webinars are open and free to all those interested and it can be followed online from any device and from any country.

The contents of each session will be led by different experts in these pathologies and will be focused on offering theoretical and practical content, always oriented to support the patients and families during the COVID-19 pandemic.

The topics discussed are of general interest related to lysosomal diseases as well as specific topics related to Fabry disease. After each conference there is time allowed for attendees to ask questions to the experts.

These sessions took place in May and the beginning of June

Session 1: May 18 *"Our strengths facing adversity"*, Eduardo Brignani, Professor in psychology. Reflections to help us get in touch with our capacity to start again, even in a complex and uncertain reality, to live each day as one new opportunity.

Session 2: June 4. *"Monitoring of lysosomal diseases and treatments in the Covid-19 situation"*, Dra. Mireia de Toro, neuropaediatrician at the Vall d'Hebrón Hospital, Barcelona. *"Home therapy"* Javier Salguero, director of the Ashfield Healthcare Patient Unit.

Session 3—: June 17: labour related topics. Lorenzo Pérez, Lawyer specialized in labour issues. The topics of the following sessions are to be determined. Suggestions made, are being evaluated.

Session 4 will be on 23th of July, 7PM CET, the importance of the family tree in Fabry disease will be presented by Dr. Roberto Barriales from the Inherited Cardiovascular Disease Unit, A Coruña Hospital.

Check the website www.mpsesp.org for the recordings of the sessions

We encourage all of you to participate — *Jordi Cruz*

In Belgium, care packages were sent to all of the members.

The packages consisted of a mouth mask and a small bottle of hand sanitizer. Our organization contacted all of the members directly by phone. We wanted to make sure everyone of our members were safe and felt supported during this difficult time. - *Lut De Baere*



Thank you for all your submissions for our
Let's get Fabry Creative contest!

We enjoyed viewing and reading your beautiful
poems, videos, drawings, stories, paintings etc.

They truly were creative!



Let's get Fabry creative

We are very happy to
announce the winner of the
contest:

Geonwoo Kim

Congratulations!

Geonwoo Kim wrote
a beautiful poem.

FABRY AWARENESS MONTH
APRIL 2020



Winner Let's get Fabry Creative Contest



길고 긴 겨울 밤

잠 못 이루고 뒤척이다 깨어서

긴 밤 홀로 깨어있네

졸음에 겨워 설핏 잠들었다

이내 다시 깬다

졸다 깨다 반복하며 영영 오지 않을 것 같은 새벽을 기다린

다

길고 긴 겨울 지나

봄이 오고 쌓인 눈 녹듯

아픔도 봄 눈 녹 듯

스르르 녹아내리길...

Spring

In a long winter night

I'm awake after tossing and turning

I'm awake all night alone

Fell asleep for a while

Soon, I wake up again

I'm waiting for the dawn that never

seems to come, while dozing off

After passing through a long winter

Like spring comes and snow melts

Hope the pain goes away

Like snow melts in spring...

FABRY AWARENESS MONTH
APRIL 2020

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with

Mary Pavlou

The Panhellenic Association of
Patients & Friends with Lysosomal Diseases
"Solidarity"
Greece



When did you join your national patient association and what was the reason for joining?

I joined my national association in 2002 after I was diagnosed with Fabry disease. It was a difficult period for me and it was the only way to cope with the news of having a rare and serious disease. It was also a way to support my family as I learned more about Fabry through the association. Since then I'm a board member and I actively support and help patients. I currently am the Vice President for our organisation, along with the rest of the board members we organize activities and projects to support, educate and empower our members. Through several activities we try to raise awareness for lysosomal diseases.

What is the vision and mission of your association?

The Pan Hellenic Association of patients and friends with lysosomal diseases "The Solidarity" was founded in 1997 in Athens, Greece and has 120 members. It is a non-profit organization acting as the sole representative of patients suffering from Lysosomal Diseases. Its main goal is to inform patients, relatives and carriers in Greece about diagnosis and the monitoring procedures of Lysosomal storage Disorders. The association was founded with the dream that all patients of lysosomal diseases would have access to treatment, early diagnosis and reimbursement of treatment from public insurance. A registry for rare disease patients was at the table early on. Moreover our continuous goal is to empower all our members.

What do you consider to be the major achievements or activities you are proud of?

Due to our Association's long and deep involvement and commitment to our patients, we are recognized in Greece as one of the leading associations concerning Rare Diseases and Lysosomal one's in particular. We are also founding members of the first official EGA, now IGA, and a proud member of Fabry, Pompe and MPS International. My colleagues are very proud of having a representative in FIN.

We are co-founders of the Greek Alliance of Rare Diseases and a permanent member of EURORDIS by participating annually in their meetings. We are actively and publicly speaking about the rights of patients with rare diseases. We organize press conferences, meetings both with doctors, nurses and patients and we publish articles in newspapers about our problems. We try to engage our members to learn their disease, speak up to their doctors about their problems and to reach out to us should they face any problems.

...

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with

Mary Pavlou

The Panhellenic Association of Patients & Friends with Lysosomal Diseases "Solidarity" Greece

Last year to celebrate International Fabry, Pompe and MPS Day we attended an inclusive art theatre play where the actors were people with disabilities. The play was simultaneously explained for people with difficulties in both hearing and vision. In the past and for several years we provide psychological support for our members through a help line, organise face to face sessions for young adults and parents. We try to be present at all the major conferences to learn more about the diseases we are representing and travel all across Greece to support and get to know our members better.

Last but not least, the participation of our Chairman as the key speaker and representative of all suffering from Rare Diseases in a debate in 2017 in the Greek Parliament concerning the Greek centers of expertise of Rare Diseases and also a debate about the new established offices concerning the rights of patients in hospitals is another achievement we are proud of.

Can you name some challenges that your association is currently facing?

One of the biggest challenges that we are facing is that we are not getting any younger! We try hard to engage young patients and involve them more in activities we organize. We hope someday to step down and let them lead the association. It's always refreshing to have the next generation around. Our doctors are getting older too. This is why we believe that the centres of expertise can play an important role to continue development in care, early diagnosis, state of the art monitoring and the support in psychological and social level. On an operational aspect it is always difficult to work with limited budgets. Last but not least our deepest concern is to raise awareness for Lysosomal Storage Diseases from the general and scientific public as Lysosomal Disorders still remain in obscurity and need to be promoted in every possible way.

Can you name some future goals or plans?

We want to continue our current projects and add some more! The pandemic has changed our plans but we still hope we will organize yet another meeting to empower our members. We would also like to start to provide social and psychological support to them again.

How would you describe the current treatment situation in your country?

Currently two ERT's are reimbursed and one oral treatment is at the stage of becoming reimbursed in Greece. Our patients are treated in general hospitals and we don't have the option of home treatment. Moreover a Fabry centre of expertise needs to be established soon.

What are the major issues with Fabry disease in your country?

Time of diagnosis is still an issue. Most of our patients went through several doctors before they received their diagnosis. The lack of a Fabry expertise centre as well as the lack of an institutional framework for home treatment, I think, is an important issue we need to address soon .

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FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Mary Pavlou

The Panhellenic Association of Patients & Friends with Lysosomal Diseases "Solidarity" Greece

How would you further raise global awareness for Fabry disease?

That's an ongoing project we are all working hard to achieve. I believe that patient advocates and representatives should have a larger presence at scientific congresses. Rare diseases such as Fabry disease will never be well known if we don't speak about them publicly. Fabry should be diagnosed as early as possible. We have to educate the general public that there are diseases like Fabry that are not obvious at first sight. Meaning that we suffer from all kind of symptoms but because someone else is not able to see them that doesn't mean they are not there. Using social media platforms could be a useful tool to do so.

What are your suggestions for future projects for FIN?

As I'm a member of the FIN board and I know some of our exiting new projects I think it's better to wait and see!! With this opportunity I would like to say that working for FIN it's a once in a lifetime experience. We are a very strong team, we have passion for what we do and I'm so proud to be a part of it.

What kind of information are we still missing about Fabry disease?

I reckon that we still need to learn more about the social and psychological burden Fabry patients are facing. Those are aspects that we haven't addressed in the past. In addition the role that food can have in gastrointestinal problems and in general how the dietary habits can affect symptoms. I would also like to see more publications on female life events, and the transition from childhood to adulthood. I think it's a highly underestimated problem and needs to be addressed.

What is your most effective means of taking your mind off Fabry disease?

Reading is my most beloved thing to do to get my mind off Fabry. Talking to friends, swimming and a trip to the countryside is always a good idea!

Is there anything else you would like to add?

Don't be afraid to raise your voice for your rights. We all have the right to be living as equal members of the society with respect to our limitations. We all have dreams and we want them to come true.

Thank you for your time!



Mary Pavlou hands the pen to Ching-chang Hsu from the Taiwan Fabry Disease Patients Association



Anne Grimsbo



Martynas Davidonis

FIN regrets to inform you that there are 2 board members resigning;
Anne Grimsbo and Martynas Davidonis.

We would like to thank Anne and Martynas for their efforts and valuable contributions to our community. We wish them all the best.

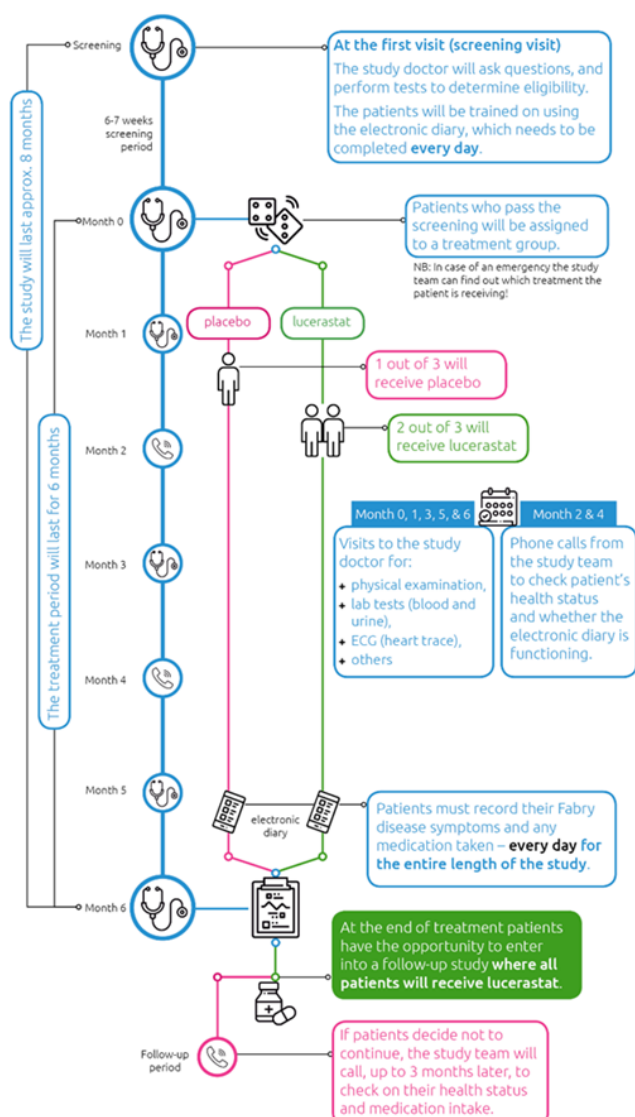
We have sincerely enjoyed the time that we have spent working with you. Thank you for all the guidance, support, and encouragement that you have shown over the years.



MODIFY - A phase 3 study investigating a potential new oral treatment for Fabry Disease

The ongoing MODIFY study is investigating the effectiveness and safety of lucerastat, a potential new oral therapy for Fabry disease. MODIFY is currently enrolling participants at medical centers in North America (USA & Canada), Europe (UK, Belgium, Netherlands, Poland, Austria & Germany) and Australia. During the COVID-19 outbreaks in these countries, enrollment of new study participants was put on hold to enable medical staff to focus on the treatment of patients with COVID-19. However, individual medical centers are now progressively lifting these restrictions and enrollment into the MODIFY study is recommencing. In addition, participant enrolment will soon be initiated at new sites in five additional countries across Europe (Ireland, Italy, Spain, Norway & Switzerland).

What is neuropathic pain?
a type of pain which feels like burning, shocks or shooting, stabbing, tingling, and/or pins and needles in the hands and feet
...sound familiar?

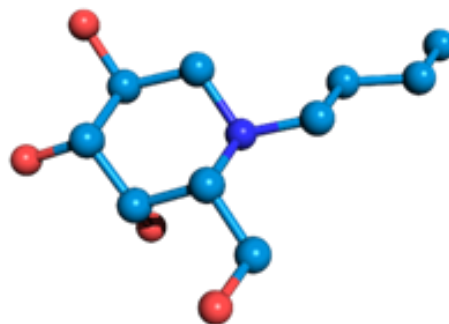


Lucerastat is administered in the form of oral capsules taken twice a day. Any adult with Fabry disease, irrespective of the type of genetic mutation that they have, may be eligible to participate in the MODIFY study. The key entry criterion is neuropathic pain, defined as sensations of burning, shocks or shooting pain, tingling, pins and needles, stabbing, and/or numbness in the hands and feet. Neuropathic pain may be permanent or occur randomly, and may be triggered by heat or cold, a fever, and/or physical activity.

MODIFY is a phase 3 study, meaning that lucerastat is now in the final stage of the clinical trial process that must be completed to evaluate the safety and efficacy of a new medicine before it is submitted to Health Authorities for review.

Participants in the MODIFY study have a 1 in 3 chance of being randomly assigned to receive placebo treatment. The capsules provided to participants assigned to receive placebo treatment will not contain any lucerastat. However, participants who complete the 6-month treatment period will have the option to enroll into an extension study investigating the long-term effects of lucerastat treatment. In the extension study, all participants will receive lucerastat. Treatment in the extension study can be continued for up to 2 years.

MODIFY - A phase 3 study investigating a potential new oral treatment for Fabry Disease



Graphical representation of the lucerastat molecule

The MODIFY study is sponsored by Idorsia Pharmaceuticals Ltd., a pharmaceutical company based in Switzerland (www.idorsia.com). Idorsia will organize and pay for travel, including air fares and hotel accommodation if required, even if study participants live far away from the nearest participating medical center in their country. For more information about the MODIFY study, visit www.modifyfabry.com, where patients with Fabry disease can submit a request to be contacted by the medical staff of one of the medical centers conducting the study in their country.

Additional information about MODIFY, including a complete list of participating medical centers in each country, can be found on the U.S. government's ClinicalTrials.gov registry (use this link www.clinicaltrials.gov/ct2/show/NCT03425539 or search using the identifier number NCT03425539).



Two news releases issued by AVROBIO one of which reported on [Fabry and cystinosis data](#), and the other on preclinical [Pompe disease data](#).

Avrobio would like to take this opportunity to summarize these updates. We recognize that this is a lot of information. We will be hosting a call in June to review these updates, as well as answer any questions our communities may have. It's important to note that AVROBIO therapies are investigational, and their safety and efficacy are still being evaluated in clinical trials.

AVR-RD-01: AVROBIO's investigational gene therapy for Fabry disease

- Nine patients with Fabry disease (four patients in a Phase 2 trial and five patients in an investigator-led Phase 1 trial) have been dosed in two ongoing clinical studies of AVR-RD-01. The Phase 2 trial continues to enrol participants; the Phase 1 trial is fully enrolled.
- This news release includes interim data on the four patients dosed in the Phase 2 trial of AVR-RD-01.
- In particular, the interim data show sustained alpha-galactosidase (AGA) enzyme activity and consistent trends in other measures up to 22 months after treatment for the first patient dosed.
- Additionally, the first patient with Fabry disease treated with plato™ gene therapy platform show a reduction in globotriaosylceramide (Gb3) one month after treatment, as well as, significantly higher white blood cell counts and AGA enzyme activity three months after treatment compared with the same timepoint for the other patients in the Phase 2 trial who were treated with the academic gene therapy platform.
- There have been no safety events attributed to AVR-RD-01 drug product in either the Phase 1 or Phase 2 trial as of the safety cut-off date (Nov. 29, 2019).



Chiesi Global Rare Diseases, in collaboration with Protalix BioTherapeutics, have submitted a Biologics License Application (BLA) to the US FDA for pegunigalsidase alfa for the treatment of Fabry Disease.

CARMIEL, Israel, May 28, 2020 /PRNewswire/ -- Protalix BioTherapeutics, Inc. (NYSE American: PLX) (TASE: PLX), a biopharmaceutical company focused on the development, production and commercialization of recombinant therapeutic proteins produced by its proprietary ProCellEx® plant cell-based protein expression system, or the Company, together with its development and commercialization partner Chiesi Global Rare Diseases, a unit of Chiesi, an international research-focused healthcare group, today announced the submission on May 27, 2020 of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for pegunigalsidase alfa for the proposed treatment of adult patients with Fabry disease via the FDA's Accelerated Approval pathway. Pegunigalsidase alfa, or PRX-102, was granted Fast Track designation by the FDA in January 2018. Pegunigalsidase alfa is the Company's purposefully-designed, long-acting recombinant, PEGylated, cross-linked α -galactosidase-A investigational product candidate.

The BLA submission includes a comprehensive set of preclinical, clinical and manufacturing data compiled from the Company's completed Phase I/II clinical trial of pegunigalsidase alfa, including the related extension study succeeding the Phase I/II clinical trial, interim clinical data from the Phase III BRIDGE switch-over study and safety data from the Company's on-going clinical studies of PRX-102. Upon the BLA approval, if approved, the Company will be eligible to receive a milestone payment from Chiesi.

"We are grateful for the assistance the FDA provided leading up to the submission of this BLA via the Accelerated Approval pathway, and we look forward, together with Chiesi, to working with the FDA as we seek marketing approval for PRX-102," said Dror Bashan, Protalix's President and Chief Executive Officer. "Together with Chiesi, we thank the investigators and study participants who have made reaching this milestone possible and have supported Protalix in our commitment to bringing this new treatment option to the Fabry patient community."

"The submission of this BLA to the FDA represents a significant milestone for our Global Rare Diseases division that was established earlier this year to strengthen Chiesi's focus on making a difference for patients living with rare diseases around the world," said Giacomo Chiesi, head of Chiesi Global Rare Diseases. "Our partnership and active collaboration with Protalix are a great example showing how we can leverage Chiesi's global reach and decades of experience in drug development to support patients and their families living with Fabry disease and many other devastating rare diseases."

[Read the full press release here](#)

This is an important step toward providing treatment options to patients.

Our thanks go to all those who are participating in the clinical trials – they are the ones who made this possible and deserve ours and the community's thanks.

FABRY INTERNATIONAL NETWORK BOARD MEMBER

NOMINATION PROCESS

There are currently two vacancies at the FIN Board. Nominations for a new Board Members are now open and an election process is herewith given together with the nomination form. If you wish to put your name forward for the selection process to become a FIN Board Member, could you please complete the nomination form, remembering that you need one supporter. Please return your completed form to FIN.

www.fabrynetwork.org

Nomination Criteria

Nominations will be considered from people who meet the following criteria:

- Have an active interest in the Fabry International Network and are prepared to invest the necessary time and effort to fulfil the duties attached to the appointment.
- Belong to a Fabry Patient Organisation Board or organization administrator.
- Have the support of at least one colleague.
- Are comfortable in the use of the English language.

Nomination and selection procedure

The nomination and selection procedure leading up to the election at the AGM is set out below:

- Nominations must be returned to the FIN Office.
- Nominations must be supported by at least one colleague.
- Nominees will attend phone or in person interviews with members of the current FIN Board.
- A selection of suited candidates will be presented at the annual AGM where the new board member will be appointed by the AGM attendees.
- All nominees will be advised of the outcome.

Role of the Board Member

- The role of the Board Member is to support the entire organisation through the process of governance.
- The Board plans FIN's future mission and priorities, it monitors performance and measures outcomes.
- The Board is responsible for all strategic planning and monitoring tasks and actions undertaken.
- Board members are individually responsible for participating in online and F2F Board Meeting. The language of these meetings is English and the appropriate expenses will be reimbursed (F2F).

[Click here for the nomination form](#)



Fabry Awareness Month at



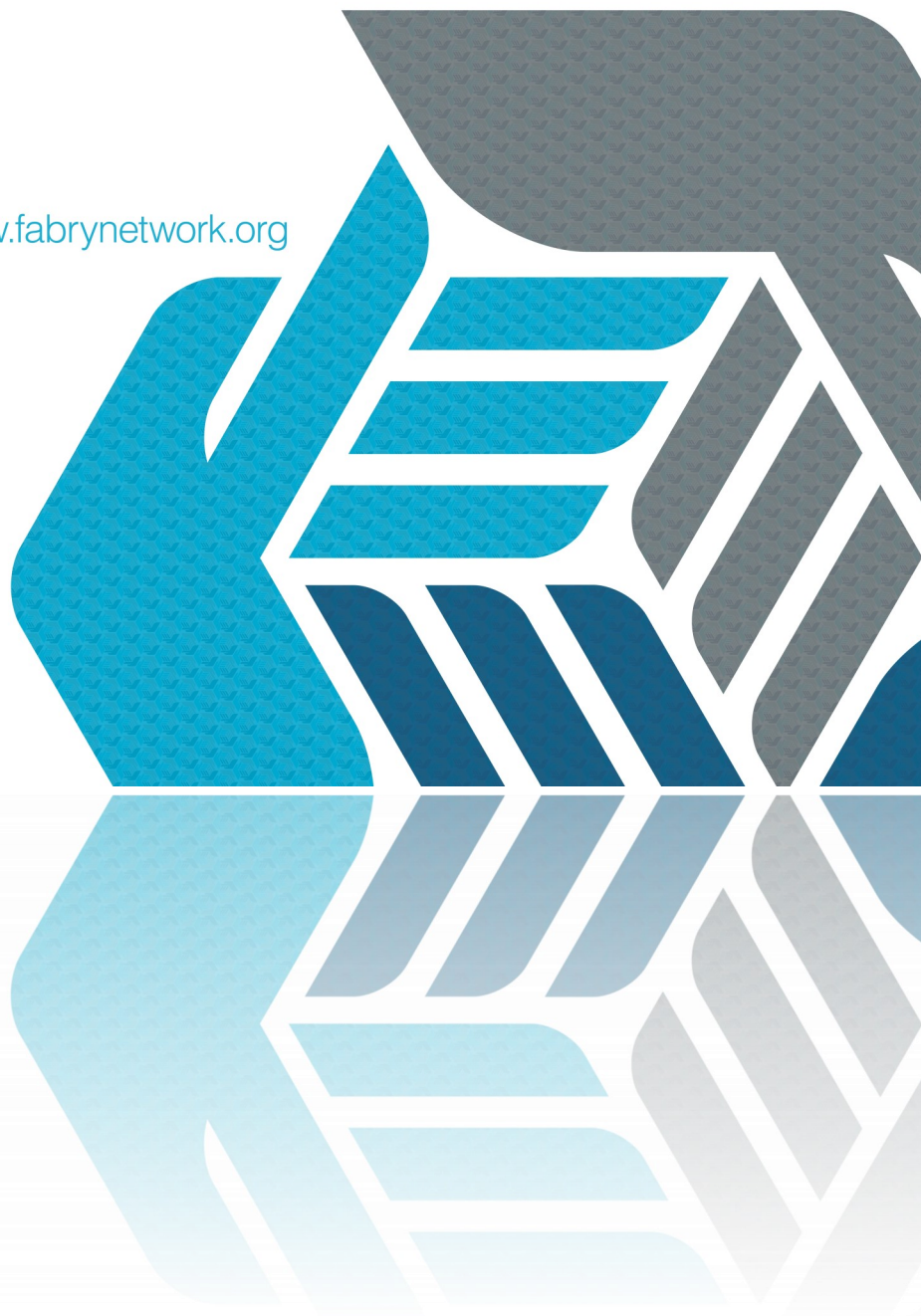
The next FIN Newsletter will go out end of September. Please send your articles and contributions by email to coordinator@fabrynetwork.org no later than September 15th, 2020. We look forward to publishing and sharing your latest news with our International Fabry Community!



FABRY
International Network

FACILITATE | SUPPORT | ENABLE

www.fabrynetwork.org



Fabry International Network

Registered address Alice Nahonlaan 7, 9120 Melsele - BE
Visiting address: Floralaan 35A, 9120 Beveren - BE

info@fabrynetwork.org
www.fabrynetwork.org

Charity Registration Number 04080030 The Chamber of Commerce Drenthe The Netherlands



Newsletter



September 2020

Contents

- FIN Expert Meeting 2021—Survey
- FIN Young Adult Webinars
- FIN Award
- “I Hand the Pen To” - Interview with Fabry Leaders around the world
- Rare Disease International
- Fabry Findings Issue 4
- News from Avrobio
- COVID-19 Survey Eurordis - *Preliminary results*
- News from Sangamo

A word from the President:

Dear All



We proudly present the latest FIN newsletter.

Fabry International Network is very committed to training the next generation of patient advocates and also providing a platform for young adults to get in contact with each other, to share their experiences and learn from each other. Our initial plan was to invite young adults to a face-to-face event this year. Due to the global pandemic, we have decided not to organise a face-to-face meeting but instead plan a series of webinars in September, October and November. We have successfully hosted the first webinar on Sept 12th, read all about on page 3!

The fourth issue of Fabry Findings is now released!

In order to make this newsletter as informative as possible we always welcome news and information from you as a caregiver, patient, patient organization and patient representative. Please send it to info@fabrynetwork.org

Stay safe and healthy!

Lut, FIN president



FIN EXPERT MEETING 2021

April 23rd - April 25th, 2021

Amsterdam, The Netherlands

We are seeking suggestions and input from our attendees with regards to the FIN Expert Meeting

A short survey is available at the link below.

When taking the survey, responses can be anonymous, or responders may enter their email address to be entered into a drawing for a complimentary FIN Expert Meeting 2021 registration.

The survey takes less than 5 minutes to complete with 3 multiple choice questions and 2 short questions with an option to provide comments.
We truly value your input!

Survey

FIN YOUNG ADULT COMMUNITY

Fabry International Network is very committed to training the next generation of patient advocates and also providing a platform for young adults to get in contact with each other, to share their experiences and learn from each other. Our initial plan was to invite you to a face-to-face event this year. Due to the global pandemic, we have decided not to organise a face-to-face meeting but instead plan a series of webinars in September, October and November.

"We understand that you are all in different stages/phases of your life but we also believe we can all learn from each other. What you all have in common is that you want to live the best life you can lead and not let Fabry stop you. We want to help you look at what is possible and help you explore your options through patient advocacy."

Anna draws her insights from her own experience as a Fabry patient. Lut, herself, has the experience with her 2 sons, now 34 and 35 years old. They don't have Fabry, but another metabolic life threatening disease. During their childhood and teenage years, her husband and she always encouraged them to pursue the things they wanted. So they did the "impossible" and we're convinced that if you really want something, you will succeed.

Although not everything happened in the same way as it would have for others but they tried to look beyond what we were told and that was exciting and satisfying. With this insight, we do know that every person is more than their disease. You are more than Fabry! We must acknowledge we have a disease, but this can't rule our lives. There is so much more out there in the world. So much to discover and so much to enjoy!

That's why FIN is very committed to provide a platform for you to get in contact with others, to share your experiences and learn from each other. FIN truly wants to build a community based on a foundation of trust, motivation and hope.

FIN Young Adult Committee, Lut De Baere & Anna Meriluoto

FIN aims to build a community and train the next generation of patient advocates, connect young adults from all over the world and form a highly motivated group of 18-35 year old's from the rare disease community. The main purpose is to instil confidence in the next generation of rare disease advocates by providing skill building opportunities to advance young adults in the next steps of their advocacy journey.

...

We were truly overwhelmed with the number of registrations and are happy to successfully have hosted the first webinar on Sept 12th.

We welcomed 21 young adults from all around the world. Amongst them, young adults from The Netherlands, Italy, Armenia, Germany, Spain, Canada, South-Korea, Taiwan, Poland,....

After introductions and discussing expectations, Wojciech Nadolski (*Poland*) shared his personal story which was very insightful and helped generating in depth discussions. We talked about passions in life and great tips were shared. From dating to informing your employer about Fabry, their social life and symptoms - we talked about it all. The role that young adults can play in the patient advocacy community was also an important topic of this webinar.



Learning patient advocacy skills will allow you not to be limited by Fabry but to be empowered by a community of patient advocates to live the life you want to lead!

Limitations can transform into opportunities to change things for yourself and the next generation!

The next webinar is taking place on Saturday Oct 3rd— 1pm-3pm CEST

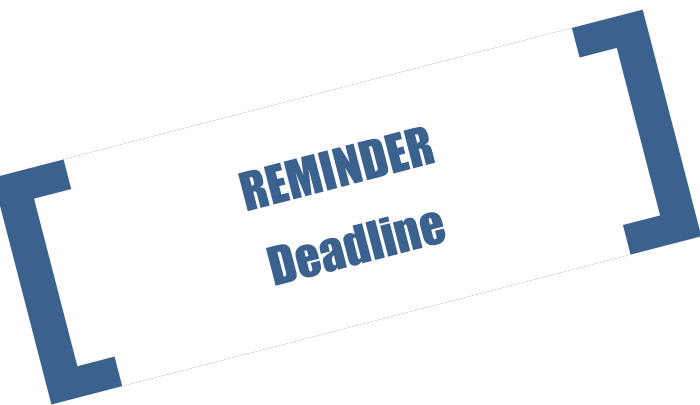
The focus of this webinar will be mental wellbeing, joining us for the discussion is Dr Nadia Ali, PhD.

We are also having a workshop hosted by Michel Van Cauter (holistic health trainer).

Want to join the FIN Young Adult community (18-35)?

[Click here](#) for the registration form.

Any questions? Send us an email on finyoungadults@fabrynetwork.org



FIN AWARD



FIN wants to encourage the membership to organise new activities and initiate projects by contributing financially and giving a platform to share with the wider community.

FIN will award a patient (association) led initiative that informs and educates about Fabry and helps raise awareness by providing a financial grant*

The winning project will be announced at the Expert meeting in 2021

The activity or initiative should be:

- ◇ Educational and raising awareness
- ◇ Bringing patients together (face-to-face or virtually)
 - ◇ Providing peer support

We encourage all out of the box ideas!

[Download the application form here](#)

**Terms & Conditions are stated in the application form
Deadline for applications: Nov 1st, 2020*



**RARE
DISEASES
INTERNATIONAL**

**Fabry International Network is happy to announce
their membership to Rare Disease International!**

RDI brings together national and regional rare disease patient organisations from around the world as well as international rare disease-specific federations to create the global alliance of rare disease patients and families.

Rare Diseases International aims to bring all umbrella rare disease patient organisations in every country and all regional and international networks for every rare disease into one global community to speak with one voice.

Rare disease patients are faced with common challenges derived from the rarity of their conditions and aggravated by the low priority given to rare diseases globally. Rare diseases are often chronic, progressive, degenerative, disabling and life-threatening.

A long road to diagnosis, lack of adequate treatments and care are challenges faced by rare disease patients around the world. These difficulties are greater in many developing nations.

Addressing rare diseases on an international level is critical to reduce health inequalities between populations worldwide and ensure that people living with a rare disease have access to the same resources as any other population.

RDI works to:

- Unite, expand and reinforce the rare disease movement of patient organisations and patient advocates
- Put rare diseases on the agenda of international organisations and multilateral institutions such as the United Nations, ECOSOC and the WHO, and on the national agenda of every country around the world
- Strengthen rare disease patient groups capacity to act at local, national, regional and global levels and to interact with other rare disease groups

RDI recognises that people living with a rare disease are facing similar challenges irrespective of where they live in the world. The over 300 million people living with one of over 6,000 known rare diseases around the world share a common interest to address their comparable or specific needs.

RDI helps ensure that people living with a rare disease worldwide and their families experience better recognition and support, improved health or social services, and overall a better life.

Follow them on [LinkedIn](#) and [Twitter](#).
www.rarediseaseinternational.com

FABRY FINDINGS

Scientific publications that are translated into lay language made available to the Fabry Community;

Every new article will be announced in the FIN newsletter.

In this issue we focus on gastrointestinal (GI) symptoms and their impact on people with Fabry disease.

This issue is based on the publication:

Pathologic substrate of gastropathy in Anderson-Fabry disease

Di Toro A, Narula N, Giuliani L, Concardi M, Smirnova A, Favalli V, et al.

Orphanet J Rare Dis. 2020;15(1):156

Fabry Findings

Issue No. 4 | Autumn 2020



In this issue we focus on gastrointestinal (GI) symptoms and their impact on people with Fabry disease

What is Fabry?

Fabry disease is a rare genetic condition which is estimated to affect around one in 100,000 people.¹

In Fabry, an enzyme called α -galactosidase A (α -Gal A) is missing or there is a reduced amount. This means that the body cannot break down a certain type of fat called globotriaosylceramide (GL-3). GL-3 continues to build up in body cells causing damage to tissues and organs. Gradually, this leads to a range of physical symptoms and complications, which vary from one person to another.¹

Study highlights

In our research news, we look at a study that was designed to investigate the accumulation of GL-3 in the GI tract cells of people with different Fabry mutations who all have GI symptoms.

 The **GI tract** is the passage for food to travel all the way from the mouth to the anus and includes all of the organs in the digestive system.

 For information on inheritance refer to [Fabry Findings Issue 1](#)

 In Fabry disease, globotriaosylceramide (GL-3), a type of fat, builds up in tissues and organs causing damage to the kidneys, heart and central nervous system^{1,2}

[Click here](#) to read the fourth issue of Fabry Findings

The Fabry Findings articles are published on the FIN website

<http://www.fabrynetwork.org/fabry-findings/>

We encourage you to translate the articles into your own language so these can be shared in your community. Source files are available upon request.

Winner Let's get Fabry Creative Contest

Fabry Korea won the 2020 Global Fabry Creative Contest Grand Price

'Fabry Korea (Chairman Lim Bong-ki)', a national Fabry disease patient association, wins '2020 Let's Get Fabry Creative Contest!'

Fabry disease is a genetic disease caused by genetic mutations in the X chromosome resulting in symptoms such as extremity pain, kidney failure, left ventricular hypertrophy, and stroke just to name a few. Initially, extremity pain can be a severe burning pain in the hands and feet. Disease progression can lead to poor quality of life, and as time passes progressing to possible organ failure. However, individual symptoms of Fabry disease are easy to mistake for other diseases making it hard to find the correct diagnose.

The Fabry International Network (FIN) is a non-profit organization established in 2005 to support Fabry disease patients around the world, working with more than 64 patient associations in 52 countries across the globe. In celebration of Fabry Disease Awareness Month every April, FIN holds various events to raise awareness of the disease and to support each other. April this year, Fabry Disease Awareness Month targets Fabry disease patients around the world.

"2020 Global Fabry Creative Contest" was held, which produced a variety of creative works such as photos, videos, drawings, and texts. In Korea, 10 patients with Fabry disease participated in the contest, among them Kim Gun-woo's poem "Spring", won the grand prize of honour.

FIN, who hosted this competition, said: 'We were truly impressed while reading Kim Gun-woo's poem.' Kim Gun-woo said, "I am very honoured to win the competition in which patients with Fabry disease from all over the world participated. I think it is because the patients sympathized a lot based on their similar experiences," he said. "I hope this poem is comforting to the patients, who feel they are not alone. And I hope the pain will disappear as soon as possible through the best treatment."



Korean patient, Kim Gun-woo (48 years old) receives the price!

The winner of this competition, Kim Gun-woo, has been working in the management team from 2018 when Fabry Korea was founded. In addition, after attending a global seminar hosted by FIN, he took the lead in improving the quality of life for patients with Fabry disease by holding workshops for patients with Fabry disease in Korea to share the main contents from the global seminar.

"Fabry Korea took a long time to become an official patient association after its inception," said Bong-ki Lim, chairman of Fabry Korea. "I would like to express my gratitude to all those who have applied. We will grow into a group in which various networks such as medical staff, support groups, and sponsor companies are organically active, not only for domestic patients through the expanded concept of the Fabry Korea Network. We will continue to carry out various projects such as providing medical information necessary for patients with Fabry disease, education for capacity building, promotion of social welfare systems, and policy research for advocating patient's rights."

Winning poem Let's get Fabry Creative Contest



길고

긴 겨울 밤

잠 못 이루고 뒤척이다 깨어서

긴 밤 홀로 깨어있네

졸음에 겨워 설핏 잠들었다

이내 다시 깬다

졸다 깨다 반복하며 영영 오지 않을 것 같은 새벽을 기다린

다

길고 긴 겨울 지나

봄이 오고 쌓인 눈 녹듯

아픔도 봄 눈 녹듯

스르르 녹아내리길...

Spring

In a long winter night

I'm awake after tossing and turning

I'm awake all night alone

Fell asleep for a while

Soon, I wake up again

I'm waiting for the dawn that never

seems to come, while dozing off

After passing through a long winter

Like spring comes and snow melts

Hope the pain goes away

Like snow melts in spring...

FABRY AWARENESS MONTH
APRIL 2020

COVID-19 and Fabry Disease

Since we wanted to keep our community informed during these uncertain times, we hosted our very first webinar in April, which was very successful. A second webinar took place in May. We were very happy to have our expert panel which consisted of Prof Germain, Prof Eyskens, Dr Ali and Dr Ortiz presenting valuable information to our members.

The recordings are available on our [YouTube channel](#).



Dr. Nadia Ali, Ph.D. Emory University, USA

[Click here to listen to the presentation](#)



Prof. dr. Francois Eyskens UZA, Belgium

[Click here to listen to the presentation](#)



Prof. Alberto Ortiz MD, Ph.D., Health Research Institute of the Jiménez Díaz Foundation Madrid, Spain

[Click here to listen to the presentation](#)



Prof. Dominique P. Germain, MD Ph.D. National Centre for Fabry disease, France

[Click here to listen to the Q&A](#)



FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Catalina Yin Taiwan Association of Fabry Disease

When did you join your national patient association and what was the reason for joining?

I joined my national association on February 19th in 2011 because I am a Fabry patient. I wanted to support and encourage other patients.

What is the vision and mission of your association?

As an organisation we focus on these 4 domains:

- Patient Rights Advocacy
- Health Educational Promotion
- Patient Care
- International exchanges



What do you consider to be the major achievements or activities you are proud of?

Our medical lectures for health education and awareness and the fellowships union.

Can you name some challenges that your association is currently facing?

Sometimes we hear that in Taiwan some consider the mutation, the GLA IVS4, not to be the same as classical Fabry disease and that it should be excluded from National social health insurance as they will not recognise it as a rare disease.

There is little focus on the mental health of patients.

Fundraising and awareness is not easy as our disease is not considered to be a burden and a reason to support us because there are no physical symptoms. To others, patient with Fabry disease look normal and it is challenging to convince them otherwise.

Can you name some future goals or plans?

Next year we will celebrate the 10th anniversary of our association, we have some exciting things planned!



Has your association had issues with the national health system or insurance problems?

We are lucky to have good national social health insurance in place.

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Catalina Yin Taiwan Association of Fabry Disease



How would you describe the current treatment situation in your country?

When patients are diagnosed with Fabry Disease, they can be treated, but the medical guidelines set by the relevant government agencies are becoming more and more strict, because the rate of Fabry disease diagnoses is increasing rapidly, which takes up a lot of the cost of rare diseases overall. In Taiwan, treatment is mainly enzyme replacement therapy, and the main drugs come from only two manufacturers.

What are the major issues with Fabry disease in your country?

Classical Fabry disease is considered an official rare disease but sometimes the later-onset subtype with the IVS4+919G>A mutation is not.

The cost of medical expenses are too high.

How would you further raise global awareness for Fabry disease?

Raising awareness that Fabry Disease also includes the later-onset subtype with the IVS4+919G>A mutation.

What are your suggestions for future projects for FIN?

Every national or local association needs to feel supported by FIN. Address more psychological issues to help young people face or plan the different stages of their life. As young people face the ethical issues of giving birth to the next generation, it would be good that they can be supported in these difficult choices.

What would you like to explain to other people / doctors / nurses / decisionmakers about Fabry disease?

Fabry disease is a rare disease. Fortunately, there are drugs for treatment now. We advocate for early diagnosis and to start treatment as soon as possible. We are still a group of people who can contribute to and serve society. We need the support of the government, people from all levels, various insurances, and the entire society. If we rely solely on our own capabilities, we do not have any patients who can receive treatment. It is very important we work together for this.

What kind of information are we still missing about Fabry disease?

Concerning the Fabry patient's gastrointestinal tract, tinnitus and hearing.... the distress and tools to cope.

Is there anything else you would like to add?

Thank you for this opportunity!

Thank you for your time!



Catalina Yin hands the pen to Ikuko Kaku from the Japanese Fabry Disease Patients and Family Association (JFA)



9 in 10 people living with a rare disease experiencing interruption in care because of COVID-19 Survey shows detrimental impact of coronavirus on rare disease community.

4 May 2020, Paris - EURORDIS-Rare Diseases Europe today announces preliminary global results from the first multi-country survey on how COVID-19 is affecting people living with a rare disease, finding that the pandemic greatly hinders access to care.

The COVID-19 pandemic has exacerbated the many challenges people living with a rare disease already face and has created extra risks in their daily lives, with collateral consequences.

5,000+ rare disease patients and their family members from all EU countries and beyond representing 993 diseases responded to the survey carried out via the Rare Barometer Programme. These results are based on survey responses submitted between 18 and 28 April 2020. These are preliminary figures and the survey continues throughout the duration of the crisis. Rare diseases are often chronic and life-threatening.

Click here to see the [preliminary results](#) from the survey.



The ASGCT is excited to share with you that the resources on gene therapy for Fabry disease are now available on their NEW Patient Education site! We hope you find that the overall site and disease-specific resources are useful.

[Click here](#) to view all of the resources!

AVROBIO Supports the Fabry Community

AVROBIO is a gene therapy company

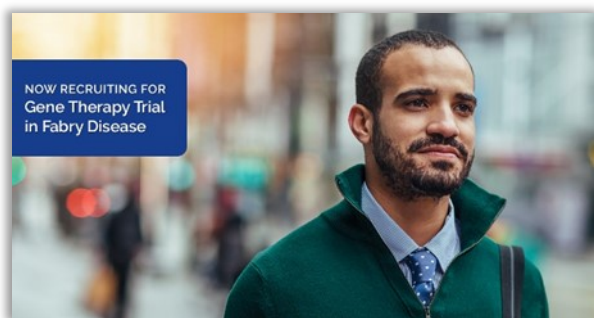
AVROBIO is a leading clinical-stage gene therapy company driven by a purpose to free people with lysosomal disorders from a lifetime of genetic disease. We aim to halt or reverse disease throughout the body by driving durable expression of functional protein, even in hard-to-reach tissues and organs including the brain, muscle and bone.

Learning from the Fabry community

In September 2020, AVROBIO hosted a meeting with leaders of the Fabry patient advocacy community from the U.S., U.K., Netherlands, and Spain to discuss and obtain advice on our investigational gene therapy program for Fabry disease and hear about the needs of the community. We are grateful for the opportunity to speak with and learn from the Fabry patient community!

AVROBIO is recruiting patients for FAB-GT, a phase 1/2 clinical trial (NCT03454893)

AVROBIO is developing an investigational lentiviral gene therapy called AVR-RD-01 for Fabry disease. AVR-RD-01 is being investigated in clinical trials and has not yet been approved by the U.S. Food and Drug Administration (FDA) or any other regulatory agency, and its safety and efficacy have not yet been established.



FAB-GT is a Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of AVR-RD-01 for the potential treatment of classic Fabry disease. We are currently recruiting patients for this trial.

If you or someone you love are interested in learning more about this trial, please contact 1-877-330-5216 or visit www.AVROBIOFabryTrial.com.

Please join AVROBIO at National Fabry Disease Foundation's Fabry Family Conference.

Please take the opportunity to stop by AVROBIO's presentation at NFDF's Fabry Family Conference where we'll provide information about our investigational gene therapy program for Fabry. We also encourage everyone to stop by and say hello in our virtual booth. We hope to see you there!

For more information, please visit AVROBIO.com or reach out to patients@avrobio.com.



New phase I/II clinical study to explore the potential of ST-920 investigational gene therapy to treat Fabry disease

The STAAR Study is Recruiting Patients Now

Fabry disease is caused by shortage of an enzyme called alpha-galactosidase A (α -Gal A). This shortage happens when the GLA gene, which provides the body with instructions for making α -Gal A, is not working correctly.

A new phase I/II clinical study has been designed to investigate the safety and tolerability of an investigational gene therapy called ST-920 to treat Fabry disease. ST-920 aims to deliver a healthy copy of the GLA gene to the liver.



It is hoped that the liver should then be able to produce the α -Gal A enzyme and secrete it via the blood stream to the rest of the body.

The STAAR Study is now recruiting men aged 18 or over who have been diagnosed with Fabry disease. Visit the STAAR Study website (www.staarclinicalstudy.com) where you can see if you qualify. You can also discuss this further with the study team, who are more than happy to help.

Contact details: clinicaltrials@sangamo.com

www.staarclinicalstudy.com



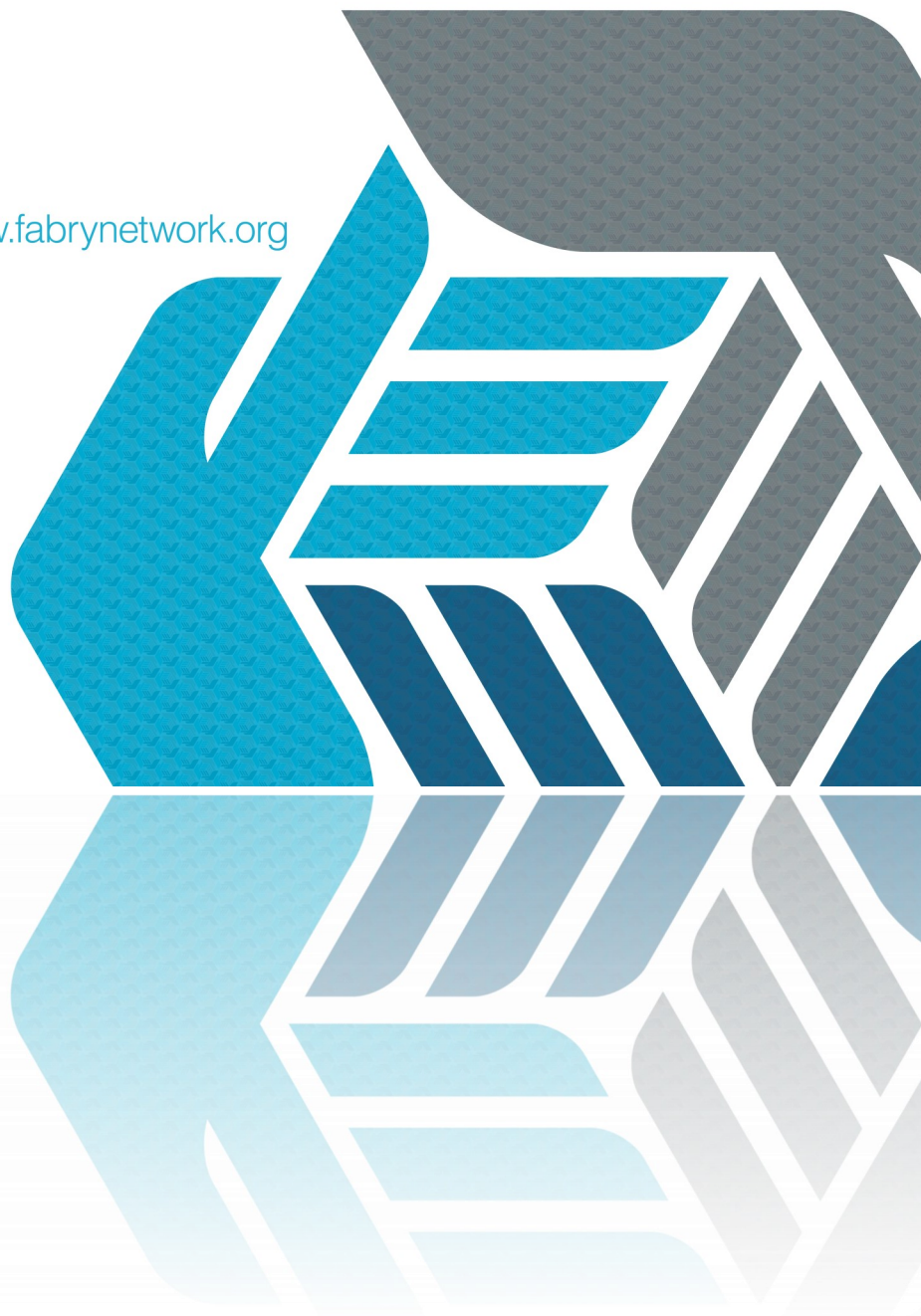
The next FIN Newsletter will go out end mid-December. Please send your articles and contributions by email to coordinator@fabrynetwork.org no later than December 8th, 2020. We look forward to publishing and sharing your latest news with our International Fabry Community!



FABRY
International Network

FACILITATE | SUPPORT | ENABLE

www.fabrynetwork.org



Fabry International Network

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Charity Registration Number 04080030 The Chamber of Commerce Drenthe The Netherlands



Newsletter



December 2020

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A word from the President:



Dear All

The last newsletter of 2020!

In this edition we share a lot of information which I am sure you will find an interesting read. We would also like to draw your attention to the save the date for our virtual FIN Expert meeting in 2021. We look forward to seeing you all at the meeting.

As we approach the festive season, I would like to pay tribute to everyone who works or volunteers to help others. This year the spirit of the season will likely mean different things to different people. Everyone's 2020 has been incredibly difficult and disruptive. Although we might not be able to spend this time of the year with all the people we love, connecting is now more important than ever. Reach out, call, Zoom, send a card and be thankful for what is and what is yet to come. On behalf of the FIN Board I would like to thank everyone for all your support and interaction, you are all truly amazing!

Stay safe and healthy!

A handwritten signature in black ink, appearing to read "Lut".

Lut, FIN president

SAVE THE DATE

2021 VIRTUAL FIN EXPERT MEETING

APRIL 24, 2021

Mental Wellbeing Tips from the FIN Young Adult Community

In light of the COVID-19 pandemic, we want to remind you that taking care of your mental health is as important as looking after your physical health. Good mental health and positive wellbeing can help you better cope with the COVID-19 threat and the uncertainty it's creating.

The FIN Young Adult Community wants to share with you their best tips for mental wellbeing!

- Practice gratitude & forgiveness
- Get some sunshine
- Spend time in nature
- Journaling
- Show kindness to yourself and others
- Being in the moment
- Positive self talk
- Meditation
- Open up about mental health issues and realise you are not alone
- Build a structure and create routines for your daily life
- Listen to music
- Talk to others or go out – spend time with family or friends
- Do fun stuff or give yourself nice things - Buy a plant when you feel down or drink a chai latte, whatever makes you happy
- Get enough sleep and eat nutritious food
- Get professional help when needed
- Exercise regularly and stay active
- Find out what makes you happy - not only what makes others happy
- Spend time alone with yourself - have conversations with yourself
- Know your limits: don't set impossible goals, know your limits in order not to deceive yourself
- Social Media Detox – don't compare yourself to others
- Take care of yourself! Use a facemask or scrub your face once in a while, dress like you are going to the awards... really spend time on yourself!
- Keep your mind busy, don't let intrusive thoughts bother you
- Always know you are valid: when there's someone criticizing because they don't understand the illness, remember it is not your fault and that you are valid in any way, you are not less than any other person in the world.



FIN proudly presents to you new members to the network



A warm welcome to:



Association Tunisienne des Maladies Lysosomales

L'ATML is an association recently founded by patients and relatives of patients suffering from rare and orphan diseases who are engaged in a daily fight against these diseases.



Mexican Federation of Rare Diseases

FEMEXER is the national alliance of rare diseases patients associations. FEMEXER is a non-governmental and totally voluntary alliance of patient organizations, directed exclusively by patients and their families; They represent more than 70 rare disease patient organizations in Mexico, which cover 450 rare diseases (RAID) among the population.



Proyecto Pide un Deseo México

PPuDM is the lysosomal patient association and is an organization dedicated to the support of Gaucher and Fabry patients in Mexico and is part of Palito and Toto Make a Wish AC (PyTPuDac), which is a grantee authorized by the SAT that meets all the requirements of Mexican law to be a non-profit organization. In addition, it belongs to the Mexican Federation of Rare Diseases (FEMEXER) as a founding member.

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Hisao Harada President, Japan Fabry Disease Patients and Family Association (JFA)



When did you join your national patient association and what was the reason for joining?

I joined the Japan Fabry Disease Patients and Family Association (JFA) in September 2010.

What was the reason for joining?

There were five reasons for me to join:

- 1. To expand ERT options available for patients including oral therapy*
- 2. To make treatment accessible wherever you live in Japan*
- 3. To make treatment affordable in our medical system*
- 4. To establish a collaborative relationship with bio/pharma industry*
- 5. To build equal relationships with medical service groups and local administrations*

What is the vision and mission of your association?

Our vision is to create communication between members as well as the development of our organization. Our mission is to establish a symbiotic society and a reliable medical system for rare disease patients so we are all able to live with dignity.



What do you consider to be the major achievements or activities you are proud of?

Currently patients in Japan have access to 3 different ERT and 1 oral drug treatment option. Regardless of where patients live, treatment is now fairly affordable. We organise annual educational seminars and symposiums at 7 main cities to cover the entire nation. We built very good relationships with local administrations, medical service groups and the bio/pharma industry.

Can you name some challenges that your association is currently facing?

Because of the global pandemic, we are now working to have home infusion available, for this we are working closely with researchers and congressmen to prepare a proposal for our Ministry of Health, L&W. Seeking radical treatment such as gene therapy and genome medicines. However, we still need to wait until the legislation part is ready.

Can you name some future goals or plans?

- More treatment options which patients and their family can choose between*
- Educate more doctors to become experts in Fabry disease.*
- From the patient centred perspective, we need to promote awareness and provide educational opportunities to learn and seek the best treatment options for themselves. Japanese patients are very passive when it comes to choosing their treatment and only rely on the doctor's advice. But nowadays we see patients also proactively taking control and communicating their preferences more.*
- As several treatment options are available for patients, we need to promote early diagnosis including new-born screening. It's important to have a follow-up system together with the screening.*

Has your association had issues with the national health system or insurance problems?

Due to the national healthcare system's coverage, all patients have access to treatment in general. There is no major problem with medical expenses due to a medical support system based on the patient's household income now. As for private life insurance, there are certain limitations and there are some conditions that need to be cleared still. This is a matter that I expect that will improve.

FOCUS ON FABRY LEADERS AROUND THE WORLD

Interview with Hisao Harada

President, Japan Fabry Disease Patients and Family Association (JFA)

How would you describe the current treatment situation in your country?

Approximately there are 1000 patients receiving treatment. After you get diagnosed, you can start treatment as soon as possible. There is a maximum cap payment system that won't exceed a certain amount. Therefore patients and family can visit multiple medical specialists if they need additional care or other medication for symptomatic treatment.



What are the major issues with Fabry disease in your country?

Many Fabry patients wish to have a pain-free life although they take pain relievers from overseas. The pain management still seems to be the biggest issue for Fabry patients I know. There is a large need for specialized medical care facilities for rare diseases. We need a prompt implementation for telemedicine including online consultation in case of future natural disasters and/or pandemics.

How would you further raise global awareness for Fabry disease?

I think it's much better to focus on children rather than adults. There is a report that says that one in 7000 patients could be diagnosed through new-born screening. The age that patients can start ERT can be as low as 4 or 5 years old. Therefore, it's ideal to work with physicians who check both kidney failures and heart failures and apply early screening interventions.

What are your suggestions for future projects for FIN?

It's more important to work together with Fabry specialists especially on gene therapy and genome medicine. I also like to see the potential which we can develop more. I think it might be nice to invite a genome specialist at your next expert meeting. Since Fabry disease is rare and doesn't meet the quota, it's important to unite the patients on a global scale.

What would you like to explain to other people / doctors / nurses /decisionmakers about Fabry disease?

Although Fabry disease is a rare disease, it's very important to think of it as something that could also happen to you or to someone close to you.

What kind of information are we still missing about Fabry disease?

Many patients worry about their future. Especially young adults may experience it more often. It tends to have their income lowered as their symptoms progress. Then it will be hard to even consider married life with children. It requires more support for them.

Is there anything else you would like to add?

I think it's important to make our voice heard. I think FIN holds a key role for the global movement in the Fabry community in the world. Thank you for your dedication! With Ikuko Kaku as the JFA representative for FIN we look forward to continue to work together.

Thank you for your time!



**Hisao Harada hands the pen to David Peña Castillo
from FEMEXER Mexico**

Supporting Cellular Resiliency

By Dr. Seema Kanwal, ND

News regarding COVID-19 is breaking daily. With all the media attention on this emergent pandemic, there is an extraordinary concern in supporting our health. We can all take basic steps to be responsible to ourselves as well as people around us in preparing and supporting our cells to try and ensure a good outcome.

COVID-19 is thought to have an incubation period of 1-14 days with signs and symptoms that last 1-4 weeks and include:

- ⇒ Nasal congestion
- ⇒ Febrile Respiratory Illness (greater than 100F (37.8C))
- ⇒ Fatigue, Headache, Cough
- ⇒ Sore throat
- ⇒ Gastrointestinal manifestations such as nausea, vomiting, and diarrhea have also been commonly found which are uncharacteristic of the seasonal flu
- ⇒ Infants and toddlers may show with only fever and lethargy

Although most sources are estimating that an individual remains contagious for seven days from initial onset of symptoms (especially with coughing and sneezing) individuals have been found to be contagious for up to 16 days after the first symptoms appeared.

SUPPORTING OUR IMMUNE SYSTEM, THUS OUR CELLULAR RESILIENCY

A healthy immune system will not only offer increased protection against the regular seasonal flu and other variations of the common cold, but also shown to minimize and improve the immune response when you are now in bed under the weather.

There is good evidence, albeit not large clinical trials, to show that persons with higher serum 25(OH)₂ D₃ levels are protected from upper respiratory tract infections. There is also good mechanistic evidence that high doses could be used to treat the flu. 25 Hydroxy Vitamin D tests are an excellent resource for obtaining a person's vitamin D levels. Other evidence also exists for the efficacious use of vitamin C, zinc, selenium, vitamin A, and Elderberry extracts.

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Supporting Cellular Resiliency

By Dr. Seema Kanwal, ND

Here are now a few things you can do to keep your immune system healthy and strong.



First, let's review the basics. Hand washing, is by far one of the key strategies for preventing viruses from spreading. Viruses are easily spread from person to person. Think of how many times a day you touch a doorknob, keypad or pick up the telephone. Wash your hands in soapy warm water for at least 30 seconds (or the time it takes to sing happy birthday) and try to avoid touching the faucet after doing so. Paper towels, although less environmentally friendly are more hygienic than cloth towels, as are hot air dryers.

Nutrition, nutrition, nutrition. Proper nutrition is the key to a healthy immune system. Would you put diesel in a gas engine? Eating fried, sugary or highly processed foods can hinder your immune system's ability to ward off infection. Brightly coloured and dark green leafy vegetables are loaded with immune boosting phytonutrients. Phytonutrients are nutrients that come from plants. Bioflavonoids, found in fruits and vegetables work with Vitamin C to help strengthen the immune system.



Sugar, consumed in even moderate amounts impairs your immune system's germ-fighting ability. Our white blood cells, which engulf bacteria and viruses, cannot function optimally when we eat high amounts of sugar, especially white refined sugar. Drink lots of water and herbal teas when you're under the weather. Stay clear of soda pop, as they are very high in sugar. Even Diet pop is a no no! Avoid sports drinks because they contain excess amounts of sugar and salt. Warm herbal teas such as peppermint, can be made cooler as a flavourful iced tea, will also boost the immune system.

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Supporting Cellular Resiliency

By Dr. Seema Kanwal, ND



Hydration hydration! This is much easier to do when the weather is warmer, versus when it is cold outside. I tend to drink less water in the winter. If water is difficult for you as well, drink lots of herbal, non-caffeinated teas as this will count towards water intake. Fall and Winter is a very drying time of year. If you are prone to nose bleeds, or sinus infections, you want to ensure you maintain hydration to moisten the mucus membranes, to prevent dryness in the first place. Do you know how much water you need to drink? Take your weight in pounds, divide by 2, this equals to the number of ounces required by your body. The 8 glasses of water a day I cannot disagree with, as this is a random number that I have no idea who came up with.

When treating any illness, herbs are best used in a combination that is individually tailored to you and your symptoms. How about taking a trip to your kitchen? Garlic is one of nature's most potent herb. Not only does it add wonderful flavour to foods, it is a powerful anti-microbial agent. It has been shown to reduce bacterial AND viral growth, thus making it an effective agent in fighting many infections. Add it to soups or stir-fry's at the end of cooking which will maintain the anti-microbial properties of garlic. Adding it to the beginning of cooking will make it lose efficacy of its antimicrobial properties.



9 in 10 people living with a rare disease experiencing interruption in care because of COVID-19 Survey shows detrimental impact of coronavirus on rare disease community.

[Out now: the COVID-19 European results in seven languages!](#)

How did COVID-19 affect people with rare diseases? Find out their [infographic](#) with the key findings from their biggest ever survey in Europe, now ready to share in French, Spanish, German, Italian, Portuguese and Russian!

Share it on social media to show the impact of COVID-19 on people with rare diseases as the pandemic continues to unfold.



**RARE
DISEASES
INTERNATIONAL**

Rare Diseases in UHC2030 Synthesis

Rare diseases have been mentioned in UHC2030's "State of Commitment to UHC" synthesis. This is the first edition of an annual report monitoring action towards Universal Health Coverage in all UN Member states.

*The synthesis report notes that the COVID-19 pandemic has magnified inequities for vulnerable groups including people living with a rare disease. (page 28)

*It highlights the need, not only for better coverage of health services, but also for improved specialized health services, making particular mention of the rare disease community. (page 34)

RDI is a member of UHC2030, a multi-stakeholder platform convened by the WHO and the World Bank. We would like to thank RDI member organisations who answered our call this September to participate in the UHC2030 survey on national UHC contexts. The recognition of the rare disease community in this seminal report is a great achievement.

[Click here to read the full report](#)

Dear

I am very pleased to introduce you to the 2019 update on the Fabry Outcome Survey (FOS). FOS is a disease registry sponsored by Takeda that collects information on patients with Fabry disease across the world. Information on symptoms, disease progression and treatment has been collected since the first patients joined FOS in 2001, and there are now over 4000 patients enrolled. The aim of FOS is to improve our understanding of Fabry disease, which will help healthcare professionals to provide the best care possible for their patients.

We hope that this report will help patients and caregivers who are involved or interested in FOS to understand better how the registry works and what has been achieved so far. It provides an update on the patients enrolled in FOS up to January 2020 and a summary of published findings from the registry. The report has a new layout with colourful infographics, is written in easy-to-understand language and provides answers to commonly asked questions about FOS.

On behalf of the FOS Steering Committee, I would like to thank patients and caregivers involved in the registry for their extremely valuable contributions. The more information we can collect on Fabry disease, the more we can learn and the more we can improve the care of patients in the future.

Mary Pavlou

Patient organization representative on the FOS Steering Committee

FIN secretary

[Click here to read the report](#)



Sleep Hygiene for Fabry Disease

People with Fabry disease frequently experience sleep problems. Because a good night's sleep is essential to physical and mental health, as well as your quality of life, it may be beneficial for you to practice sleep hygiene.

[Click here to read the article](#)



Enter the EURORDIS PHOTO AWARD 2021

Do you have a photograph that captures life with a rare disease during the COVID-19 pandemic? Perhaps you have the perfect picture already, or perhaps you haven't taken it yet. Either way, don't forget to submit it to the [EURORDIS Photo Award 2021](#)! This annual competition is your chance to show the world what it means to live with a rare disease.

AVROBIO held a webcast on November 18th where the company announced:

- 3 month Gaucher type 1 data for patient #1. AVR-RD-02 for Gaucher disease type 1: Positive early reductions in plasma lyso-Gb1 and chitotriosidase activity at three months as compared to baseline, when Patient 1 was on ERT; additional positive trends observed across multiple other measures
- AVR-RD-06 for Gaucher disease type 3: New program leveraging the same vector as AVR-RD-02 for Gaucher disease type 1
- AVR-RD-01 for Fabry disease: Potential accelerated approval strategy planning underway as clinical data across Phase 1 and Phase 2 trials continue to show positive and durable clinical activity and safety data. 42 month data for the phase 1 Fabry program
- AVR-RD-04 for cystinosis: Functional and clinical improvements for the first patient at 1 year; third patient in the trial dosed
- New preclinical data for AVR-RD-03 for Pompe disease: Preclinical data show normalization of substrate levels in multiple hard-to-reach organs
- Pre-clinical update for AVR-RD-05 for Hunter syndrome: Normalization of multiple biomarkers in mouse model of the disease

The company also announced End-to-end plato® platform ready to enable global commercialization and data on its industry-leading plato® platform highlighting advances in chemistry, manufacturing and controls (CMC) to prepare for planned upcoming trials and potential global commercialization

[Click here to read the full press release](#)



This holiday season is unlike any other, to cap off a year unlike any other. Throughout this season, and as we move into a new (and hopefully better) year, we wish you moments of peace amid the difficulties, connections with family and friends even if they can't be in person, the warmth of memories from holidays past, and wonderful glimpses of the joy that still lives under the surface.

We are here for you through it all. We wish you endurance, strength, health, and as much happiness as these times can allow!

Best wishes for the holidays and the coming year from the entire FIN Board.



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