

## ALIVE IN EUROPE!

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**In 1980, Eppu Normaali (famous Finnish band) released the double live album with the title of this article — long before Finland's EU membership — as if to awaken Finns who were stuck in the 1970s to a new, more European dawn. In a somewhat similar vein, six FSHD Finland members traveled to Amsterdam for an international FSHD meeting to obtain the latest information about the disease, its treatment and research, and to meet fellow sufferers from all over Europe.**

The FSHD Society (USA) and FSHD Europe organizations jointly organized two major international FSHD events in Amsterdam, the Netherlands, from June 12-15th, 2025: a conference for clinicians and medical and rehabilitation professionals, and the FSHD Connect Europe meeting for FSHD patients and their loved ones. There has been a need for a Europe-wide event, as evidenced by the fact that the event for sufferers was fully booked even before the registration period closed.

### **Internationality as an inspiration**

For the FSHD Finland, founded last year, internationality is a given, as its name suggests.

- The request to establish an FSHD patient group came from abroad in 2023, and at the same time an invitation to the FSHD meeting in Milan also came. At the Amsterdam meeting, Finland was well represented in terms of its population, says the association's chairman, founding member and primus motor **Lauri Pirinen**.

- We are now also members of the Finnish Neuromuscular Disorders Association, says Lauri, who is studying to become a Master of Science in Engineering, and is delighted with the prospects of cooperation on behalf of the entire small association.

Face-to-face meetings, peer support, WhatsApp groups, networking with other players in the field, and association routines are also an essential part of its activities.

- Large amounts of research money for muscular diseases are spent in the USA and Central Europe, and one of our association's tasks is to make fresh research information available to all interested parties in Finland, explains **Lora Chun**, vice-chair of the association.

Lora was also an active player right from the association's founding, and both Lauri and Lora are members of the FSHD Europe board.

### **Clinical research and patient responsibility**

Clinical research refers to medical research that is conducted on humans, for example to determine the effectiveness and possible side effects of a drug or treatment method. For the past year and a half, **Nicol Voermans**, MD, neurologist at Radboud University Nijmegen (Netherlands), and **Ria de Haas**, PhD, Chief Executive Officer of FSHD Europe, have been closely organizing these FSHD events within the ranks of FSHD Europe.

- There is currently no cure for FSHD, but that does not mean that there are no treatments available, Voermans points out.

Existing treatments should be utilized to alleviate the symptoms caused by the disease. Good treatment requires a multidisciplinary team, and in addition to the treating neurologist, the team may include, among others, a physiotherapist, occupational therapist, speech therapist, nutritionist, psychologist and social worker. The task of clinicians is to research and develop even better treatments.

- The patient is responsible for living a life that is committed to different treatments and a healthy lifestyle in general, emphasizes Voermans.

### **Finnish guts and hope**

Fshd1 (95% of those with fshd) and fshd2 are both caused by the DUX4 gene, which is an essential gene for the development of the embryo as it multiplies from 4 cells to 8 cells. This gene is normally inactive or “turned off” in the later embryonic period, but in the case of fshd1, the gene suddenly and unexpectedly becomes active, forming the “toxic” DUX4 protein, which causes the disease. Now that the cause of the disease is clear, it is easier to target its cause. This is probably why there are about 20 drug trials underway around the world for fshd alone.

- There are about 12 drug trials underway in the USA, either in the clinical or preclinical phase, and we are very confident that one or more drugs will work, says **Lucienne Ronco**, Ph.D. from the FSHD Society.

Most of these drugs in development aim to affect the harmful DUX4 protein in FSHD.

- Finns are a resilient bunch, and we must maintain our hope. We all need to learn about the disease, monitor the progress of drug trials, and do genetic testing to confirm the diagnosis. We should also educate and train primary care physicians, neurologists, and decision-makers about FSHD, Ronco encourages.

- The political situation in the USA, Europe, and Asia may have a negative impact on the funding of medical research, however currently many biotech and pharma companies are committed to testing new drugs in fshd. We expect these drug trials to go ahead, Ronco continues.

- But as human rights activist Martin Luther King, Jr. said: “If you can’t fly, run. If you can’t run, walk. If you can’t walk, crawl. But whatever you do, you have to keep going.”, Ronco concludes the interview.

### **Stop the press**

A couple of days before the start of the Amsterdam FSHD meeting, our group receives hopeful news in their earbuds and on the screens of their computer gadgets: Avidity Biosciences’ investigational drug for FSHD has advanced to phase III of clinical trials. This is the second drug whose research has progressed this far, and it means that the scientific community considers it promising. However, the first investigational drug in this phase did not ultimately lead to the desired outcome. Several participants who themselves suffer from the disease will be included in the phase III study, and if the results of this phase are good, the drug may receive a marketing authorization and be on the shelves of Finnish pharmacies in a few years. It is important to remember that the road to a drug that slows or even stops the progression of the disease is still rocky. But it is starting to look increasingly clear that that day will come for FSHD someday. And it is good to be aware that even a drug trial that does not bring a working product to the market also advances research in some way. This door didn’t open, so let’s try the next door! The price of drug treatment and its possible reimbursement for patients is another matter entirely.

### **FSHD Eurovision i.e. Bara bada bastu (Finnish Eurovision Song contest song 2025)**

- A significantly larger conference than any I have been to before. This was an opportunity for FSHD Europe to show that it can organize a big event, and also to show the level of European research. As one patient mentioned, the atmosphere was like the FSHD Eurovision. There were many complicated issues at the professional conference, but, for example, tremendous progress had been made in biomarkers and the development of disease progression models, Lauri analyzes.

**Eveliina Jormanainen**, a specialist in general medicine and a board member of FSHD Finland, accompanies Lauri.

- It was a high-level scientific congress with a record number of participants (approx. 300) from all over the world, which shows that research into FSHD has increased significantly in recent years and is of wide interest. New results were presented from both basic research (e.g. elucidating the disease mechanism, finding biomarkers that describe the activity of the disease) and research aimed at rehabilitation and drug treatment, Eveliina summarizes her experiences.

- The scientific community was confident that at least some specific drug treatment would be available within a few years, but a lot of research is still needed in different areas of the disease, Eveliina emphasizes.

Lauri and Eveliina accomplished a feat by participating in both events in Amsterdam from morning to night for four days. They had wisely reserved some time to explore Amsterdam after the events, and so the journey from Schiphol Airport to the hotel was not the only memory of the trip besides the hotel.

Lora Chun thought the intensive workshops and speeches by FSHD experts were very interesting.

- The event was also a great opportunity to network and meet people working on the same issue from all over Europe and the world, Lora enthuses.

For many patients, FSHD causes leg fatigue and stumbling, and later mobility aids (such as walking sticks and a wheelchair) may become relevant.

- It felt a bit strange to suddenly meet almost a hundred fellow human beings who move in the same unstable and unique style as me. Soon this became the new normal, and it felt like looking in a mirror. On the third day I began to wonder why so-called normally walking people was walking in such a weird way, **Rami Jokinen** sums up his experiences.

- Well, these are questions of perspective and attitude, Rami thinks.

Rami also liked the sauna at the event hotel and especially its foot bath.

- Without the scalding hot foot baths in the evenings, my sense of humor might have run out in the middle of the tournament, Rami sighs.

The group agrees that it would be great to host a similar event in Finland sometime in the future, preferably in cooperation with the Finnish Neuromuscular Disorders Association. FSHD Finland also wishes to have more clinics in

Finland that conduct research aimed at muscular dystrophies, so that Finnish sufferers could participate better in clinical trials if they wanted to.

### Let's see action!

Irish **Eoghan Curry** thinks that the event provided a lot of new research information about the disease, but the Best thing was meeting other sufferers. In addition to his muscular dystrophy diagnosis (FSHD2), Eoghan is burdened by a severe shoulder and nerve injury as a result of a work accident.

- It feels strange to say, but for the first time in 10 years I felt like an important part of the community, and not a lone wolf, Eoghan says after returning home to Dublin after the Amsterdam meeting.

The most valuable thing we can give to each other – and ourselves – is time, respect and the effort to see another person as they really are. So come join the activities of FSHD Finland, your local muscular disorders association or the Finnish Neuromuscular Disorders Association, and together we will improve the world one muscle cell at a time. As Eppu Normaali (famous Finnish band) sings on the live album I mentioned at the beginning of this article, covering famous rock band The Who: "Let's see action, let's see people, let's see freedom, let's see who cares."

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Information corner:

FSHD Finland: <https://fshdfinland.org>

- Finnish FSHD Association

FSHD Society: <https://www.fshdsociety.org/>

- US FSHD Association

FSHD Europe:

<https://fshd-europe.info/>

- European FSHD Association

MD Nicol Voermans' article on FSHD in English: <https://www.fshdsociety.org/2023/12/04/treatments-for-fshd-a-broader-view/>

On this site you can search for information about clinical medical trials around the world, using your own diagnosis as a search term, and also apply to participate in a trial: [www.clinicaltrials.gov](http://www.clinicaltrials.gov)