ECMTF General Assembly Report – 29/06/2024, Barcelona

The following Delegates of the Federation's Member Associations were present:

Daniel Tanesse – Delegate from CMT France, Full Member, President of the Federation
Filippo Genovese – Delegate from ACMT-Rete (Italy), Full Member, Interim President of the Federation
Ingolf Pernice – Delegate from the CMT Group in DGM (Germany), Full Member, Vice President of the Federation
Marco van der Linden – Delegate from Spieziekten Nederland, Full Member, General Secretary of the Federation
Simon Bull – Delegate from CMT UK, Full Member, Treasurer of the Federation
Alexander Leysen - Delegate from Spierziekten Vlaanderen (Belgium), Full Member
Arabela Acalinei – Delegate from Neuro Move CMT (Romania), Full Member, and EAMDA, member of the Federation Board
Zsuzsanna Varga – Delegate from WonderFlora & CMT (Hungary), Full Member
Andreea Szabo and Simona Marcu – Delegates from CMT Romania, Full Member
Michela Padovan - Delegate from AICMT (Italy), Full Member
Kerstin Fieber – Delegate from CMT Austria, Full Member
Roberto Fernandez Lopez and Fernanda Lamos Ojea – Delegates from CMT Spain, Associate Member
Nechemya Rubin - Delegate from CMT Israel, Associate Member
Eva Úbeda Baizán and Alfredo García López – Delegates from El Motor de tus Pasos (Spain), Associate Member
Katherine Forsey – Delegate from CMTA (USA), Associate Member

The following persons were also present:
Alexandre Hoyau, President of CMT France
Marina Brocca, secretary of the European CMT Federation

The General Assembly is declared open at 2.30 pm.

Welcome to the participants

The President welcomes the participants and introduces the Board Members. He also announces his decision to resign from the position of President due to health issues. Filippo Genovese, Interim President of the Federation, holds the meeting.

Quorum

11 Full Members of 12 are attending the Meeting (Progetto Mitofusina2 is absent and has not sent a proxy). The General Secretary confirms that the quorum has been reached.

Approval of the minutes of the 2023 General Meeting

The 2023 General Assembly was held in Paris on 10th June. The General Secretary requests approval of the 2023 General Meeting minutes which are approved unanimously.

The Federation's Member Associations

Delegates from member associations are asked to present a short PowerPoint on their activities, goals, and hurdles.
All presentations are available at this link: https://tinyurl.com/ECMTFGA2024
The Interim President concludes by illustrating the common points of the presentations, the problems that all associations face and the activities implemented in the interest of CMT patients. The Interim President concludes this point with a PowerPoint presentation on the Federation, outlining its main activities, goals and hurdles. This presentation is also available at the link above.

2023 Activity Report

Board composition variation

In November 2023, Daniel Tanesse, the ECMTF's President, asked Filippo Genovese to replace him in this role and take charge of the Interim Presidency. Filippo kindly agreed to take on this position.
Dany Maes, the ECMTF's Treasurer, resigned from the position of Delegate of the Spierziekten Vlaanderen association and that of Treasurer. The new Delegate for Spierziekten Vlaanderen is Alexander Leysen, while Simon Bull, Delegate for CMT UK, accepted to be the new ECMTF Treasurer.
Arabela Acalinei was appointed to the EFNA Board of Directors. She is also a member of the Euro NMD Peripheral Neuropathies Working Group and the Patient Advocacy Board, and she will be a member of the Gene Therapy Working Group.
Ingolf Pernice, the second ECMTF Vice President, was also appointed to the EFNA Board of Directors.

Long wave of the 1st European Charcot-Marie-Tooth Specialists Conference

The Conference was the Federation's most relevant event in 2023, followed by the creation of ECRA (European CMT Researchers Association) and a mailing group of all the participants.
All reports and recordings of the event are available on the Federation's website: https://ecmtf.org/events
Furthermore, an opinion paper will be ready by the end of the year.

Pharnext's trial results

Pharnext has been our leading funder in recent years. The topline results from the pivotal Phase III clinical trial were made known in December 2023, and unfortunately, they were not as positive as hoped. The laboratory is going through a delicate financial phase, and the consequence has been the closure of the Federation's sponsorship and the CMT&Me program. In any case, we have an open channel with Pharnext, and we have signed a confidentiality agreement.

2023 CMT Awareness Campaign

As every year since 2017, also in 2023 we have run the CMT Awareness Campaign.
Pharnext's lack of financial support had an impact on the budget dedicated to the Campaign, which was lower than usual (10.000 €). No new content has been produced, but the large amount of material from past years has been reworked and adapted for the 2023 Campaign.
The material for the Awareness Campaigns is available on the dedicated website, https://cmt-awareness.com. Every Member can view, use, and share videos and interviews with patients and specialists in different languages.

The Campaign's results have shown us that all the social media indicators improved in a good, steady, and dynamic rhythm. The partners mentioned in posts (Associations Members, Pharnext, ambassadors) who

shared the content further increased the Campaign's resonance and helped promote the Federation's networks.
All these positive results can also be attributed to regular posting on social media even outside of the Campaign period, activity we should increase.
Overall, we were unsatisfied with the service offered by Aggelos (the agency that has managed the Campaigns since the beginning). Thus, we are thinking of finding a new media agency.

Federation's Members
This year we have added a new, and welcome, member: CMT Spain.
At the moment, the Federation is composed of 12 Full Members and 10 Associate Members.
The interim President invited all Associate Member in the EU to switch to a full membership.

New contacts and partnerships
We have recently contacted new laboratories dealing with CMT:

- Novartis, with the DTx-1252 drug under development.
- Actio Bioscience, developing a CMT2C drug.
- Alesta Therapeutics, developing new kinase inhibitors for the treatment of neuro-related diseases.
- Helena and Wolfgang Pernice, Ingolf's kids, are developing a phone application called GaitMate. The application helps patients create a clinical file on individual CMT journeys to share with clinicians, enabling remote care and easier specialist access. The project is under development, and they are looking for funds. The Federation promotes and supports this valuable project.

Helena is an assistant physician for neurology at the Charité Berlin and a clinical researcher on hereditary neuropathies and amyloidosis.
Wolfgang is an Assistant Professor at the Laboratory for Digital Biology (LDB) at the Merritt Center for Neuromuscular Diseases.

EU Election Manifesto
Thank to Ingolf, the Federation prepared the EU Election Manifesto and asked the association members to send it to the national EU Delegates to raise awareness of our condition and what living with CMT means.

Brice Chapuis
Brice is a French student completing his studies in Austria. In 2023, he tried to run the Vienna Marathon, but an injury during training prevented him from participating. Nevertheless, Brice raised nearly 500 euros for the Federation. This year, he finally ran the marathon to raise awareness of CMT. Congratulations to Brice.

The General Secretary requested approval of the 2023 Activity Report, which was approved unanimously.

2023 Financial Report

The Treasurer presents the income and expenditure of the 2023 final budget.
The costs of the 1st European Charcot-Marie-Tooth Specialists Conference were entirely covered by grants.
The lack of Pharnext's usual funding has prevented us from covering the Federation's ongoing operating expenses. The final balance is - 21,699.75 euros.
The Treasurer then presents the 2023 treasury. The savings on outsourcing in past years have allowed us to have a positive treasury balance at the end of 2023. The final balance is 117.642.28 Euros.

The General Secretary requested approval of the 2023 Financial Report, which was approved unanimously.

Renewal of the Board Members

Two candidates have come forward to fill the role of Board Member.
Alexander Leysen is a delegate from Spierziekten Vlaanderen (Belgium) and is responsible for the CMT group within the Belgian association.
Alexandre Hoyau, Delegate from CMT-France (replacing Daniel Tanesse) and President of the French association.
Both are elected unanimously.

The office term of the Board Members lasts three years. At the end of this period the members of the Board must be reconfirmed. Two Board Members must be reappointed.
Filippo Genovese is confirmed unanimously.
Marco van der Linden is confirmed unanimously.

Congratulations to the newly elected and confirmed members of the Board.

Actions and Projects 2024/2025

Forecast Budget

The Treasurer presents the income and expenditure predicted for 2024. No subsidies are foreseen; a negative balance of -34,468.09 euros is expected.
The Treasurer then presents the predicted 2024 treasury. Once again, thanks to the savings on outsourcing in past years, a positive treasury balance of 47.104.39 euros is expected.

New President

Daniel's health problems prevent him from maintaining his position as President, and Filippo has kindly accepted the role. His work commitments, however, prevent him from dedicating all his time to the Federation. Arabela, also proposed taking care of fundraising. She would be paid a percentage of the funds raised, but since board members cannot be paid, she would have to resign from the Board. The name we are defining for her position would be “Strategic Partnership and Funding Director / Officer”.

ECMTF Board

The new composition of the board is as follows:
Honorary President – Daniel Tanesse
President – Filippo Genovese
Vice President – Ingolf Pernice
General Secretary – Marco van der Linden
Treasurer – Simon Bull
Board Member – Alexander Leysen
Board Member – Alexandre Hoyau

Paradigm Shift

Filippo, as the new President, asks the Board Members and all the Members to be more proactive in contributing to the Federation's life. A more significant number of meetings is planned: the board will meet every two months, and meetings will be organized to let all the Members participate in the Federation's activities and share experience on specific topics.

Filippo invites the Member to interact with the Federation's social media (reposting and commenting) and consider the idea of a cyclic takeover, giving each association the Federation account for a short period (maybe a week) to post content. Filippo also invites the associations to inform the Federation on their activities and initiatives to give them as much prominence as possible.

According to our motto, together we are stronger.

Financial Sustainability

As we have seen from the financial report, the economic sustainability of the Federation is a priority issue. We are working on reducing costs, developing projects to apply for funds, looking for new partners, and considering reformulating the outsourcing service and the membership fee.

The 2025 General Assembly will most likely be entirely virtual, considering that most of us might want to join the 2nd European CMT specialist conference (see below).

Meetings Participation

Arabela and Ingolf, as mentioned ahead, are both part of the EFNA Board, representing the Federation and the CMTs within this organization.

EFNA stands for the European Federation of Neurological Associations. Although it brings together associations that deal with the most diverse neurological diseases, it has been very focused on mental problems. The presence of two of our Board Members and a third CMT will contribute to broadening the focus of EFNA to neuromuscular diseases, including CMT.

The first example is the already mentioned Memorandum for EU Delegates. The one originally drafted by EFNA focused mainly on mental issues. Ingolf drafted and proposed an integration that includes neuromuscular diseases and CMT for EFNA. What Ingolf proposed was taken into consideration, but not to the extent he wanted. This is how Ingolf decided to draft the above document, a complement to the EFNA one that adds a greater focus on neuromuscular diseases.

1st European Charcot-Marie-Tooth Specialists Conference follow-up

After the Conference, we set two main objectives: to create a European Registry of CMT patients and an association that would bring together scientists, clinicians, and other health professionals who work with CMT.

The European CMT database can be created starting from the national databases of the individual centres with CMT patients. The harmonization of different databases is a long and complex challenge that requires above all funding.

The procedures for establishing the research association began immediately after the Conference, and ECRA (European CMT Researchers Association) is now ready to be registered. The main objectives of this organization are to create synergies among professionals, share knowledge and information, improve research by discussing new ways to promote it and draft common projects between senior and junior researchers. It will also be important to develop professionalism in drafting projects to submit to the European authorities to obtain funding, and to encourage young researchers to focus on CMT.

2nd CMT Specialist Conference

The 2nd CMT Specialist Conference will be held in Antwerp on 24 and 25 October 2025 under the impetus and help of Vincent Timmerman. Vincent has organized conferences for years at the European CMT Consortium, an organization that became part of the PNS and is now under the direction of Mary Reilly. Of course, ECRA's activity is not a replica of that of the PNS; the Board members ensure that this unnecessary eventuality does not occur.

The reactions of CMT researchers and specialists were enthusiastic about organizing a new conference in Antwerp. This event will be more scientific than last year's in Paris, to attract a greater number of young researchers.

Projects to be submitted to potential sponsors (proposals from the Board Members)

- 2025 CMT Awareness Campaign

  Arabela presents Seta Consulting's proposal for implementing the Campaign. The Board will evaluate the proposal.

- Activities for the 2025 Rare Disease Day
- CMT's patients and doctors dedicated webinars / thematic CMT online webinars
- TV program on Rare Diseases
- CMT chatting circles
- ECRA scientific workshops

Collaboration with Pharnext

It was fruitful and beneficial for both parties. On our side, we always supported their research, lent a hand with awareness, promotion, and the CMT&Me study, and contributed to the realization of a guide for patients (in several languages), providing significant feedback on patient-oriented material.

This experience is something to stress while interacting with new sponsors.

New partnership

Our need is to consolidate partnerships with the last year Conference sponsors and start relations with new laboratories and pharma companies.

Other Topics

Update of the trials & studies for the CMT diseases

Filippo presents a PowerPoint on the state of the art of CMT research. The first part is dedicated to the description of how a trial works (phases, randomized double-blind, approval), and then it focuses on the most exciting and promising studies. A part of the presentation is dedicated to Pharnext's trial results, as reported in their latest press release. Unfortunately, they are not as positive as expected. Pharnext is waiting for the results of the trial in China; if they are positive, they will apply to the FDA / EMA for the drug. While waiting for positive results from these studies, let's remember we have some treatments: surgery, physical/occupational therapy, orthotics, and a healthy life.

The meeting was declared closed at 6.30 pm; thanks to everyone who attended.

All presentations from the meeting are available here: https://tinyurl.com/ECMTFGA2024

20/08/2024

Filippo Genovese
President

Marco van der Linden
General Secretary