ECMTF General Assembly Report – 28/06/2025 3 PM CEST, Online

The following Delegates of the Federation's Member Associations were present:
Filippo Genovese – Delegate from ACMT-Rete (Italy), Full Member, President of the Federation
Ingolf Pernice – Delegate from the CMT Group in DGM (Germany), Full Member, Vice President of the Federation, President of ECRA
Marco van der Linden – Delegate from Spierziekten Nederland, Full Member, 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
Alexandre Hoyau – Delegate from CMT France, Full Member
Arabela Acalinei – Delegate from Neuro Move CMT (Romania), Full Member, Partnerships & funding director
Michela Padovan - Delegate from AICMT (Italy), Full Member
Luisa Perrero – Delegate from Progetto Mitofusina2 (Italy), Full member
Rita Drávucz – Delegate from WonderFlora & CMT (Hungary), Full Member
Barbara Chaloupek – Delegate from CMT Austria, Full Member
Katherine Forsey – Delegate from CMTA (USA), Associate Member
Laura Mac Neill, CEO CMT Research Foundation (USA), Associate Member

The following people were also present: (not for the entire meeting)
Marina Brocca, former consultant of the European CMT Federation (acknowledgments)
Karolina & Lukasz, CMT Poland
Benjamin Arnaud, Cycling across Europe for Charcot-Marie-Tooth

Welcome to the participants

The President welcomes all the participants. The meeting began with a moment of silence to honor the memory and legacy of Daniel Tanesse, a foundational and inspiring figure in the federation, honorary President and board member of the Federation.

Quorum

11 Full members out of 12 are attending the meeting (Asociația Charcot Marie Tooth România is absent and didn't send any proxy). The Secretary confirms that the quorum has been reached.

Approval of the minutes of the 2024 General Meeting

The 2024 General Assembly was held in Barcelona on 29th June. The Secretary requests approval of the 2024 General Meeting minutes, which are approved unanimously.

2024 Activity Report (Presented by Filippo Genovese)

A comprehensive overview of the federation's work in the past year was presented.

- ## Board and Administration:

- **Marina's Departure:** Marina Brocca, a long-serving consultant, announced her departure. She was warmly thanked by the board and members for her invaluable contributions since the federation's inception.
- **Administrative Hurdles:** The federation is facing significant issues with its Belgian bank (Belfius), which has frozen operations pending new identity verification rules. This is compounded by a delay in the official publication of updated statutes in the Belgian Gazette, a necessary step to switch banks. Alexander Leysen together with Dany Maes, former treasurer of the Federation, are actively managing this process.
- **Board Diversity:** Filippo Genovese made a strong appeal for a woman to consider joining the currently all-male Board of Directors to bring diverse perspectives.

- ## Awareness Campaign: "Let's Step Together for CMT"

- **Budget & Scope:** A €13,000 budget (fully covered by grants) funded a three-month campaign with social media content, five webinars with specialists and patients, and a multi-language press release.
- **Results:** The campaign achieved significant growth in social media engagement, with follower counts, views, and interactions increasing substantially across Facebook and Instagram.
- **Areas for Improvement:** Key feedback from a member survey indicated a need for better coordination. Specifically, materials for translation were provided with short notice, and member engagement in sharing content could be improved.

- ## Membership & Collaboration:

- **Membership Status:** The federation consists of 12 full members and 9 associate members. Concerns were raised that some full and associate members had not yet paid their 2024 membership fees. This concerns three full members and five associate members who had not yet paid their 2024 dues at the time of the general meeting.

- **Conference Participation:** Board members represented ECMTF at key events, including the EAMDA workshop (Arabela Acalinei), the Eurordis PAG Federations conference (Alexandre Hoyau), and the PNS Meeting in Edinburgh (Filippo, Alex, Simon), where they strengthened pharma partnerships and successfully advocated patient representation on the PNS board.
- **Cross-Border Initiatives:**
    - **CMT-Danub' Challenge:** Benjamin Arnaud presented his inspiring 732km cycling journey along the Danube, which raised over €16,000 for research and fostered collaboration between CMT France, Austria, Slovakia, and Hungary.

    - **The "El motor de tus pasos" fundraising initiative:** Another initiative saw cyclists raise €25,000 for Nervosave Therapeutics.

- **Launch of ECRA (European CMT Research Association)** Presented by Ingolf Pernice: This is a major new organization born from the 2023 1st CMT specialist conference.
- **Mission:** To boost cooperative, multi-stakeholder (researchers, clinicians, patients, industry) research on CMT, support young researchers, and improve access to health data.
- **Status:** ECRA is officially registered and has approximately 40 paying members. It is open to global members.
- **Next Steps:** ECRA is organizing the **2nd European CMT Specialist Conference** in Antwerp in October 2025. All ECMTF members were strongly encouraged to join ECRA.

The Secretary requested approval of the 2024 Activity Report, which was approved unanimously.

2024 Financial Report (Presented by Simon Bull)

- **Income:** Primarily from grants (NMD, Applied Therapeutics) and membership fees.
- **Expenditures:** Major costs included secretariat services (€25,000), the 2024 awareness campaign, and costs associated with the previous General Assembly.
- **Closing Balance:** The year ended with a balance of **€62,725**.

The Secretary requested approval of the 2024 Financial Report, which was approved unanimously.

Discharge of the Directors

The assembly voted to discharge the Board of Directors for their work in 2024.

Renewal of the Board Members

The office term of the Board Members lasts three years. At the end of this period the members of the Board must be reconfirmed. The term of Ingolf Pernice has expired. Ingolf must be re-elected for the next term. Ingolf Pernice is unanimously re-elected.

Actions and Projects 2024/2025

The federation outlined several key strategic initiatives for the coming year.

- **CMT Patient Journey:** A new project in collaboration with the European Reference Network (ERN) to map the complete patient experience with CMT from the first symptoms to care. This will serve as a powerful advocacy and research guidance tool. Arabela Acalinei will lead this effort.
- **European CMT Kids Summer Camp:** Presented by Alexandre Hoyau, this project aims to create a European summer camp for children with CMT, inspired by successful models from CMTA (USA) and CMT UK. The project is in its initial learning phase, with funding already secured from Applied Therapeutics.

- **Antwerp Specialist Conference (Oct 2025):** Practical information regarding accommodation and registration was shared. The reimbursement of travel for members is being explored but depends heavily on the final conference budget.
- **2025 Awareness Campaign:** A budget of €6,000 is proposed. The campaign will focus on storytelling to highlight the disease burden and the challenge of measuring slow progression in clinical trials. A stronger call was made for members to contribute patient videos and stories.
- **EU Advocacy:** The board will explore hiring a professional agency for EU-level advocacy and will support the Eurordis/EPF campaign to ensure patient representation at the European Medicines Agency (EMA).

- **ECMTF Member Organisation Satisfaction & Engagement Survey results** were presented

Clinical Trials Update (Presented by Katherine Forsey, CMTA)

Katherine Forsey provided a highly informative and optimistic overview of the current clinical trial landscape.

- **Applied Therapeutics (Govorestat for CMT-SORD):** Phase 2-3 trial is complete. The company is preparing for regulatory submission, supported by positive MRI data showing slowed nerve damage.
- **NMD Pharma (NMD-670 for CMT1 & 2):** Phase 2a trial is currently recruiting in Europe and the US, testing a drug designed to improve muscle activation and walking speed.
- **Augustine Therapeutics (HDAC6 Inhibitor):** A Phase 1 trial in healthy volunteers has begun for this potentially subtype-agnostic therapy.
- **Actio Biosciences (for CMT2C):** A Phase 1 trial is underway for this precision medicine targeting the TRPV4 mutation.
- **Novartis (DTX-1252 for CMT1A):** DTX Pharma was acquired from Novartis; their promising siRNA therapy is in the final stages of pre-clinical preparation, with an Investigational New Drug (IND) submission anticipated later in 2025.

Other Trials: Updates were also given on trials for HSN1 (L-serine), an N-of-1 trial for CMT2S (Vanda Pharma), and a stem cell therapy in South Korea (Encel).

The meeting was declared closed at 6.00 PM; the ECMTF board members thank everyone who attended.

All presentations and documents from the meeting are available here: https://tinyurl.com/ECMTFGA2025

Bruxelles, 2/9/2025

Filippo Genovese
President

Marco van der Linden
Secretary