

1st European Charcot-Marie-Tooth Specialists Conference

organized by the
European Charcot-Marie-Tooth Federation
Venue : French Ministry for Health and Prevention

Draft programme

« Together against CMT »

9 & 10 June 2023



PARIS



EUROPEAN
CMT
FEDERATION

- ecmtf.org
- cmt-awareness.com



Editorial :

“**Together against CMT**” is the watchword of our initiative to bring together European scientists, clinical researchers and therapists specialized in CMT, to join together their efforts in developing effective causal therapies for this rare and orphan disease.

The **European CMT Federation** uniting most of the CMT advocacy groups across Europe, aims to stimulate and promote interdisciplinary cooperative research in the interest of all patients affected by this disease.

We simultaneously strive to **mobilize the patients** to cooperate more closely with the specialists involved and to contribute when they can (in studies and with their data) to broaden the basis for successful research.

The different groups of specialists no doubt already have their own gatherings and schemes of cooperation, but for the first time we call on you to create synergies and innovative ideas **in a joint conference**.

And we are happy to include representatives of the profession and of organizations from overseas in order to learn from their experience and stimulate cooperation **on a global level**.

Friday 9th June :

Draft Programme (proposed names and functions to be confirmed)

13.00

Registration – refreshments /exhibitions

14.00

Opening Plenary :

- Welcome : Daniel TANESSE, President of ECMTF (CMT-EUROPE)
- Greetings : Gilles BOUCHARD, President of CMTA
- Introduction : « CMT as a common challenge for scientists, clinicians and therapists – disciplinary cooperation and potential of interdisciplinary synergies »

 **European** projects/perspectives : Shahram ATTARIAN

American projects/perspectives : Stefan ZÜCHNER 

15.00

Working Groups (3 presenters, 15' each)

Three disciplinary approaches to problem solving

1. **Bio- and medical sciences : from data (management and analysis) to therapies**

Given the potentials of digitization and bio-informatics in basic and applied sciences in the field of genetically caused neuropathies, including CMT, the aim of the workshop is to establish the state of the art developments in this field, and to assess the role that health data generally, as well as genetical data in particular, play for researchers working on the development of causal therapies of CMT.

- Co-moderation : Michael SEREDA & Filippo GENOVESE
- Rapporteur : Alexander ROSSOR
- Presenters : (1) Davide PAREYSON / (2) Francesc PALAU MARTINEZ / (3) Wolfgang Maximilian PERNICE / Helena F. PERNICE

Friday 9th June :

2. Clinical diagnosis and treatment : Potential of genetic analysis and engineering

The aim of the workshop is to shed some light on the state of the art applied sciences for the diagnosis and treatment of CMT. Clinicians with specific research interests in genetic analysis and engineering are invited to report recent achievements, expectations and perspectives of the progress of science on CMT and its complex genetic causes.

- Co-moderation : Peter DE JONGHE & Arabela ACALINEI
- Rapporteur : Maike DOHRN
- Presenters : (1) Valérie DELAGUE / (2) Angelo SCHENONE / (3) Jan SENDEREK

3. Therapeutic practice and feedback : Challenges and specific tasks for the therapists

The aim of this workshop is to understand the fundamental role clinical and independent therapists – physio-, logo-, ergotherapists and nutritionists – play in the treatment as well as in research on CMT, in order to establish how therapists contribute not only to the well-being of each patient but also to the study of CMT through their daily observations and collected data on the effect of specific therapies (or new drugs?).

- Co-moderation : Carsten SCHRÖTER & Alexandre HOYAU
- Rapporteur : Roberto CANNATARO
- Presenters : (1) Cale FABIEN / (2) Gita RAMDHARRY / (3) Joshua BURNS

16.30

Coffee break / Exhibitions

17.00

Plenary II (chair : Mary REILLY)

- Podium : Highlights from the working groups (10' each Rapporteur)
- General discussion : key insights and learnings and then open questions

18.30

20.00

Aperitif

Venue : **Maison de l'Amérique Latine** – Paris, 7th Arrondissement

20.30

Gala Dinner (with dinner-speech by Health Minister of France or EU Commissioner)

Venue : **Maison de l'Amérique Latine** – Paris, 7th Arrondissement



MAISON DE L'AMÉRIQUE LATINE

1. Cooperation : Establishing a “European CMT Research Association (ECRA)”

With a view to institutionalizing closer cooperation among research centers and scientists in Europe we would hope that the workshop can come up with some guidelines and provisions for how scientists and researchers, clinicians and therapists with a scientific aspiration can organize synergies of their efforts, eventually by setting up a European CMT Research Association aiming at stimulating the process of CMT research.

- Co-moderation : Laurent MAGY & Dany MAES
- Rapporteur : Liliane MASSADE
- Presenters : (1) Gian Maria FABRIZI / (2) Teresa SEVILLA MANTECÓN / (3) Lisa REINECKE

2. Data management for CMT : Towards an Integrated European CMT Patient Registry

Research on CMT often suffers from the very limited access to data; as CMT is a rare disease even for specialized clinicians CMT patients are rare. This means researchers depend on data from many other centers specialized on CMT in order to reach a minimum sample of data to base their studies upon. Patient registries, either general ones on neuromuscular diseases or specialized for CMT, exist in some countries. But they are organized according to different criteria and standards, so there is no interoperability and their usefulness is limited.

The aim of the workshop is to determine the need for quality data to be made available to researchers as a basis for promising research efforts, and to develop proposals for how to have a coherent system of compatible registries which makes available all the data needed.

- Co-moderation : Alessandra BOLINO & Marco VAN DER LINDEN
- Rapporteur : Andrea CORTESE
- Presenters : (1) Jean-Christophe ANTOINE / (2) Simone THIELE / (3) Theresinha EVANGELISTA

3. Mobilizing the Patients : Scientists, Clinicians and Therapists expectations and advice

CMT patients often seem to be reluctant to take a stake in the development of research and treatment of their disease, let alone raising public awareness and understanding in politics for their special problems and needs. The aim of this workshop is to find methods and mechanisms for mobilizing the patients in their own interest. Mobilization means that we would wish for more of them to join our patients advocacy organizations and efforts of self-help. It also means closer cooperation with the scientists, clinicians and therapists in studies and by making their data available to researchers, e.g. through patient registries or apps. There may be many other ideas on how to use the potential that the mobilization of patients can offer for the benefit of more efficient research and, finally of a better quality of life for the patients themselves.

- Co-moderation : Tanya STOJKOVIC & Inbal BEN DAVID
- Rapporteur : Signe VAETH
- Presenters : (1) Raluca Loana TELEANU / (2) Kleopa KLEOPAS / (3) Nicolas TRICAUD

Saturday 10th June :

10.30 Coffee break / Exhibitions

11.00 **Plenary III** (President : Peter YOUNG)

- Podium : Reports from the working groups (10' each Rapporteur)
- Comments : An American Perspective : Katherine FORSEY (CRO of CMTA)
- Final plenary discussion

12.30 **General Conclusions and Perspectives** (Vincent TIMMERMAN / Ingolf PERNICE)

13.00 **Press Conference**

