PIONEERS IN BOOSTING RESEARCH ON RARE DISEASES

PRESS RELEASE

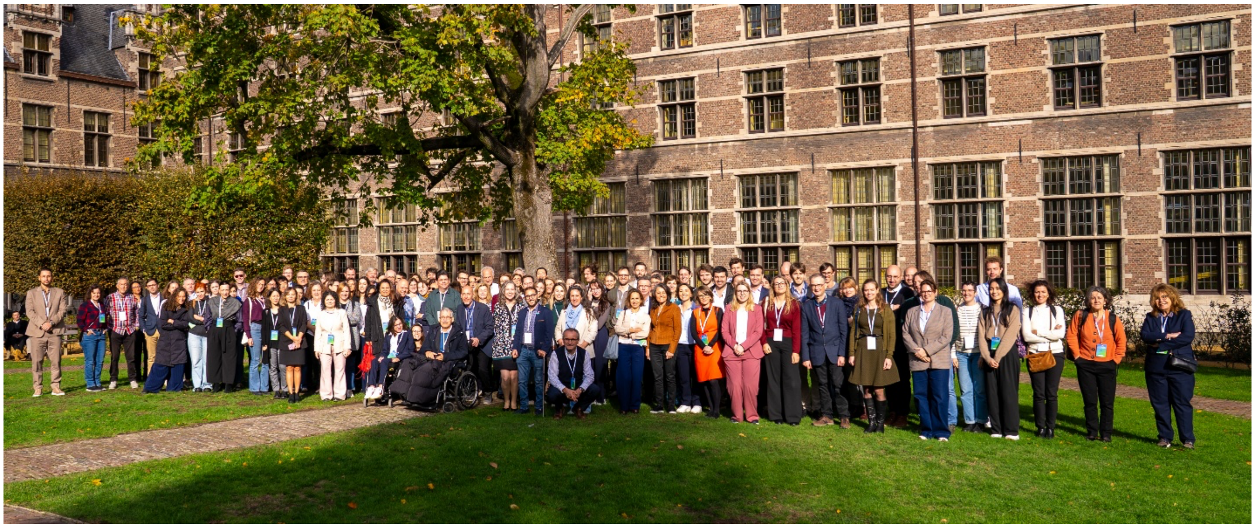
BERLIN, NOVEMBER 11, 2025

Driven by a patients' initiative, the **2nd European Charcot-Marie-Tooth Specialists Conference** was held in Antwerp, Belgium, from October 23 to 25, 2025.

Charcot-Marie-Tooth disease (CMT) is an inherited disease of peripheral nerves, which leads to progressive muscle weakness and sensory disturbances. The daily life consequences are gait problems such as stumbling and falls, up to wheelchair dependence, and impaired motor skills affecting activities such as writing, eating with cutlery, and many more. Many genetic causes of CMT remain to be discovered, and the mechanisms of nerve damage need to be understood. As of now, there is no approved disease-modifying treatment on the market.

This conference was a common project of the European CMT Federation (ECMTF), the University of Antwerp as the host, and the newly established European CMT Research Association (ECRA), to stimulate cooperative research on CMT. Building on the success of the EJPRD-funded 1st European CMT Specialists Conference (Paris 2023), it aimed to leverage the diverse group of Charcot-Marie-Tooth diseases as a model to create an urgently needed forum for EU-wide coordination to confront key roadblocks shared across the field of inherited neuromuscular diseases (iNMD), and rare diseases in general.

With 136 highly committed participants, top scientists, early stage researchers, clinicians, patient- and industry representatives from Europe and Israel, but also from the United States, Brazil, and South Korea, the conference was a unique experience of a vibrant multi-stakeholder conversation about new ways to boost biomedical research and address unmet needs in the field of inherited neuromuscular diseases and CMT in particular.



The organizers are grateful for the support of the EU Commissioner for Health, Olivér Várhelyi, who encouraged the participants with a video message at the Opening Ceremony, and for generous grants under the EU4Health program, and for support from diverse institutional and private sponsors. Highly committed researchers presented and discussed topical results and new projects in basic sciences on CMT, groundbreaking research on diagnostics and genetics of CMT neuropathies, diverse new therapeutic approaches including genetic engineering, initiatives for accelerating clinical trials, the development of patients registers and data sharing, AI driven diagnostics and outcome measuring, advanced methods in physiotherapy and an innovative approach on digital care to ensure better access to health for patients in remote regions, far away from specialized centers. All this was done in a spirit of cooperation with "patients as partners", but also with the industrial partners present. It allowed concrete proposals to be developed also for improving the framework conditions for more effective action against CMT, such as:

- Recommendations for specific CMT/iNMD-related training programs and a model CMT online seminar
- A data sharing framework for health data management, ensuring free access for researchers to the data they need
- A digital care strategy facilitating patient access to specialists and treatment, and a strategic collection of relevant health data
- A model joint research project including patients and industrial partners as a step towards advanced research funding

The program with all the abstracts of presentations and posters is still available at:

https://www.uantwerpen.be/en/conferences/2nd-european-cmt-specialist-conference/.

Also a link to the videos of three pre-conference webinars and of selected speeches and presentations at the conference, as well as a photo-documentation giving an impression of the highly concentrated work at this unique event, will soon be found on the conference website and on the ECMTF YouTube channel.

If you wish to know more about the idea and outcomes of the Conference, please join us at the **Post-Antwerp Webinar,** titled

Towards a cure for CMT? Takeaways from the Antwerp Conference: Highlights from research. What are the promises, what was missing, what should be the next steps?

with the host of the event, **Prof. Vincent Timmerman** (Antwerp University) for the scientists' perspective, the coordinator of the conference, **Prof. Ingolf Pernice** (ECMTF) representing the patients' perspective, and, acting as the moderator, **Dr. Maike Dohrn** (University Hospital Aachen). Maike Dohrn will comment and give answers to questions of the participants from the clinicians' perspective.

Registration is open at the link https://tinyurl.com/ECMTFPostAntwerpWebinar.

Filippo Genovese Vincent Timmerman Ingolf Pernice

We are most grateful for the generous support of this Conference by:

Flanders
State of the Art

Co-funded by the
European Union

APPLIED
THERAPEUTICS

fwo

AFMTÉLÉTHON
CURE THROUGH INNOVATION

DGM
Deutsche Gesellschaft
für Muskelkranke e.V.

ULYSSES
NEUROSCIENCE

NMD PHARMA®
NEUROMUSCULAR DISORDERS

Any donation to make the work initiated at the Conference a success and, in particular, to secure the implementation of the Decisions taken in Antwerp through ECRA is highly appreciated.

Donate Now

Background Information

CMT, also known as HMSN, is a hereditary, slowly progressive peripheral neuromuscular disease that often manifests in childhood. The common clinical features are progressive muscle wasting and weakness, together with sensory loss starting in the feet and progressing more proximally, including the hands. Foot deformity is common. The gait becomes clumsy, and twisting an ankle and stumbling are typical, even in children and adolescents. Muscle pain, fatigue, and increasing loss of mobility are common – and over time, the need for walking aids, walkers, and sometimes wheelchairs follows. Many doctors are not familiar with the disease, its typical symptoms, and neurological signs, which can lead to a delay in diagnosis. There is still no effective therapy for CMT, but there are many research efforts ongoing. However, obstacles to developing therapies include a lack of awareness of the disease, a lack of financial support, the low profitability prospects for the industry, and the unavailability of sufficient data for researchers. This is typical also for the approximately 8000 other rare diseases.

Due to the relatively low number of patients with CMT, meaningful research requires cooperation across national borders. European-wide, even global networking and collaboration are imperative. Thus, cross-border research groups have been formed to enable projects and later publications on specific aspects. Among the EU-funded European Reference Networks (ERN), there is the network for neuromuscular diseases (EURO-NMD), to which CMT is assigned. The ERN aims to bring together highly specialized hospitals and reference centers to provide experts with a forum for exchanging experiences and advice on diagnosis and the best available treatment. Specialists in CMT also meet at the annual conferences of the International Peripheral Nerve Society (PNS) in a particular working group, the CMT and Related Neuropathies Consortium (CMTR), to discuss the latest research highlights. All of this facilitates the scientists to find collaborative partners for their research interests. Many important achievements in research have already been made in this way. Yet, there is still no cure for any of the many variants of CMT.

The European patient advocacy groups, jointly organized in the ECMTF, have therefore taken a new initiative which has been well received by many people working in research and clinical practice, as well as in industry and politics. By institutionalizing cooperative research in the form of a European CMT Research Association (ECRA), the interests of researchers can be combined, and joint research projects can more easily be organized, mentored, and supported. This includes assistance in applying for research funding, so that young talent can be integrated into the field of CMT and relevant companies engaged in the development of innovative therapies.

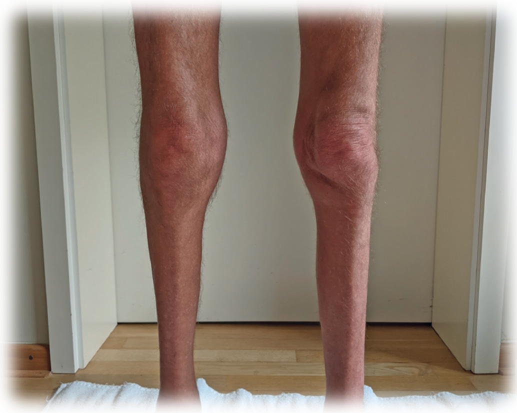
ECRA was founded to provide a multi-stakeholder forum and support structure for developing interdisciplinary cooperation and joint projects in close partnership with patient organizations and industry. Accordingly, the goals of ECRA include:

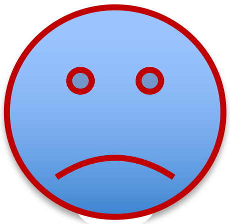
- pooling the expertise of highly motivated researchers, clinicians, and other health professionals through forums and forms of structured interdisciplinary cooperation, including young scientists;
- mobilizing the ‘patients as partners’, from self-help groups and local assistance schemes to active collaboration with researchers;

in order to:

- mobilize the patients and put their care on a new footing of self-responsibility and commitment on the way to effective remedies for their disease;
- ensure better access for researchers to patient data through an interoperable system of patient registries and data in the European Health Data Space (EHDS);
- boost research funding through closer cooperation with industrial partners in joint research and development projects on CMT.

The Antwerp Conference was for ECRA an opportunity to celebrate its own inauguration, have its first General Meeting, and start its operational work. One of its first tasks will be to ensure the implementation of the decisions taken at the Conference.





Deformed feet ?
Thin & weak legs ?
Hollow hands ?

It could be CMT

Together we are stronger
Let's step together to defeat CMT !