



A European forum to align clinical care, outcome measures, data sharing, therapeutic development and patient partnership in CMT and inherited neuromuscular diseases

1. RATIONALE AND CONFERENCE ARCHITECTURE

Strategic objective

Identify shared system-level roadblocks and opportunities to improve clinical care and accelerate biomedical research for rare inherited neuromuscular diseases, using CMT as a model.

Core roadblocks addressed

- Low public and clinical awareness; delayed diagnosis.
- Incomplete genetic landscape and variant interpretation.
- Need for pathomechanism-based therapeutic pipelines.
- Insufficiently sensitive clinical outcome assessments for trials.
- Data-sharing barriers and limited trial-ready cohorts.



2. SCIENTIFIC PROGRAMME

Programme blocks

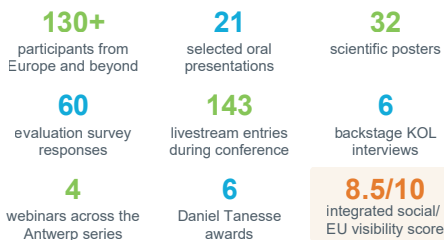
- Opening session: EU health policy, ERN/Euro-NMD, diagnostic challenge, industry and patient perspective.
- Basic sciences and the many faces of CMT.
- Diagnostics, genetics, sensors, robotics and AI.
- Therapeutic approaches, gene therapy and pharmacology.
- Clinical trials, data sharing and outcome measurement.
- Round table on access to therapy and reimbursement.
- Open session: physiotherapy, digital care and patient partnership.

The conference combined plenary sessions, fishbowl idea workshops, poster sessions, live-streaming, backstage interviews and public webinars to connect scientific exchange with implementation.

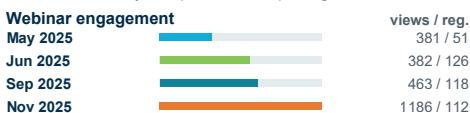
Watch the recorded sessions and the interviews on **YouTube**



3. KPI DASHBOARD



Preparation and dissemination included a call for abstracts, reference materials, patient-readiness survey, multilingual awareness activity and post-event reporting.



11. CORE MESSAGE

4. TRANSLATIONAL SCIENCE HIGHLIGHTS

Diagnostics and clinical phenotyping

- Long-read sequencing reported a 28% diagnostic uplift in previously unsolved CMT cases.
- AI-assisted differential diagnosis explored CMT versus mimics such as CIDP, with performance up to 87% in one study.
- Sensor-based gait and hand assessment, robotics and telemedicine were positioned as objective monitoring tools.

Therapeutic pipeline and experimental models

- iPSC-derived motor neuron and muscle-sphere hybrid models to study myelination and neuromuscular junction mechanisms.
- Pharmacological approaches: SORD/govorestat, L-serine, NMD670, HDAC6 inhibition, SARM1, TRPV4 modulation.
- Gene-therapy and gene-editing approaches for CMT1/4 and CMT2, including PMP22 modulation and MFN2 correction.
- Rehabilitation-oriented markers including plantar fascia ultrasound and structured self-management.

Clinical trials and outcomes

- DANCER proposed AI-supported video tracking for scalable patient-partnered outcome assessment.
- Outcome standardization and biomarkers as prerequisites for trial feasibility and regulatory confidence.

5. CONCRETE DELIVERABLES

Antwerp moved beyond discussion by producing implementation-oriented materials for ECRA and the wider CMT research ecosystem.

- D2.1-D2.2** Medical training programme for CMT: recommendations and online seminar outline
- D3.1** Data-sharing framework aligned with GDPR and the European Health Data Space
- D3.2** AI in CMT: diagnostics, research and outcome assessment
- D3.3-D4.3** Model multistakeholder joint research project
- D4.1** Towards rehabilitation guidance for adults living with CMT
- D5** Outcome measures and standardization of clinical outcome assessments
- D6.1** Digital care strategy for CMT across Europe and beyond
- D6.2** Cooperative R&D for CMT: patients as partners

Download

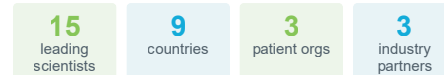
the Conference Deliverables



6. MULTISTAKEHOLDER MODEL

CureCMT Doctoral Training Network

A model project translating the Paris-to-Antwerp trajectory into a trial-readiness programme for rare CMT neuropathies.



European Patient Journey in CMT

Route to earlier diagnosis and care, with support for expansion towards a Europe-wide joint project and cost-of-illness assessment.

7. EVALUATION AND PUBLIC IMPACT

Participant evaluation

60 survey responses from participants documented an overwhelmingly positive assessment, with recurring emphasis on networking, cross-disciplinary breadth, high scientific level and attention to the patient perspective.



Dissemination performance



Public outputs: conference website, programme and abstracts, pre/post webinars, plenary recordings, best-of video, photo gallery, backstage interviews, post-event report and ECMTF article.

8. FROM ANTWERP TO IMPLEMENTATION

Follow-up priorities

- Adopt and execute the ECRA 5-year action plan.
- Develop interoperable registries and research access to data.
- Prepare ENMC workshops and multi-country project proposals.
- Move standardized COAs and digital outcome measures towards trial use.
- Integrate digital care and patient readiness into trial recruitment pathways.



The long-term objective is not another isolated meeting, but a coordinated research and care infrastructure able to support earlier diagnosis, trial-ready cohorts, regulatory-grade outcomes and patient-partnered research.

9. JOIN ECRA!

European CMT Research Association

ECRA membership is open to scientists, clinicians, other health professionals, patient advocates and industry partners from Europe and beyond.

- Incubate joint research projects and grant applications.
- Mentor next-generation researchers.
- Coordinate webinars, workshops and data initiatives.
- Build trial-ready cohorts with patients as partners.



10. NEXT EUROPEAN CONFERENCE

SAVE THE DATE

3rd European CMT Specialists Conference
Cyprus | 21-23 October 2027

The Antwerp outputs define the working agenda for the next cycle: ECRA consolidation, stronger data infrastructure, clinical trial readiness and sustained European coordination.



Antwerp converted a rare-disease conference into a practical implementation platform

The operational shift: move from fragmented expertise to an integrated European model where clinicians, researchers, patient organizations, industry and policy actors co-develop infrastructure for earlier diagnosis, trial feasibility and equitable access to future therapies.

Working principle: patients as partners - data as infrastructure - outcomes as regulatory currency - ECRA as implementation engine.

