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Model multistakeholder joint research project

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The 2nd European CMT Specialists Conference, held in Antwerp October 23 to 25, 2025, brought together more than 130 scientists, clinicians, other health professionals, patient advocacy groups, representatives from the pharmaceutical industry and policy-makers for the joint effort to boost research on CMT by setting up a structure of cooperation and partnership aiming at, finally, developing a cure against CMT. It so developed further what already was the philosophy of the 1st European CMT Specialists Conference, Paris 2023, the **“partnership approach”** including “patients as partners” and “industrial partners” at eyes level in the research process.

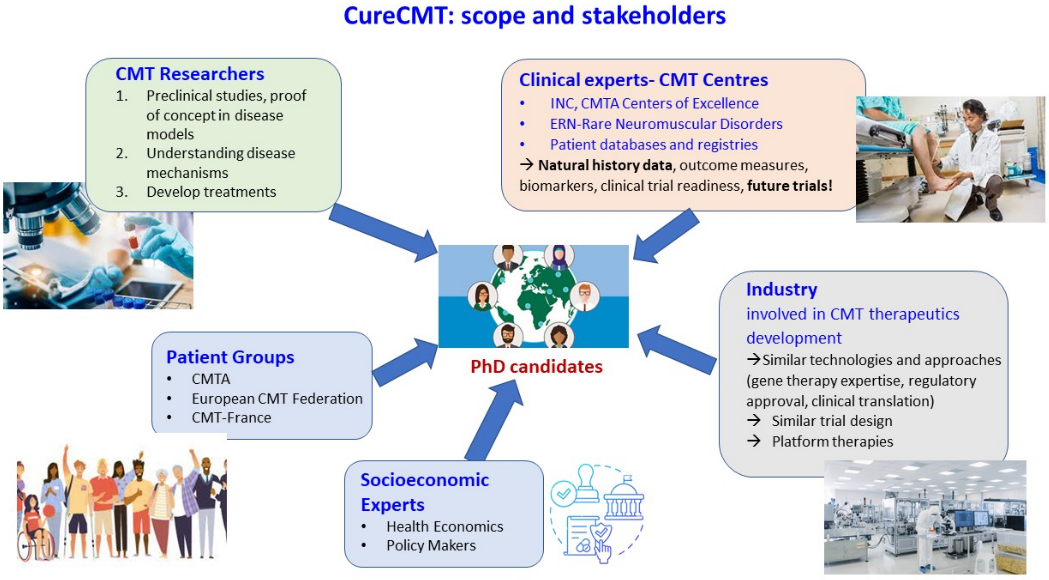
It was a privilege for all participants to see Prof. Kleopas Kleopa from Cyprus presenting the **CureCMT Doctoral Training Network**, and this amazing project found tremendous recognition, applause and support. Three new participants could be added to the project during the Conference. Given its multidisciplinary scope and its cooperative structure CureCMT is herewith presented **as a first model joint research project** based upon the novel partnership approach, which is promising far beyond the narrow field of CMT as a model for many other inherited neuromuscular diseases and rare diseases at large, that face similar challenges to effective research.

This outstanding proposal was meanwhile submitted for funding under the EU MARIE SKŁODOWSKA-CURIE ACTIONS program by a European team under the lead of Kleopas Kleopa, Cyprus, and it is hoped to be accepted and can be implemented as soon as possible.

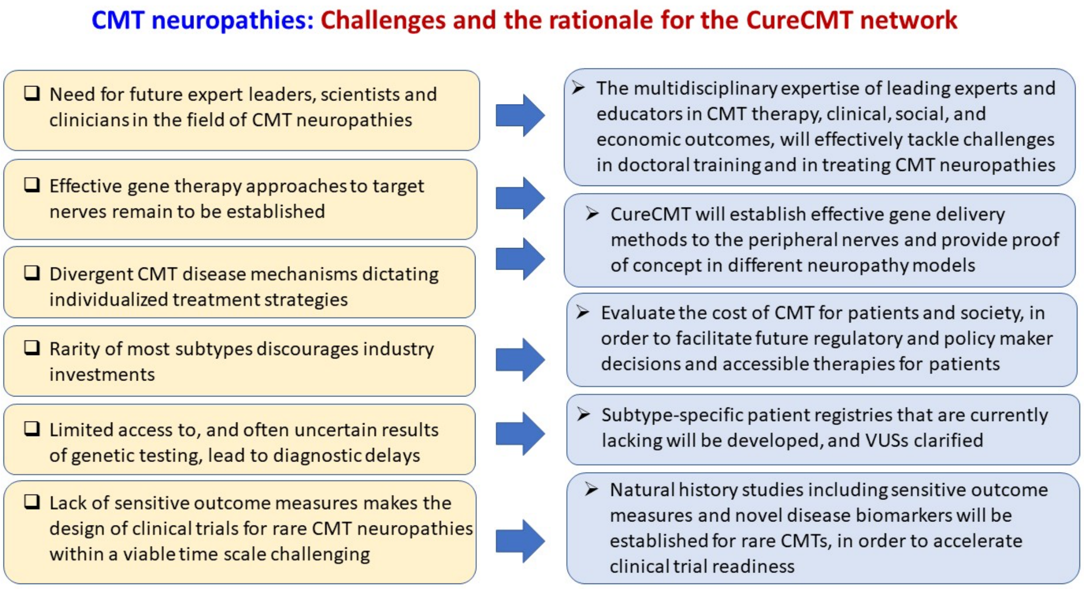
CureCMT combines a multidisciplinary doctoral training program with the topical challenge of developing effective treatments for several subtypes of CMT, in close partnership with patients and industry. It is a test case and, if successful, will serve as a model for many other projects on rare diseases.

Composition and scope

The collaborative network of 15 leading scientists and clinicians from 9 countries (Cyprus, France, Belgium, Bulgaria, the Netherlands, Spain, Italy, UK and Turkey) includes neurologists, geneticists, molecular biologists, computational scientists, bioinformaticians and even economists as well as legal and business experts, all leading researchers in the field of CMT in Europe. CureCMT so guarantees multidisciplinary expertise. With the active participation of three patient organizations (the European CMT Federation, the U.S. CMT Association and CMT-France) and three industrial partners (Augustine Therapeutics, Belgium, ArmatusBio,USA, and XtRNA from the Netherlands) all stakeholder groups relevant in CMT research are represented. 15 PhD students will be recruited at the participating research centers to form an international cooperative research group. Within the framework of an intense common training program, together they will pursue their respective projects that reach from preclinical models following the entire pathway up to feasible clinical translation and socio-economic studies on the cost of CMT.



CureCMT will include the establishment of CMT subtype-specific patient registries and natural history studies to enable an adequately powered and sensitive clinical trial design and the validation of clinically relevant biomarkers to serve as surrogate outcome measures. The scope of the project follows the specific challenges and unmet needs that are characteristic for rare diseases, and CMT with its many subtypes in particular. The following Figure shows some of the key issues and how the project intends to tackle them:



CMT neuropathies: Challenges and the rationale for the CureCMT network

- ☐ Need for future expert leaders, scientists and clinicians in the field of CMT neuropathies
- ☐ Effective gene therapy approaches to target nerves remain to be established
- ☐ Divergent CMT disease mechanisms dictating individualized treatment strategies
- ☐ Rarity of most subtypes discourages industry investments
- ☐ Limited access to, and often uncertain results of genetic testing, lead to diagnostic delays
- ☐ Lack of sensitive outcome measures makes the design of clinical trials for rare CMT neuropathies within a viable time scale challenging

- ➢ The multidisciplinary expertise of leading experts and educators in CMT therapy, clinical, social, and economic outcomes, will effectively tackle challenges in doctoral training and in treating CMT neuropathies
- ➢ CureCMT will establish effective gene delivery methods to the peripheral nerves and provide proof of concept in different neuropathy models
- ➢ Evaluate the cost of CMT for patients and society, in order to facilitate future regulatory and policy maker decisions and accessible therapies for patients
- ➢ Subtype-specific patient registries that are currently lacking will be developed, and VUSs clarified
- ➢ Natural history studies including sensitive outcome measures and novel disease biomarkers will be established for rare CMTs, in order to accelerate clinical trial readiness

Structure and partnership approach

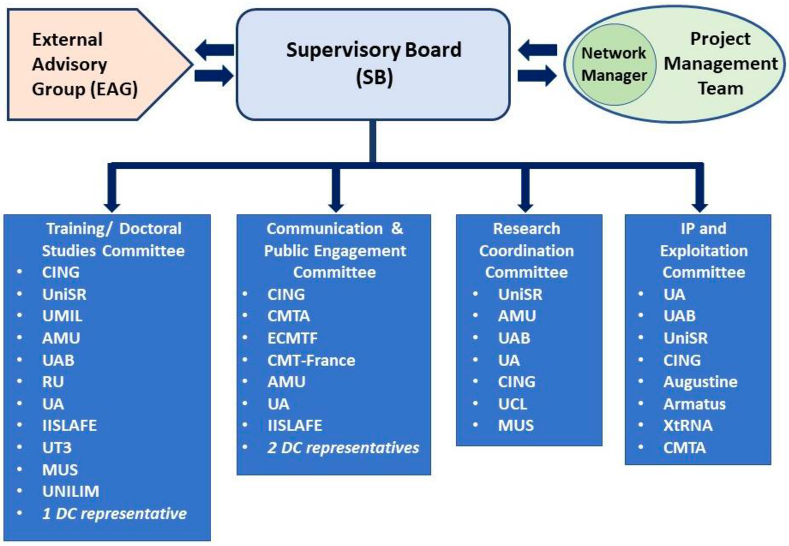
The basis of the project will be a multidisciplinary doctoral training program specifically designed to provide a framework for intensive training in the most advanced methods and discoveries in the field, and for mutual exchange and learning among the Doctoral Candidates (DCs). A consortium of leading scientists and clinicians, supervisors of the diverse PhD projects, along with industry experts and patient representatives provide all the necessary expertise, cross-sectoral transferable skills, and experience in training successful DCs towards full productivity and academic growth. Three one-week summer schools, and yearly 2-day Scientific Meetings and joint activities organized with other relevant networks and associations bring the group of DCs regularly together and allow the group to develop its own dynamics and synergies.

Current doctoral training in CMT research often lacks a multidisciplinary dimension, faces limitations in acquiring new technological advances for therapy design and delivery, and includes limited interaction between basic and clinical aspects of CMT research, that could cross-fertilize new treatment developments. CureCMT will close these training gaps as well as facilitate the currently limited interaction of researchers with the CMT patient community and associations as well as with relevant Industry, the major stakeholders in translation of therapeutics. It offers the added value of a consortium with wider expertise and resources than any single center or research group.

Patient associations as well as industry partners already participated in designing the project; they will be directly involved in the training program and play their role in the governance structure of CureCMT. For the first time, patients with their natural disease history and personal experience are being included as full participants in a research process involving multiple stakeholders; the inclusion of industry representatives ensures that their experience is utilized and, in particular, that the conditions for translating scientific findings into marketable products are not lost sight of.

Governance

CureCMT has a Supervisory Board (SB) as the central body of the network, with an External Advisory Group (EAG) and the Project Management Team (PMT) on its sides. Specialized Committees are established to facilitate the implementation of the objectives of the network. They report to the SB.



The overall management of CureCMT will be monitored by the Supervisory Board that consists of the supervisors of the 15 DCs. It takes its decisions by majority vote. The Network Manager, a full-time officer of the PMT, serves the SB as a secretary and prepares the agenda and the minutes of its meetings.

The PMT with the Project Coordinator and the local support staff based at the Coordinating Organization (Cyprus Institute of Neurology and Genetics) is responsible for the day-to-day management of the Network, the financial record keeping, the planning and arrangements of meetings and training evens, the planning of doctoral studies, any IP and exploitation related issues, and the completion of regulatory and contractual obligations of the Network. It will report to the SB and organize and monitor the activities of the various Network Committees.

Network Committees are formed as part of the management plan with the task of facilitating the implementation of the objectives of the network. They include the Training/Doctoral Studies Committee, the Communication and Public Engagement Committee, the Research Coordination Committee, and the IP and exploitation Committee. Each committee consists of delegates of participating research centers and, as appropriate, also of representatives of the patient organizations and industrial partners. The committees receive support by the Project Manager and report to the SB.

The External Advisory Group will consist of prominent leading scientists and clinicians in the field. Their task is to provide independent guidance to the overall Network Strategy and progress, as well as to ensure the quality of the Networks training and research activities.

Conclusion

CureCMT combines its ambition to boost research in CMT with a practical initiative to systematically promote young talents and prepare them for an international career as leaders in both academic and industrial positions. Its multidisciplinary character, its international and cooperative structure and its openness for including patients and industry as partners, with their respective knowledge and experience, in the training and research process promise considerable added value and witness an extraordinary spirit of innovation setting a precedent for many future research projects in the field of rare diseases.