

## 2<sup>nd</sup> European CMT Specialists Conference Antwerp, 23-25 October 2025



### BOOSTING RESEARCH ON RARE DISEASES

## EVALUATION REPORT

BERLIN, NOVEMBER 11, 2025

The 2<sup>nd</sup> European Charcot-Marie-Tooth Specialists Conference was held in Antwerp, Belgium, from October 23 to 25, 2025. It 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 give research on CMT a new impetus by joining efforts of scientists, clinicians with patients and industry as partners. Initiated by patients the aim was to leverage the diverse group of Charcot-Marie-Tooth diseases as a model to create an urgently needed forum for EU-wide coordination of the relevant stakeholders to confront jointly key roadblocks shared across the field of inherited neuromuscular diseases (iNMD), and rare diseases in general.

The program with the abstracts of the presentations and posters, as well as the preparatory materials, the links to three pre-Antwerp webinars and one post-Antwerp webinar, as well as video-takes of selected talks and presentations, video-interviews exploring the challenges of research in the field and a “best-of” movie reflecting the atmosphere among the participants remain, accessible at the conference website (<https://www.uantwerpen.be/en/conferences/2nd-european-cmt-specialist-conference/>).

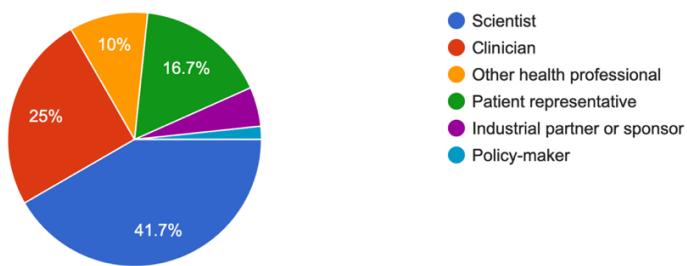
The more than 130 CMT specialists present were invited after the Conference to evaluate the event by an anonymous online-survey (Google forms). It addressed four categories of questions and ended with an invitation to comment what was liked most, what was disliked and what participants would wish for a future conference. A general part regards the composition of, and the first information about the conference, the invitation management and the topicality of the subject (Q.1-4). Part 2 is about the innovative approach, concept and organization (Q. 5-6). Part 3 focuses on quality and

the scientific level of each plenary session (Q.7), while Part 4 is about the cost-benefit and an overall evaluation of the event (Q.8-9).

60 participants replied as follows:

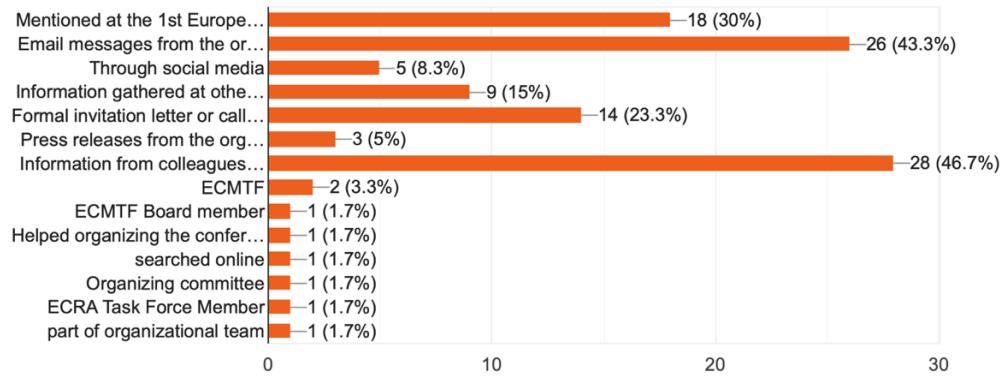
### 1. Your Role at the Conference

60 responses



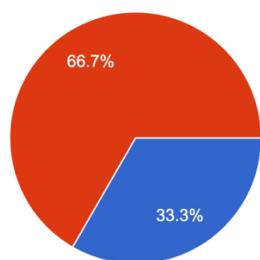
### 2. How Did You Learn About the Conference?

60 responses



### 3. Invitation Management and Scheduling

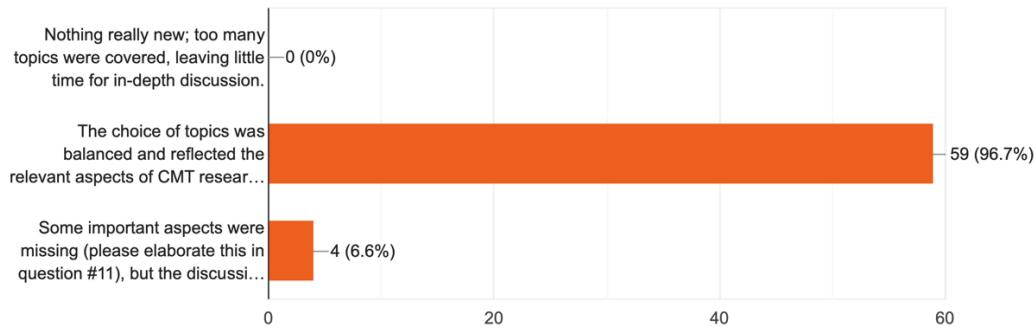
60 responses



- The information reached me too far in advance of the conference date.
- I learned about the conference just in time to save the date and prepare my participation.
- The formal invitation arrived very late, making it difficult for me to attend

#### 4. Topicality and Relevance of the Conference

61 responses



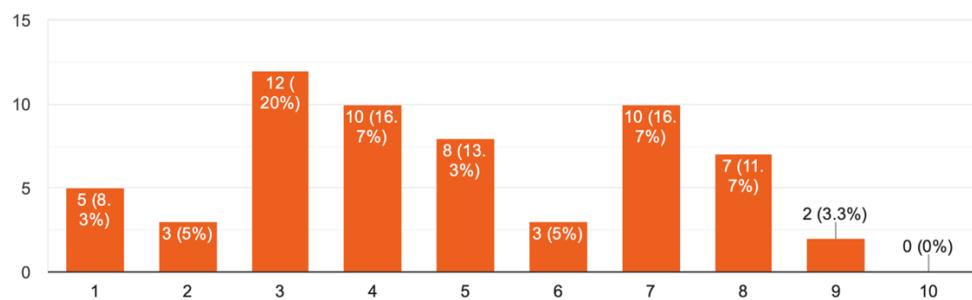
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#### 5. Innovative Approach of the Conference

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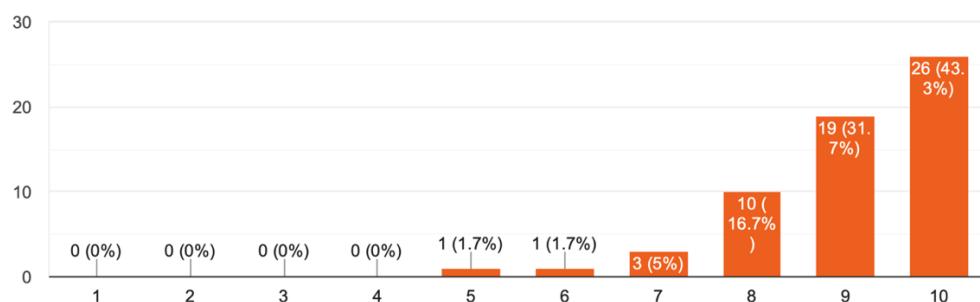
##### a. The conference was similar to other gatherings in the field

60 responses

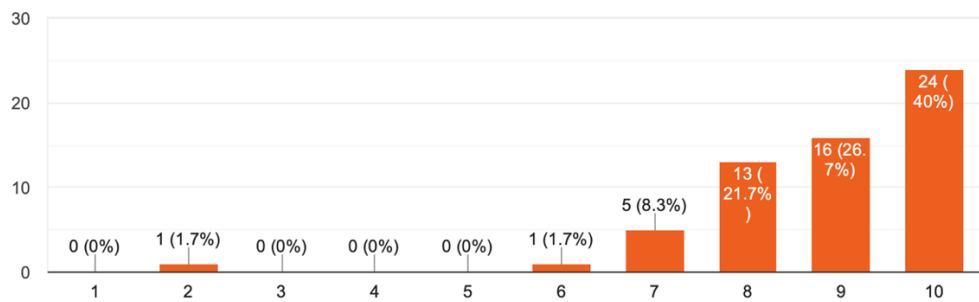


##### b. The collaborative approach (multi-stakeholder, patients as partners) is innovative and highly promising for advancing research in CMT and iNMDs

60 responses

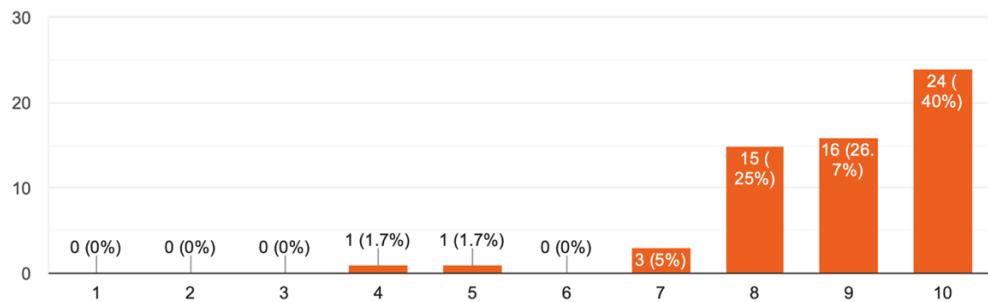


c. The conference design should serve as a model for promoting research on other rare diseases:  
60 responses

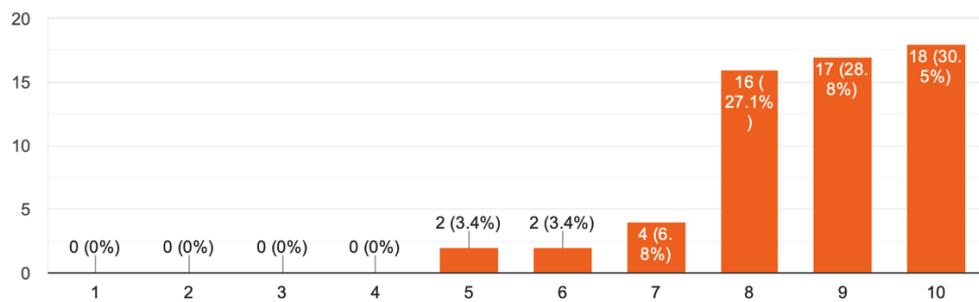


## 6. Preparation and Organization

a. Information and invitation management – the conference website  
60 responses

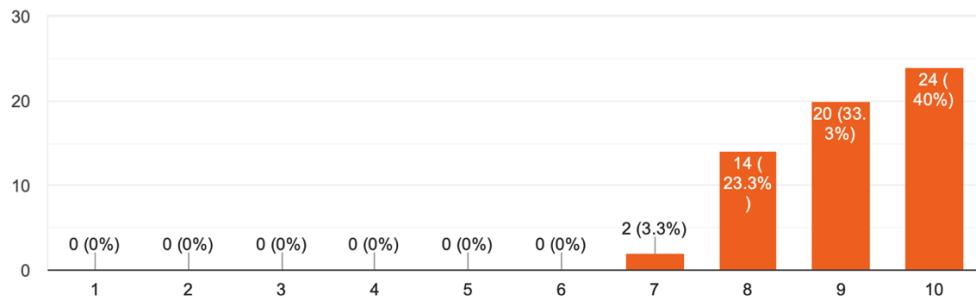


b. Accommodation information  
59 responses



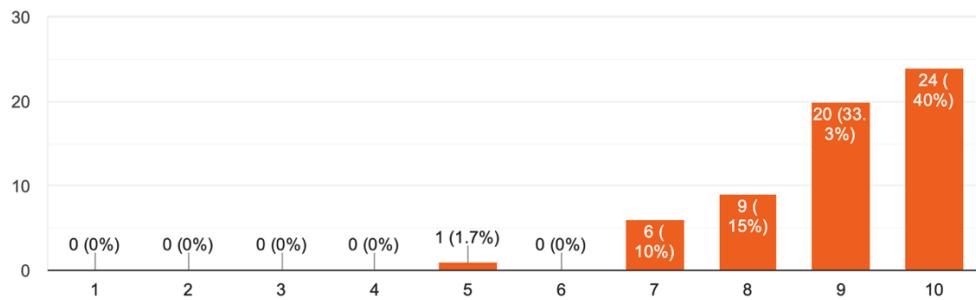
**c. Registration and financial management**

60 responses



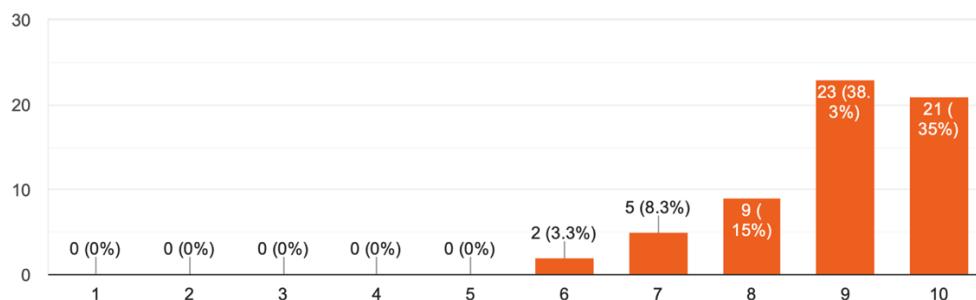
**d. Call for abstracts and conference programming**

60 responses



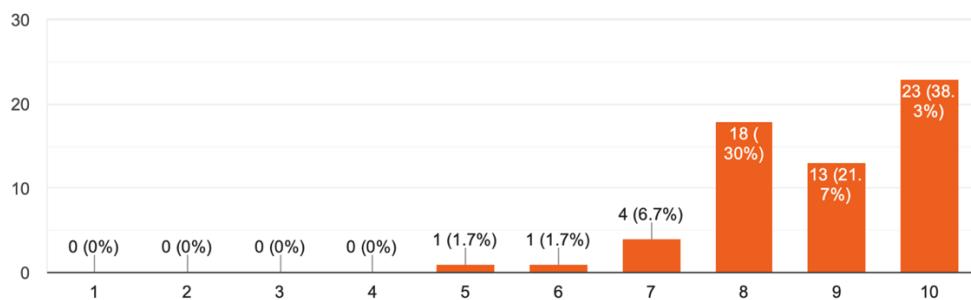
**e. Documentation and preparatory materials provided**

60 responses



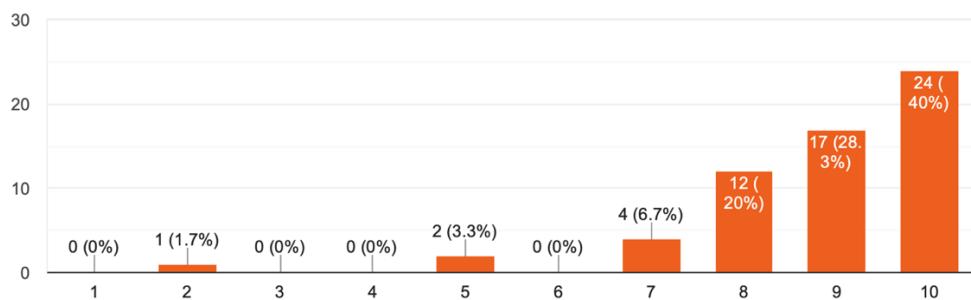
**f. Quality and timing of catering, food, and drinks**

60 responses



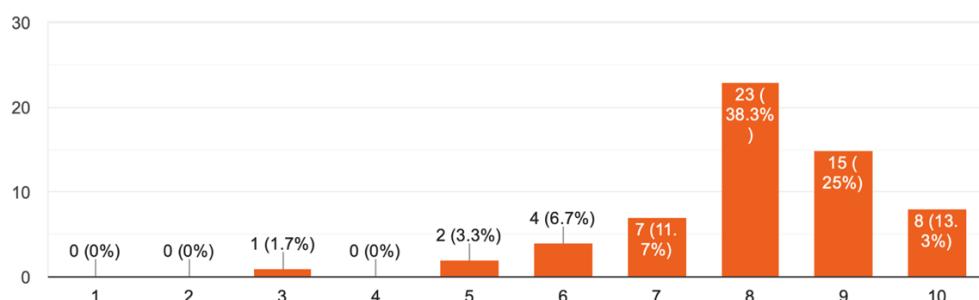
**g. Room and time for networking and social relations**

60 responses



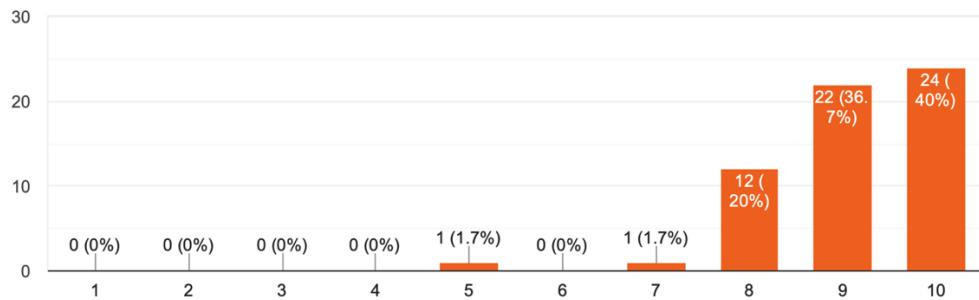
**h. Position and presentation of posters**

60 responses



i. Overall rating for preparation and organization

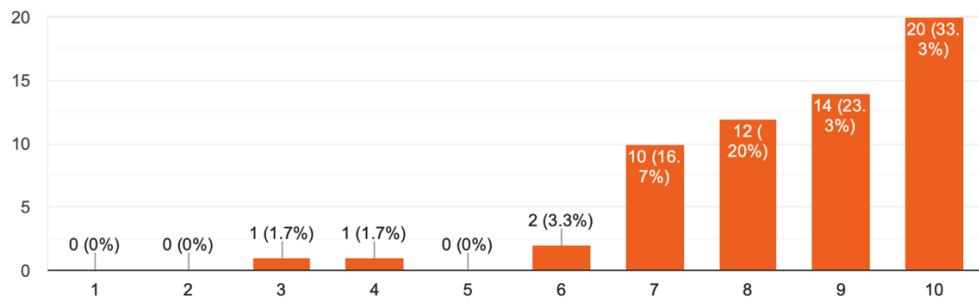
60 responses



## 7. Quality of Sessions and Presentations

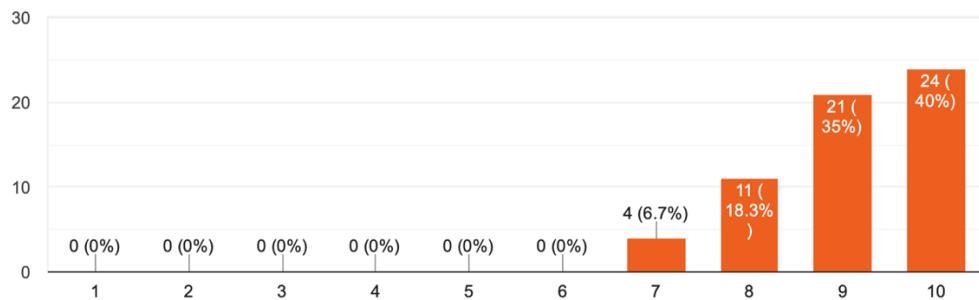
a. Opening Ceremony

60 responses



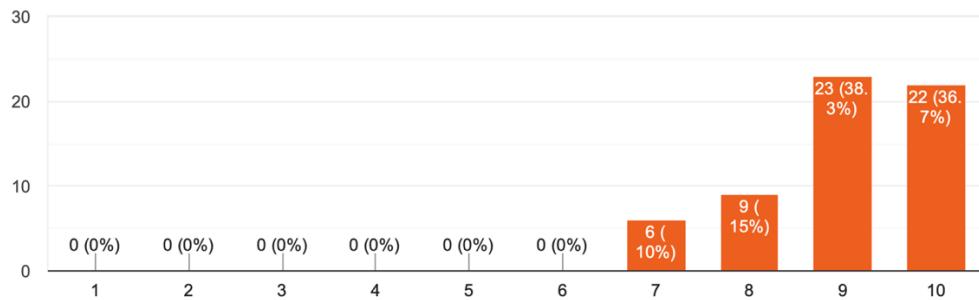
b. Plenary Session 1: Basic sciences and the many faces of CMT

60 responses



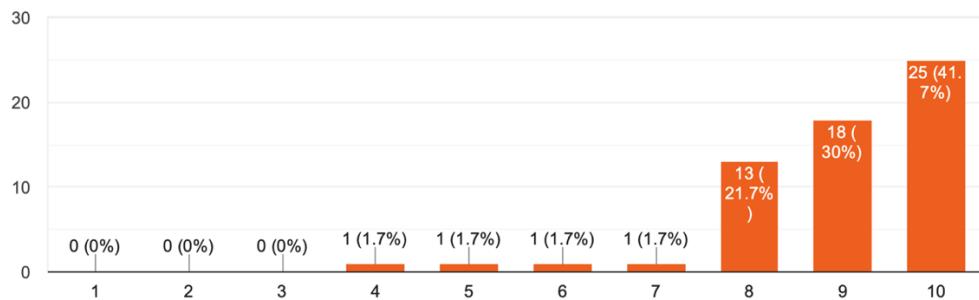
c. Plenary Session 2: Diagnostics/genetics of CMT neuropathies

60 responses



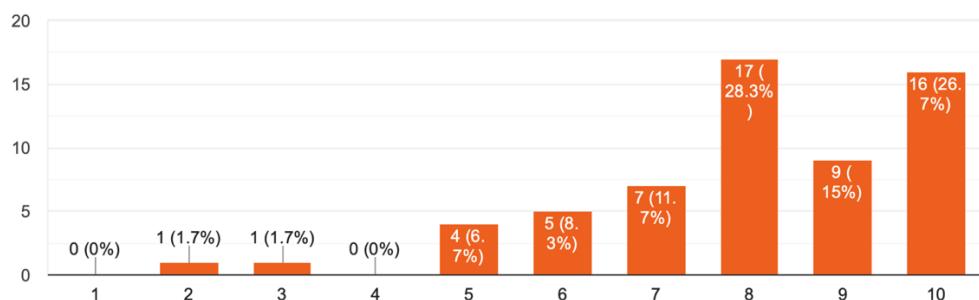
d. Plenary Session 3: Therapeutic approaches for CMT neuropathies

60 responses



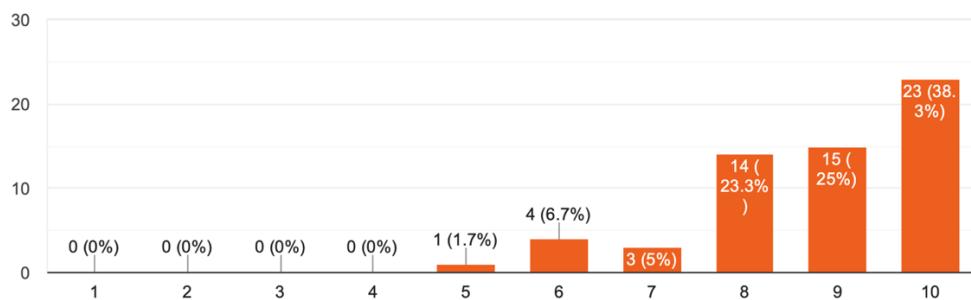
e. Fishbowl "Idea Workshops" – joint projects and initiatives

60 responses



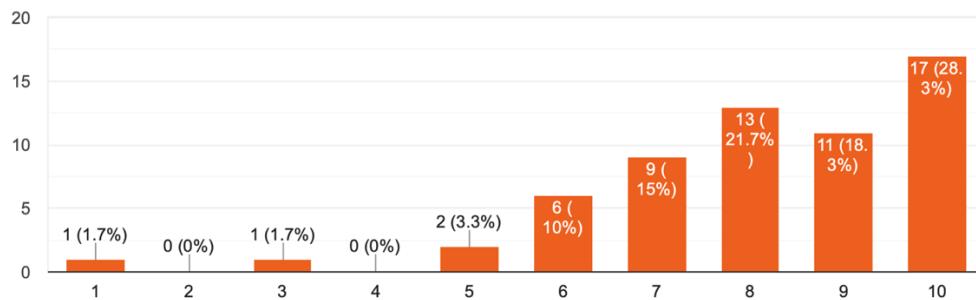
**f. Plenary Session 4: Clinical trials, data sharing, and outcome measurement**

60 responses



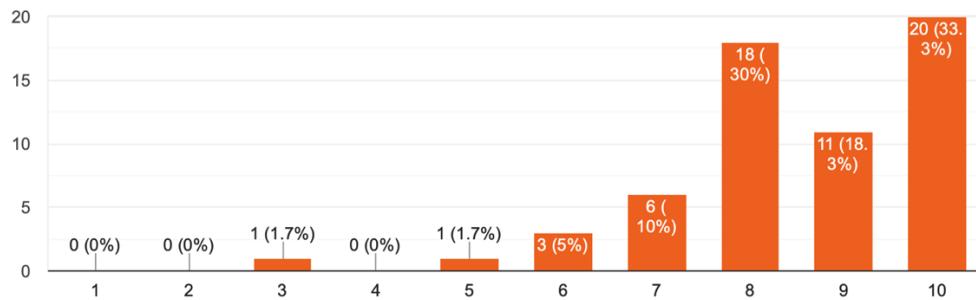
**g. Plenary Session 5: Round Table "Access to therapy for patients with a rare disease"**

60 responses



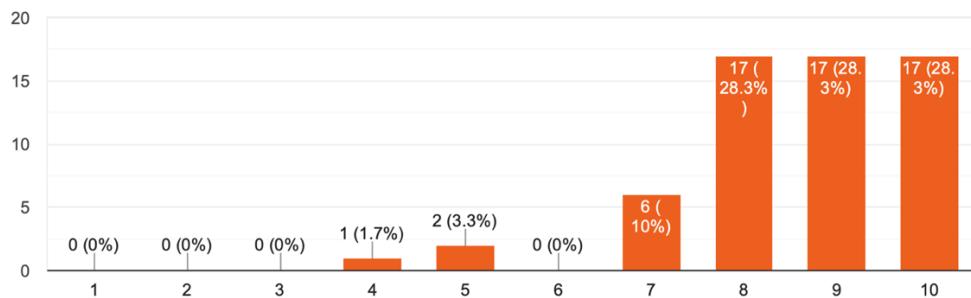
**h. Open Session: Physiotherapy/digital care/patients as partners**

60 responses



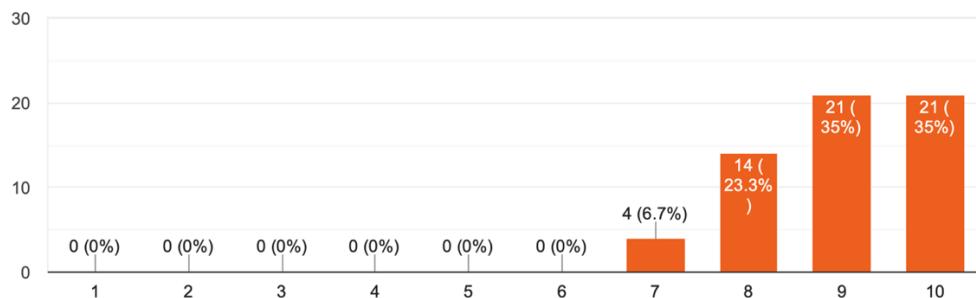
i. Poster Sessions 1 & 2

60 responses



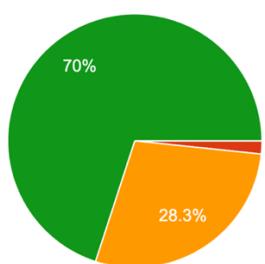
j. Overall rating for sessions and presentation

60 responses



8. Cost-Benefit of the Conference

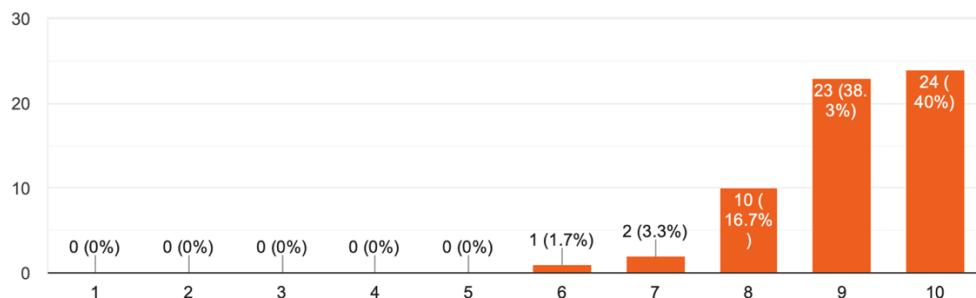
60 responses



- It was a loss of time and money; I would not attend again
- Only some sessions were of interest to me
- Relevant aspects of research and development in CMT were covered
- Excellent speeches, presentations, and posters; the conference exceeded my expectations

## 9. Overall Evaluation of the event

60 responses



## 10. Open Comments

### a. What did you like most about the conference, and what should be emphasized in the future?

- Networking
- variety of topics, many different approaches, networking
- I liked that the topics covered were cross-disciplinary and addressed several aspects of the disease, with significant attention to the patient.
- networking, good balance of presentations
- top experts , in the future maybe more about new disease genes discoveries and collaboration
- Presence of the different stakeholders
- Relatively small and great heart and focus
- I really loved the opportunity and interaction between patient organisations and scientists.
- Excellent 15 min presentations from selected abstracts. Really good to have these 10 Minute talks with 5 min discussions. Quality was excellent.
- challenges in gene therapy, more attention on children/ adolescents
- Fostering collaborations between researchers, clinicians and patients
- Mutual contacts with researchers and fellow patient representatives
- The personal relationships
- meeting colleagues and having new opportunities to collaborate with them; listen to lectures on topics that I never have time to do the bibliography
- Interaction between patients clinicians and researchers
- friendly atmosphere, good networking, good catering, lovely venue
- Meeting Friends and colleagues
- the patients as partners approach, the inclusion of industrial partners and the open and friendly cooperative atmosphere. High scientific level of presentations.

- The involvement of different players in the field of CMT: patients, scientists ...
- I liked the high quality of the presentations and the opportunity for discussion and networking.
- The collaboration between patients, scientists, clinicians, and partners! It is wonderful to have a space where everyone can learn from each other, specifically for CMT.
- The number and range of delegates, the patient's voice, the venue.
- I liked seeing patient organisations and scientists coming together
- I especially liked the collaborative and sharing atmosphere among the participants
- I particularly appreciated the high scientific quality of the presentations and the constructive exchanges between specialists. This collaborative approach should definitely be emphasized in future conferences.
- That it was really patient centred and patients attended
- The key differentiator for this conference from other scientific meetings was, as intended, the unique format of bringing together all stakeholders (not just scientific and clinical) in the field of CMT (including physical therapists, patients, advocacy groups, companies, and political leaders), and the emphasis on fostering small-group discussions and strategic coordination. The comparatively smaller size of the conference was really an asset, as were the abundant opportunities to mingle and network, i.e. longer breaks in between stimulating talks). Together, these features delivered major value for the all participants and I hope it will be continued.
- great talks and enough time for the poster session and networking
- Constructive discussions and exchange of opinions at every speech
- Liked every part, nicely balanced
- the different themes
- The most valuable aspect was the clarity and depth of the presentations. The invited speakers were excellent, and the sessions were well-structured. Going forward, I'd suggest emphasizing emerging topics and providing more time for Q&A after each talk.
- This conference was really barrier-free, everyone was open-minded, and there were great opportunities for discussion and exchange
- Variety of talks
- The collaborative approach (multi-stakeholder, patients as partners)
- Scientific content
- Networking for clinicians
- Small group size, opportunities for networking, great atmosphere
- physiotherapy
- I really appreciated attending a meeting that was fully focused on CMT. it created a unique sense of shared purpose. I also really enjoyed the interactions with the different stakeholders (researchers, clinicians, patient organizations).
- There was a lot of time for networking and I managed to make lots of new and meaningful connections.
- Excellent preparation of speakers and poster presenters. Go on in this directions!

- Gala Dinner
- Liked: patients as partners. What should be more emphasized next time: drugs in development and the KOLs' view on the most promising molecules in the pipeline; most recent guidelines on clinical trial design and endpoints.
- Discussions, Networking, Idea Sessions, Plenary Sessions - All outstanding
- The networking, collaborative climate, new collaborations and ideas
- Future therapies
- The conference had a good variety, was very well organized, and struck a nice balance between researchers, patients, and companies — also in the talks, whether fundamental or clinical.
- bridging all aspects of CMT
- The quality of the speakers and presentations.
- Facilitated interactions with Cmt leaders and students
- Th open Session about Physiotherapy and care
- High amount of inputs. The time of the presentations could be limited with 10 minutes so that there is more time for discussion
- the talks but i think organizing round tables on a specific topic could be an asset
- Connection between scientist, clinicians and patients
- I really appreciated that it was a relatively small conference, which made interactions and discussions very easy and engaging, even though it was my first time attending
- "familial" size, easy to speak with everybody, researchers + clinicians + patients = efficacy
- The scientific quality and the ability to have time for networking.
- It was remarkable how well balanced it was between all the fields of study and the time dedicated to them.

**b. What did you like least about the conference, or what could be improved in future European CMT Specialists-Conferences?**

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- the round table concept
- There wasn't much room for discussion after each talk. It might help to move the discussion to the end of the entire (n-talk) plenary session, with all the speakers on stage.
- round table was too long
- everything was fantastic
- dissemination of information was not so easy
- Some more allied health professional lectures
- For me, the round table went a bit off topic. Although a great idea, the focus was not as much on CMT as I would've preferred
- 1st day was excellent. The second day did not work quiet as well in the afternoon . I think parallel patient and scientist sessions work better with shorter but more focused joint sessions.
- pediatric part was missing

- the poster positions, maybe the rooms were too small
- Many programs offered opportunities for medical technical developments within CMT. Natural history studies, such as those related to living with CMT, are also essential. That should be more.
- the stairs
- continue the same as this time !
- I do not know
- more money for travel rather than accomodation
- More room
- lack of time for networking and discussion beside of the plenaries. Half a day more could have helped to
- the fishbowl concept was less interesting. I would have a more open table moment to discuss what is needed in science.
- In the future, I would emphasize more interactive sessions and practical case discussions.
- The days were quite long. Maybe some shorter breaks in between to have a bit more time in the evening.
- The journey home!
- Seeing the same few scientists "collaborating"
- I would reduce the redundancy in some topics
- Everything was very well organized; I have no particular suggestions for improvement.
- I would have liked guided poster sessions
- I think it would be great to further expand the involvement of political stakeholders, such as representation of the EMA, FDA, or national political leaders. This may be difficult, but very high value.
- Political talks are important regarding EU funding and policy, the video message of the commissioner was very formal.
- There was little discussion about the rehabilitative aspect.
- Venue and hotels were not very close
- Some studies are very limited in terms of patient numbers and involve very exceptional cases. This is less attractive for patients.
- Nothing major to criticize, but improving the balance between presentation time and question time would enhance participant engagement.
- The weather (not the organizing committee's fault)
- Poster layout and length of poster sessions made talking to presenters a bit difficult
- slightly more comfortable chairs :)
- I think overall everything was fine. Maybe more financial support could be provided for young researchers and also international collaboration could be strengthened.
- More clinicians
- round table should have been more interactive rather than a monologue

- poster sessions could be more interactive
- I was actually very satisfied overall. The talk on stem cell therapy was not really my cup of tea, and although the fishbowl concept is a good idea, it felt like it didn't have the intended impact. perhaps it could be organized differently next time.
- The weather! (This is out of anyone's control), honestly nothing, I thought it was a great and very well organised conference.
- No round tables, please
- More spacious venue
- Time for Lunch very long
- On demand availability of recorded presentations.
- Nothing, it was Perfect
- Nothing
- Nothing was bad
- Poster sessions were maybe a tad too long
- Not extremely accessible for CMT patients. Especially during coffee breaks or lunch time.
- Food
- The presence of children. This is not a place for them.
- time to submit abstracts was to short
- poster session, food (:D)
- Everything was great. I guess one important issue would be how to raise funds for CMT research in Europe
- My poster was located in a slightly hidden area, so it did not receive much attention and interaction.
- For the questions and comments, it would be nice for the moderators to let the juniors speak before the seniors who maybe monopolize too much the floor first.
- Everything was perfect, nothing to improve!
- It would be desirable to see greater participation from smaller groups dedicated to the study of CMTs.

**c. If there will be a 3rd European CMT Specialists Conference, what topic would you like to see addressed?**

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- new basic sciences approaches
- As a first-time attendee, I found the conference excellent. It might be worth considering whether a session dedicated to animal models would be of interest.
- this conference had a very good balance so i would like the same format
- new disease genes discoveries and collaboration for new causes of CMT
- therapy - clinical trials
- More allied health

- For my personal interest, I would love to dive deeper into the basic sciences and patients viewpoints. Also, for some presentations more time for questions could create very interesting discussions.
- 2 days. First day parallel patient organisation and scientific sessions. 2nd day am scientific session for both groups but maybe more on the translation end so whole audience would be interested. Pm joint focussed session with both groups
- pediatric patients
- same topics for an update + clinical trials developpement and acceleration
- A great deal of research focused on "cure," while "care" is also very important. It would be great if future conferences could facilitate exchanges with more specialists in this area (surgery, orthoses, living with CMT, etc.).
- the cure or the treatment
- genetics on late onset CMT
- similar to these selected here
- more genetics
- Classification
- data management within the European Health Data Space - better access of researchers to patients data around the world.
- I would like to see a focus on advances in genetic diagnosis and on collaborative approaches for unsolved CMT cases
- How collaboration will be managed going forward.
- I would like to see presentations from people different than those who present at the PNS meeting
- yes
- Same as in 2nd
- The topics were well selected.
- The 1st and 2nd conferences were great successes and will pave the way for the next conferences. In the future, results (positive and negative) of on-going clinical trials will be very relevant to communicate. Also new trials could be initiated and discussed at the next conference. Epidemiology (disease frequency) of CMT could also be a relevant topic as we still rely on very old studies. The meeting program should maintain a multi-disciplinary character. Results of collaborative EU-level projects could be presented, as well as novel cross-border and multi-disciplinary initiatives and expertise. Examples of 'patients as partners' can be presented at the next conference.
- Conservative and rehabilitative therapeutic approaches
- how to strengthen collaboration - ideas on specific projects among CMT researchers in the EU including funding opportunities
- Drug development
- Please pay more attention to non-scientific treatments such as physiotherapy
- It would be interesting to include more international perspectives and collaborative initiatives to harmonize care and research efforts across Europe.
- Update on DMTs, ways of participation, networks, funding, publications

- Pre-clinical models
- Perspectives for CMT patients - ongoing clinical trials, physiotherapy guidelines etc.
- Clinical trials
- Treatment and diagnosis
- steps in therapy development
- surgical management CMT; pre-implantation diagnostics
- I particularly enjoyed the basic science sessions, but all parts were valuable. I'd be happy to see a similar balance in the future.
- N/A
- I think that all relevant topics have been covered
- Patient Advocacy groups' voices
- More clinical management and available registries for collaborative work
- Drugs in development and the KOLs' view on the most promising molecules in the pipeline; most recent guidelines on clinical trial design and endpoints.
- Joint project developments and Advanced in Therapy trials
- Similar as the 2nd, with updates
- Genome editing approaches
- NA
- More real-world data covered.
- Epigenetics and phenotypic variability
- The place of occupational therapist, physiotherapist... in the care of CMT patient.
- more about supportive therapies
- how to translate therapy towards clinical trials
- Probably even more basic science (disease mechanisms, animal models)
- I would appreciate seeing more updates and results related to clinical trial readiness in future conferences.
- While gene therapy is very interesting and promising, it could be also nice to invite speakers working on other therapeutical approaches (small molecules, Crispr, etc ...)
- CMT preclinical trials
- It might be useful to address the field of prenatal and preimplantation testing given their rapid spread.

## **Summary and discussion**

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### *Part one: Composition, invitations, topicality*

77% of the participants having responded to the survey were scientists, clinicians and other health professionals (Q.1). Representatives of patient organizations (16,4%) and pharmaceutical industry (less than 5%) were smaller groups, while one person

only identified herself as policy-maker. The conference, thus, was basically professional with a considerable participation of patient representatives. Industry and policy-makers have perhaps not been invited with sufficient emphasis. Another explanation could be that a particular rare disease or rare diseases are not what industry or policy-makers expect as beneficial enough to invest time and attention for. The replies to Q.2 on how participants learned about the conference may add another explanation. The great majority indicated to have got the information from colleagues or direct invitation by mail or formal letter.

The invitation management and scheduling (Q.3) was positively appreciated, though almost a third of the participants held that invitations started too early. There were no complaint or mention of issues. Almost all respondents rated positively the topicality and relevance of the conference (Q.4), only 6.6% said that some important aspects were missing.

### *Part two: Innovative approach, concept and organization*

Was the conference like all the others, or was there anything new, progressive and worth to develop further for boosting research in CMT and other iNMD's (Q.5)? The response to the very direct question if the conference was similar to other gatherings in the field (Q.5.a) was clear: With an average of 4.79 rate the answer is positive, this means that around 22% of the responses only tend to say yes, while the majority appreciates an innovative character. More precisely, the collaborative approach was appreciated with an average rating of 9.03 (Q.5.b), and with an average rating of 8.85 the conference design was recommended as a model for promoting research on other rare diseases (Q.5.c). Similarly positive are the average ratings regarding the concrete preparation and organization of the conference (Q.6):

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|   |             |
|---|-------------|
| a. Information and invitation management – the conference website | 8.89        |
| b. Accommodation information                                      | 8.68        |
| c. Registration and financial management                          | 9.11        |
| d. Call for abstracts and conference programming                  | 8.97        |
| e. Documentation and preparatory materials provided               | 8.93        |
| f. Quality and timing of catering, food, and drinks               | 8.85        |
| g. Room and time for networking and social relations              | 8.80        |
| h. Position and presentation of posters                           | 8.05        |
| <b>i. Overall rating for preparation and organization</b>         | <b>9.08</b> |

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### *Part three: Quality of the sessions and presentations*

The ratings regarding the quality of the various sessions and presentations are probably the most important part of the evaluation of the conference. As can be seen from Q.7 they are similarly high. Here are the average ratings for:

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|   |             |
|---|-------------|
| a. Opening Ceremony   | 8.52        |
| b. Plenary 1: Basic sciences and the many faces of CMT            | 9.08        |
| c. Plenary 2: Diagnostics/genetics of CMT neuropathies            | 9.02        |
| d. Plenary 3: Therapeutic approaches for CMT neuropathies         | 8.97        |
| e. Fishbowl "Idea Workshops" – joint projects and initiatives     | 8.03        |
| f. Plenary 4: Clinical trials, data sharing, and outcome measures | 8.79        |
| g. Plenary 5: Round Table "Access to therapy..."                  | 8.11        |
| h. Open Session: Physiotherapy/digital care/patients as partners  | 8.52        |
| i. Poster Sessions 1 & 2  | 8.57        |
| <b>j. Overall rating for sessions and presentations</b>           | <b>8.97</b> |

#### *Part four: cost-benefit and an overall evaluation*

Not one participant responded that attending the conference was a lost of time and money. Instead, almost 100 % answered that there were "Excellent speeches, presentations, and posters; the conference exceeded my expectations" (68.9%) or at least "Relevant aspects of research and development in CMT were covered" (29.5%). Only 1.6% said that "Only some sessions were of interest to me". The average rating for the "overall evaluation of the event" came to a similarly exceptional result: 9.11. If we call an average rating of 8 "good", an average of 9 "very good" and an average of 10 "outstanding" it might be of interest to know that 39.3% rated 10 (outstanding), 39.3% rated 9 (very good) and 16.4% rated 8 (good=). 3.3% (two participants) rated 7 and 1.6% (one participant) rated 6.

#### ***The open comments***

The open comments basically reflect what is visible in the responses to the questions, in part they underline and further explain.

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#### **a. What did you like most about the conference, and what should be emphasized in the future?**

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This positive list is characterized by keywords such as (comments grouped as far as possible), some comments from b. (critique) are quoted here as they are rather positive, such as: "everything was fantastic", or "everything was perfect, nothing to improve!":

#### **Quality – scientific level**

Discussions, Networking, Idea Sessions, Plenary Sessions - All outstanding; top experts, quality was excellent; high scientific level of presentations; high quality of the presentations and the opportunity for discussion and networking; high scientific quality of the presentations and the constructive exchanges between specialists; the most valuable aspect was the clarity and depth of the presentations. The invited speakers were excellent, and the sessions were well-structured; excellent preparation of speakers and poster presenters. Go on in this directions! The scientific quality and the ability to have time for networking.

#### **Topics**

listen to lectures on topics that I never have time to do the bibliography: really appreciated attending a meeting that was fully focused on CMT. it created a unique sense of shared purpose; Excellent 15 min presentations from selected abstracts cross-disciplinary topics, good balance of presentations, excellent 15 min presentations from selected abstracts; great talks and enough time for the poster session and networking; conference had a good variety, was very well organized, and struck a nice balance between researchers, patients, and companies – also in the talks, whether fundamental or clinical; bridging all aspects of CMT; It was remarkable how well balanced it was between all the fields of study and the time dedicated to them.

### **Multistakeholder-collaborative approach – patients as partners**

The collaborative approach (multi-stakeholder, patients as partners); the patients as partners approach, the inclusion of industrial partners and the open and friendly cooperative atmosphere attention to the patient; interaction between patients clinicians and researchers presence of different stakeholders; loved the opportunity and interaction between patient organizations and scientists; involvement of different players in the field of CMT: patients, scientists ...; the collaboration between patients, scientists, clinicians, and partners! It is wonderful to have a space where everyone can learn from each other, specifically for CMT; patient organisations and scientists coming together; it was really patient centred and patients attended; really enjoyed the interactions with the different stakeholders (researchers, clinicians, patient organizations); the networking, collaborative climate, new collaborations and ideas; there was a lot of time for networking and I managed to make lots of new and meaningful connections; networking; fostering collaborations between researchers, clinicians and patients; mutual contacts with researchers and fellow patient representatives; the personal relationships, meeting colleagues and having new opportunities to collaborate with them; especially liked the collaborative and sharing atmosphere among the participants; this collaborative approach should definitely be emphasized in future conferences; connection between scientist, clinicians and patients

### **Networking opportunities – good atmosphere, size**

relatively small and great heart and focus; this conference was really barrier-free, everyone was open-minded, and there were great opportunities for discussion and exchange; small group size, opportunities for networking, great atmosphere; "familial" size, easy to speak with everybody, researchers + clinicians + patients = efficacy

### **Next generation researchers**

Facilitated interactions with Cmt leaders and students; appreciated that it was a relatively small conference, which made interactions and discussions very easy and engaging

### **b. What did you like least about the conference, or what could be improved in future European CMT Specialists-Conferences?**

Not all the critical comments are taken up here. Some just criticize what others had specifically appreciated, if not with special reasoning they are not taken up here. Thus, the list of critical comments is more limited (comments grouped as far as possible):

### **The round table concept**

round table was too long; the round table went a bit off topic. Although a great idea, the focus was not as much on CMT as I would've preferred; round table should have been more interactive rather than a monologue; no round tables, please

### **Topics**

Considering that the development of medications for CMT is only just beginning, rehabilitation remains the main therapeutic option, and it deserves greater emphasis in this conference – across all age groups (children, adolescents, adults and older people). In my opinion, this topic could have been explored more thoroughly in your event, especially given the growing body of high-quality research in this area; natural history studies, such as those related to living with CMT, are also essential. That should be more; I would reduce the redundancy in some topics.

### **Format**

The second day did not work quiet as well In the afternoon; I think parallel patient and scientist sessions work better with shorter but more focused joint sessions; the fishbowl concept was less interesting. I would have a more open table moment to discuss what is needed in science; I would emphasize more interactive sessions and practical case discussions; I would have liked guided poster sessions; Poster layout and length of poster sessions made talking to presenters a bit difficult; my poster was located in a slightly hidden area, so it did not receive much attention and interaction; although the fishbowl concept is a good idea, it felt like it didn't have the intended impact. perhaps it could be organized differently next time.

### **Organization**

the poster positions, maybe the rooms were too small; more room; more money for travel rather than accommodation; Venue and hotels were not very close; more financial support could be provided for young researchers and also international collaboration could be strengthened; time to submit abstracts was to short; one important issue would be how to raise funds for CMT research in Europe; for the questions and comments, it would be nice for the moderators to let the juniors speak before the seniors who maybe monopolize too much the floor first

### **Stakeholder participation**

It would be great to further expand the involvement of political stakeholders, such as representation of the EMA, FDA, or national political leaders. This may be difficult, but very high value; political talks are important regarding EU funding and policy, the video message of the commissioner was very formal

**c. If there will be a 3rd European CMT Specialists Conference, what topic would you like to see addressed?**

Some interesting ideas have been added at this rubrique, beyond what was already suggested supra under a. and b., such as:

**Topics**

new basic sciences approaches; Pre-clinical models; focus on advances in genetic diagnosis and on collaborative approaches for unsolved CMT cases; genome editing approaches; more about new disease genes discoveries and collaboration; what should be more emphasized next time: drugs in development and the KOLs' view on the most promising molecules in the pipeline; most recent guidelines on clinical trial design and endpoints; genetics on late onset CMT; gene therapy is very interesting and promising, it could be also nice to invite speakers working on other therapeutical approaches (small molecules, Crispr, etc ...); it might be worth considering whether a session dedicated to animal models would be of interest; new disease genes discoveries and collaboration for new causes of CMT; therapy - clinical trial; how to translate therapy towards clinical trials; dive deeper into the basic sciences and patients viewpoints; pediatric patients; a great deal of research focused on "cure," while "care" is also very important; surgical management CMT; pre-implantation diagnostics It would be great if future conferences could facilitate exchanges with more specialists in this area (surgery, orthoses, living with CMT, etc.); pay more attention to non-scientific treatments such as physiotherapy; conservative and rehabilitative therapeutic approaches; the place of occupational therapist, physiotherapist..... in the care of CMT patient; the use of assistive devices to improve hand and foot functionality; data management within the European Health Data Space - better access of researchers to patients data around the world; more clinical management and available registries for collaborative work; conservative and rehabilitative therapeutic approaches; how to strengthen collaboration - ideas on specific projects among CMT researchers in the EU including funding opportunities; drug development; address the field of prenatal and preimplantation testing given their rapid spread.

**Presenters**

I would like to see presentations from people different than those who present at the PNS meeting

**Format**

Organizing round tables on a specific topic could be an asset

Going forward, I'd suggest emphasizing emerging topics and providing more time for Q&A after each talk; for some presentations more time for questions could create very interesting discussions; include more international perspectives and collaborative initiatives to harmonize care and research efforts across Europe; 2 days: First day parallel patient organization and scientific sessions. 2nd day am scientific session for both groups but maybe more on the

translation end so whole audience would be interested. Pm joint focused session with both groups.

## Conclusions

The feed-back received in this survey is very positive. The size was appropriate for the objectives to be achieved. The format chosen too served the purpose of creating an atmosphere of trustful cooperation and openness for a new, a multi-stakeholder, approach with patients and industry as partners in a cooperative research process. This new approach was greatly appreciated and put into practice already on the spot. One of the comments under supra a. seems best to describe the innovation which – as a model for other rare diseases – may contribute to boosting research in CMT:

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*"The key differentiator for this conference from other scientific meetings was, as intended, the unique format of bringing together all stakeholders (not just scientific and clinical) in the field of CMT (including physical therapists, patients, advocacy groups, companies, and political leaders), and the emphasis on fostering small-group discussions and strategic coordination. The comparatively smaller size of the conference was really an asset, as were the abundant opportunities to mingle and network, i.e. longer breaks in between stimulating talks). Together, these features delivered major value for the all participants and I hope it will be continued".*

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Another comment, found in supra c., gives an idea for the way to go in future

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*The 1st and 2nd conferences were great successes and will pave the way for the next conferences. In the future, results (positive and negative) of on-going clinical trials will be very relevant to communicate. Also new trials could be initiated and discussed at the next conference. Epidemiology (disease frequency) of CMT could also be a relevant topic as we still rely on very old studies. The meeting program should maintain a multi-disciplinary character. Results of collaborative EU-level projects could be presented, as well as novel cross-border and multi-disciplinary initiatives and expertise. Examples of 'patients as partners' can be presented at the next conference.*

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The evaluation including all the comments in detail encourage to further proceed in the direction chosen for the 2<sup>nd</sup> European CMT Specialists Conference, 2025 in Antwerp also for future conferences, including workshops, joint projects and other collaborative action as discussed and decided in Antwerp.

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Berlin, November 2025

Filippo Genovese

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