CAPACITY BUILDING WORKSHOP FOR ASSOCIATIONS OF PATIENTS WITH HAEMOGLOBIN DISODERS THALASSAEMIA AND SICKLE CELL DISEASE

2-3 July, 2022

Venue Hotel: **Courtyard by Marriott Stockholm Kungsholmen**

THALASSAEMIA INTERNATIONAL FEDERATION

PATIENTFÖRENINGEN KRONISK BLODSJUKDOM • KBS •

ORGANISER:
Thalassaemia International Federation

in collaboration with
Kronisk Blodsjukdom - KBS Sweden

THALIA

Co-funded by the Health Programme of the European Union

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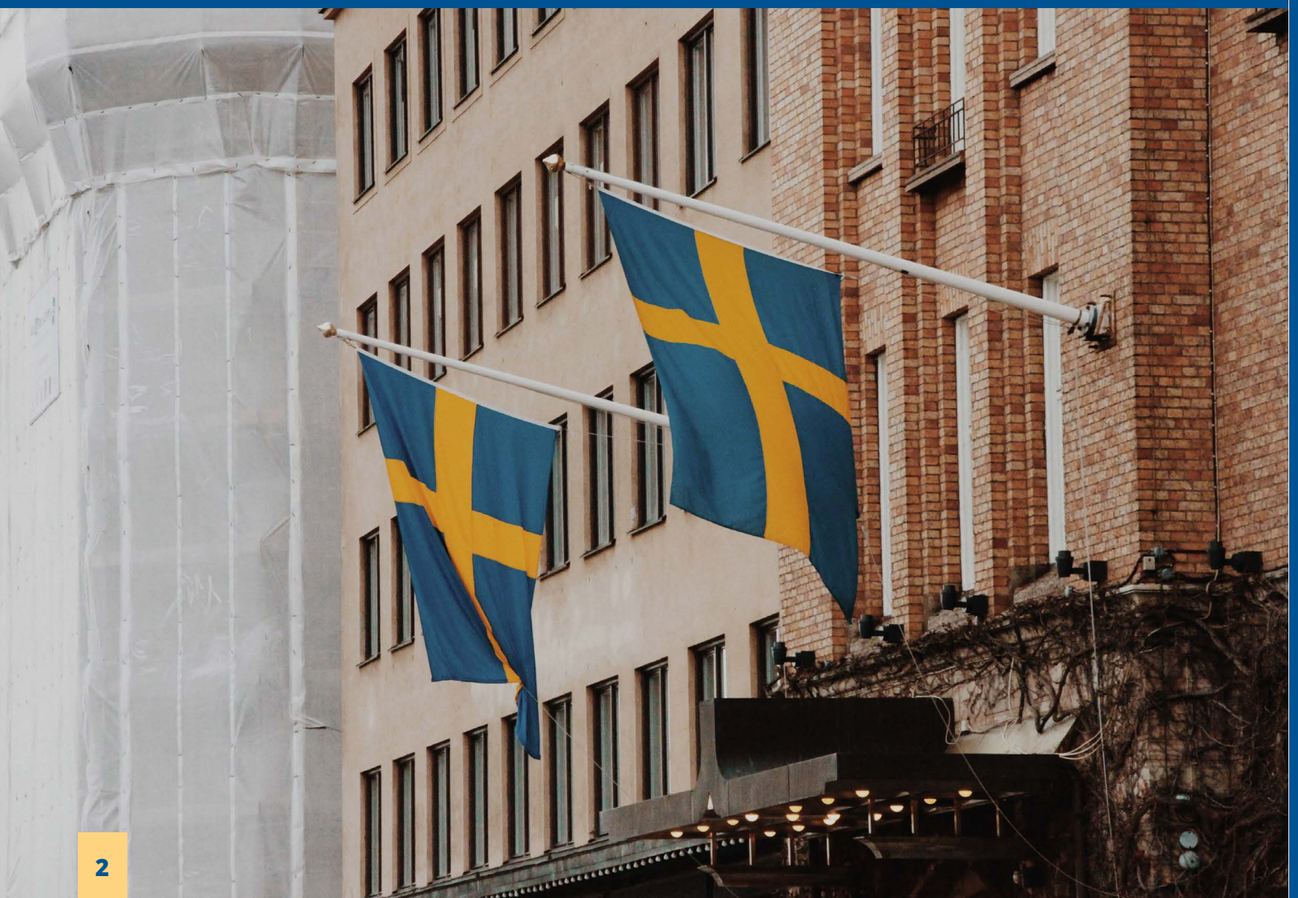
THE REPORT



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ORGANISATION AND COORDINATION

THALASSAEMIA INTERNATIONAL FEDERATION

Dr Androulla Eleftheriou
Executive Director

Mrs. Lily Cannon
Operations Manager

Mrs. Eleni Antoniou
Senior Policy Officer

Mr Rawad Merhi
Countries Officer

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Hotel Express Cyprus Ltd

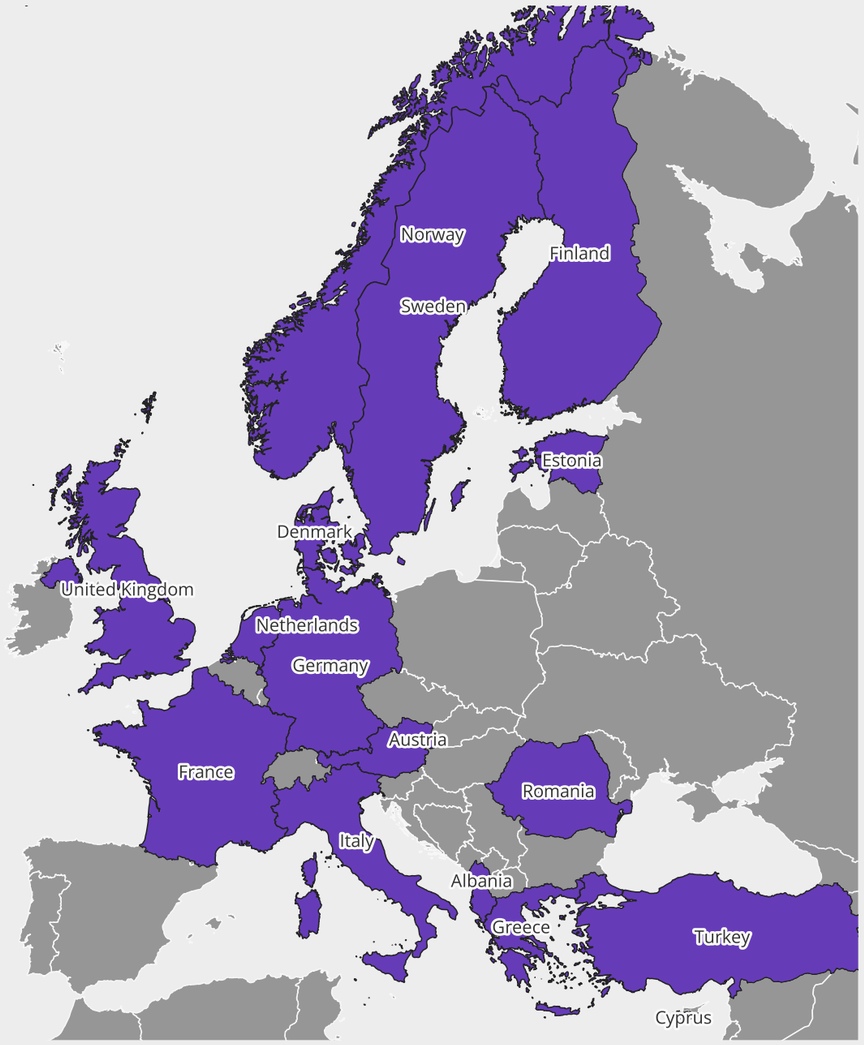
i FILM

Conference videography and photography:
iFilm by Christos Fourlis

SPEAKERS AND CHAIRPERSONS

Dr. Cecilia Karlström MD, PhD	Specialist in hematology, working group on Benign Hematology, member of the board of the Swedish Hematology Association, Sweden
Mr. Abdalsalam Alaraj	Member of Kronisk blodsjukdom KBS, T-PAG Member, Sweden
Prof. Baba Inusa	Professor of Paediatric Haematology, King's College London, UK
Prof. Antonio Piga	Professor of Paediatrics & Dean of the Medicine School, San Luigi University Hospital, Turin University, Turin, Italy
Dr. Perla Eleftheriou	Consultant Haematologist, Joint Red Cell Unit, Haematology Department, Clinical Lead for Red Cell Haematology, Honorary Clinical Senior Lecturer, University College London, London, UK
Prof. John Porter	Professor of Haematology, Head of the Joint Red Cell Unit for UCLH & Whittington Hospitals, Haematology Department, University College London, London, UK
Dr. Annika Sonesson	Clinical Chemistry, Laboratory medicine, Lund University, Sweden
Dr. Mary Petrou	Honorary Senior Lecturer Institute of Women's Health University College London, UK
Prof. Andreas E. Kulozik	Professor of Pediatrics, Chairman, Department of Pediatric Oncology, Hematology and Immunology, University of Heidelberg, Germany
Dr. Ulf Tedgård MD, PhD	Department of Paediatric Hematology Oncology, Skåne University Hospital, Sweden
Dr. Andreas Glenthøj, MD, PhD	Head of the Danish Center for Hemoglobinopathies, Department of Hematology, Copenhagen University Hospital - Rigshospitalet, Denmark
Dr. Karin Magnussen	Donors and Donation Working Party Chair, International Society of Blood Transfusion - Medical Director, Dept. Blood Centre and Laboratory Medicine, Innlandet Hospital, Norway
Dr. Marika Grönroos	Paediatric Hematologist and Oncologist, MD, PhD. Department of Paediatrics and Adolescent Medicine at Turku University Hospital, Turku, Finland
Dr. Dimitrios Farmakis	Associate Professor at University of Cyprus Medical School, Consultant Cardiologist at Nicosia, General Hospital, Nicosia, Cyprus
Dr. Anne Yardoumian	TIF Educational Advisor, Consultant Haematologist, North Middlesex University Hospital NHS Trust, Sterling Way, Edmonton, UK
Dr. Michael Angastiniotis	Medical Advisor, Thalassaemia International Federation (TIF), Cyprus
Mrs. Lily Cannon	Operations Manager, Thalassaemia International Federation (TIF), Cyprus
Mr. Radu Ganescu	President, Asociatia Persoanelor Cu Thalasemie Majora, Romania
Mr. George Constantinou	TIF Board Member - Ass. Secretary, UK
Mr. Angelo Loris Brunetta	TIF Board Member, President of Associazione LigureThalassemici, Italy
Ms. Joud Alaraj	Member of Kronisk blodsjukdom KBS, Sweden
Mrs. Maria Montefusco	Chairperson of Rare Diseases Sweden and a Member of the EURORDIS Board of Directors, and a Member of the EURORDIS Board of Officers.
Ms. Eleni Antoniou	Senior Policy Officer, Thalassaemia International Federation (TIF), Cyprus
Mr Miltos Miltiadous	President of the Board, Cyprus Thalassaemia Association, Cyprus
Ms. Simona Annese & Mr. Guiseppe Selvarolo	Patients from Italy
Mr. Ali Ibrahim	Patient from UK
Mr. Vassilis Dimos	Chairman of the Board of the Greek Thalassaemia Association
Mrs. Katia Pelides	Research, Department of Languages and Literature, Thalassaemia International Federation (TIF), Cyprus

PARTICIPATION



104 participants from 16 countries

WORKSHOP AIMS AND DETAILS

The workshop was organized by Thalassaemia International Federation (TIF), in collaboration with the Swedish Kronisk Blodsjukdom (Chronic Blood Disease) association (KBS), with the aim to promote exchange of knowledge and experiences on optimal practices and recent advances on the management of haemoglobinopathies and to share concerns on challenges and unmet needs among participating healthcare professionals and patients. A special focus of the workshop was patient education and capacity building for advocacy.

More specifically, the objectives of the workshop included:

1. building capacities for patients their families and carers through strengthening their disease specific knowledge;
2. informing the medical and patient communities on the current state-of-art clinician management of thalassaemia and SCD, the latest scientific advances in the field and the regulatory development for novel therapies;
3. extending the knowledge gained in countries long experience in thalassaemia and SCD management to other European countries through sharing experiences and best practices;
4. developing skills and capacities for advocacy and productive participation in decision making at local, country and region levels;
5. creating core groups of patients in each country to support and strengthen their role in the management of their own disease;
6. developing a robust infrastructure for National Patients' Associations, rendering them truly supportive to patients, families and carers at country level.

DAY 1
JULY 2, 2022

OPENING CEREMONY

The opening ceremony, chaired by Dr. Androulla Eleftheriou, started with TIF's President, **Mr. Panos Englezos,** addressing the audience on behalf of the Board of Directors and stressing the importance of educational and capacity building events such as those organized by TIF (pre-recorded video). **Mrs.. Bwomono** welcomed the delegates on behalf of the Swedish patient association KBS. **Dr. Karlström** also welcomed the delegates on behalf of the Swedish Haematology Association. Finally, **Mr. Alaraj,** an immigrant thalassaemia patient, presented his own journey from his home country, Syria to Sweden in a moving talk that received a hearty applause by the audience.

SESSION 1

Session 1, chaired by **Prof. Porter and Prof. Piga,** provided an overview of the current state-of-art management of patients with thalassaemia and SCD. The session included the following presentations:

- **Prof. Piga** addressed a series of key questions concerning blood transfusion therapy for thalassaemia.
- **Prof. Porter** described the various aspects of multidisciplinary care for patients with thalassaemia.
- **Prof. Inusa** talked about multidisciplinary care for patients with SCD.
- **Dr. Anika** described the main principles of thalassaemia diagnosis.

The discussion that followed addressed several issues concerning blood transfusion practices, blood donor selection, safety of iron chelators and challenges of genetic counselling. Dr. Androulla Eleftheriou stressed the importance of national prevention programmes and the challenges in thalassaemia prevention, due to migration flows and rarity of the disease and the challenges incurring in the establishment of national prevention programmes.

SESSION 2

Session 2, chaired by **Prof. Inusa and Dr. Karlström,** presented the main novel and innovative therapies for thalassaemia and SCD. The session consisted of the following presentations.

Prof. Kulozik explained the principles of gene therapy and gene editing, stressing the perspectives for patients, but also the potential safety issues that may arise in the course of time for which caution is required.

Prof. Piga described the mode of action of luspatercept and the results of hitherto phase II and phase III trials in patients with transfusion- and non-transfusion-dependent thalassaemia, in terms of efficacy and safety, while also stressing cost-related issues of this therapy.

Prof. Inusa talked about innovative therapies in SCD including voxelotor, the P-selectin inhibitor crizanlizumab, and the pyruvate kinase activators etavopivat and mitapivat, explaining their modes of action and the hitherto findings of clinical trials.

The discussion that followed addressed important issues brought up by patients, including the challenges of curative therapies such as safety issues, the ideal age of application, the cost-to-benefit ratio, the retraction of Bluebird Bio gene therapy product Zynteglo from Europe, the extent and value of hemoglobin response with innovative therapies in SCD, regulatory issues, advocacy actions for innovative therapies and real-world experience with luspatercept.

SESSION 3

Session 3, chaired by **Prof. Porter and Dr. Tedgård,** consisted of two parts; the first part provided an overview of thalassaemia and SCD care in Nordic countries, while the second focused on TIF's educational activities for healthcare professionals and the value of thalassaemia registries. The session included the following presentations:

PART 1
THALASSAEMIA AND SCD CARE IN NORDIC COUNTRIES

SWEDEN: Dr. Tedgård talked on the burden of thalassaemia and SCD in Sweden and the impact of migration, the current status regarding **(i)** education and awareness level; **(ii)** prevention and screening; **(iii)** clinical management; **(iv)** multidisciplinary care; **(v)** mental health. More specifically, the number of patients is increasing in Sweden due to migration. There is a lack of national screening programmes, but there is an established national thallassaemia and SCD registry, an established patient association (KBS) and available national guidelines, although not widely disseminated. There are domains with some progress, but with considerable room for improvement, including multidisciplinary care, genetic counselling and transition to adult care.

DENMARK: Dr. Glenthøj provided an overview of the Danish healthcare system, a typical Nordic system - public, equal and free – and described the organization of care provision to thalassaemia and SCD patients, the organization of the Danish centre for Haemoglobinopathies in Copenhagen University Hospital Rigshospitalet that follows most of the patients in the country, the ongoing research activities on thalassaemia and SCD, the increasing burden of haemoglobin disorders in Denmark (57 thalassaemia patients and 107 SCD ones), the established national screening program, the treatment and monitoring strategies for thalassaemia and SCD patients, including novel treatments and HSCT. Overall, the care offered to thalassaemia and SCD in Denmark seemed well organized (prerecorded presentation).

NORWAY: Dr. Magnussen provided an overview of the haemoglobinopathies in Norway. More specifically, all haemoglobinopathy patients are immigrants (although there are some Norwegian carriers); the estimated numbers are 250 patients with thalassaemia and 120 with SCD. Disease management guidelines have been developed and patients are treated mostly in regional hospitals, where treatment and monitoring strategies have been developed. There is no patient association thus far, but both a national registry and a reference centre are on the way.

FINLAND: Dr. Grönroos described the situation in Finland, where patients receive care mainly in 5 University Hospitals and a small number of additional hospitals across the country and patients often have to travel long distances to seek care. The diagnostic, treatment and monitoring strategies regarding thalassaemia and SCD in the country were briefly discussed. There is lack of a national screening programme, although genetic counseling is available upon request.

The discussion after each of the above presentations concerned mainly patients from the aforementioned countries expressing their experiences with the national healthcare services.

DAY 1
JULY 2, 2022

PART 2
TIF'S EDUCATIONAL RESOURCES FOR HEALTHCARE PROFESSIONALS AND THE VALUE OF THALASSAEMIA REGISTRIES

Three physicians coming from Austria **(Dr. Novak),** Sweden **(Dr. Bjorkman)** and Germany **(Dr. El Missiri),** described briefly their experience during their training on haemoglobinopathies at University College London Hospital, UK, in the context of the **Renzo Galanello Fellowship** programmeme (prerecorded talks).

Prof. Farmakis presented an overview of TIF's educational activities for healthcare professionals as one of the main pillars of TIF's work.

Prof. Farmakis described briefly the 2021 edition of the TIF guidelines for TDT as well the e-ThalED platform that provides to physicians specialized education and certification on thalassaemia prevention and management, based on TIF guidelines, and stressed the impact of TIF's education activities.

Dr. Yardumian presented TIF's SCD educational platform for healthcare specialists, stressing the need for special training on SCD and describing the process of the platform's development, the implicated experts and the content of the course.

Dr. Perla Eleftheriou presented an overview of the Renzo Galanello fellowship programme, created by TIF and held at the University College London Hospital under the lead of Prof. Porter and Dr. Perla Eleftheriou, a structured 2-week programme offering hands-on training on the management of thalassaemia and SCD (prerecorded presentation).

Dr. Angastiniotis presented an overview of the national thalassaemia registries and their value as unique tools to document the burden of disease and understand the actual healthcare needs to be addressed. Forms and content of registries, examples of national thalassaemia registries and the role of electronic healthcare records and informational technology, as well as the related challenges were covered (prerecorded presentation).

DAY 2
JULY 3, 2022

SESSION 4

The session was chaired by Dr. Androulla Eleftheriou, Mr.s. Cannon and Mr.. Ganescu.

Dr. Androulla Eleftheriou presented the importance of patient organizations (replacing Mr.. G. Constantinou who could not attend the meeting) and the value of patients' engagement that should concern every aspect of the disease. She presented the example of Cyprus where patients and parents organized themselves in mid-1970s in order to spread the awareness of the disease to all stakeholders, while convincing the government to establish a prevention program. She described briefly how TIF promotes patient engagement.

Mr. Brunetta discussed the importance of stablishing multi-level collaborations and networking for strengthening advocacy and achieving improvements and recognition of the disease-specific needs at the national level, while the rest of Session 4 and the whole of **Session 6** focused on the importance of establishing and strengthening patient oriented organisations and how, through their work, improvements in all levels and at all aspects of care including social care can be achieved. These were covered by country examples such as Cyprus by Mr. Miltos Miltiadous or personal testimonials by Mr. Miltos Miltiadous, Mr.s Simona Annese, Mr. Guiseppe Selvarolo and Mr. Ali Ibrahim.

SESSION 5

In Session 5 a series of educational presentations focused on advanced therapies, the landscape of EU policies and what those mean for haemoglobinopathy patients as well as the future of Rare Disease polices in Europe through EURORDIS perspective and invaluable work presented by Mrs. Maria Montefusco.

SESSION 7

Last Session 7 was focused on a comprehensive description of TIF's educational programme for patients/ families and the community at large and how patients' voice can be acknowledged and heard through strong advocacy which constitutes the main theme of this workshop: capacity building for strong and meaningful advocacy. The patients' educational programme of TIF was comprehensively presented by Mrs. Katia Pelides, TIF's Educational Scientists and Coordinator of TIF's electronic educational programmes.

CONCLUSIONS

Dr. Androulla Eleftheriou and Mr. Loris Brunetta concluded on behalf of the TIF President and the Board of Directors with the commitment of producing and sharing with all the participants, recommendations for the next steps forward in the Nordic countries having acknowledged their great efforts to-date and current status with regards to the management of haemoglobin disorders.

ASSESSMENT OF THE VALUE OF THE WORKSHOP - THE PARTICIPANTS' PERSPECTIVE

The greatest percentage **(≥85%)** assessed as very good to excellent the:

- **Quality of the programme**
- **Relevance of the contents to their concerns**
- **Usefulness of the meeting the role of the Associations**
- **Opportunities provided for networking**

*More details can be retrieved from the analysis of the survey - **Appendix I.***

The presentations are to be uploaded soon on TIF's website.

HEMANEXT SIDE MEETING

This side meeting was organized by TIF and Hemanext and held on July 1, 2022. The meeting was attended by thalassaemia and sickle cell disease (SCD) patients, Hemanext representatives and TIF delegates.

In the first part of the meeting, the participating patients were asked to discuss their experience concerning blood transfusion therapy in Nordic countries, including blood transfusion regimens and practices applied in different units attended by the patients and the related drawbacks, as well as the degree of their knowledge on blood processing and storage procedures. This first part continued with a broader discussion on the organization and challenges of haemoglobinopathy care in the context of the Nordic healthcare systems, including patients' expectations, concerns and unmet needs and the way they cope with these challenges.

In the second part, a Hemanext representative presented a new proprietary method for the improvement of the quality of transfused blood. This method constitutes an additional step in pre-storage processing, during which the blood is transferred into a special bag equipped with an internal membrane that extracts oxygen from the stored red blood cells. The extraction of oxygen prevents the oxidative injury of the stored red blood cells, thus improving their survival and the overall quality and efficacy of transfusion, with the potential to reduce transfusion burden and, in turn, transfusional iron overload. The presentation was followed by a further discussion on this method, including its mode of action, related costs and time burden, and the potentials and challenges of its integration in the current blood management settings.

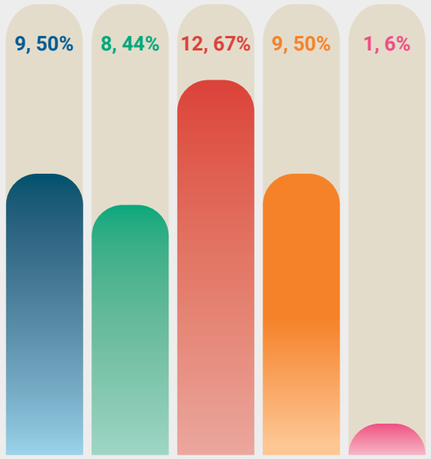
In the final part of the meeting, the patients further discussed their unmet needs in terms of specialized haemoglobinopathy centres, dissemination of disease-specific information and knowledge, access to novel therapies such as luspatercept, advocacy efforts and difficulties and psychosocial challenges. In this context, TIF Executive Officer, Dr. Androulla Eleftheriou, stressed the support that TIF provides to patient groups for promoting education and advocacy.

APPENDIX I

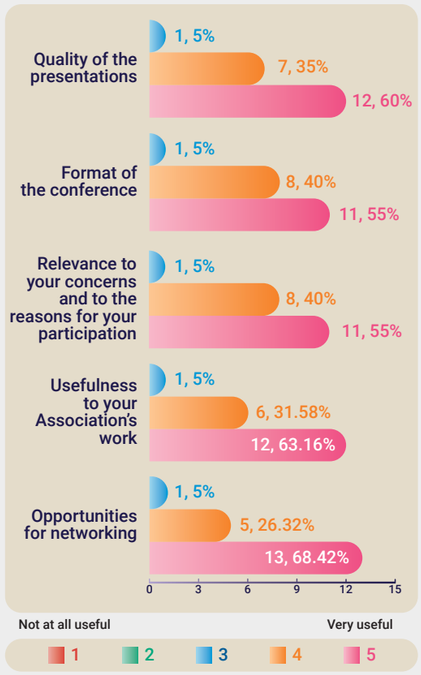
SURVEY PROFILE

11 Patients | 1 Parent | 4 Medical Professionals

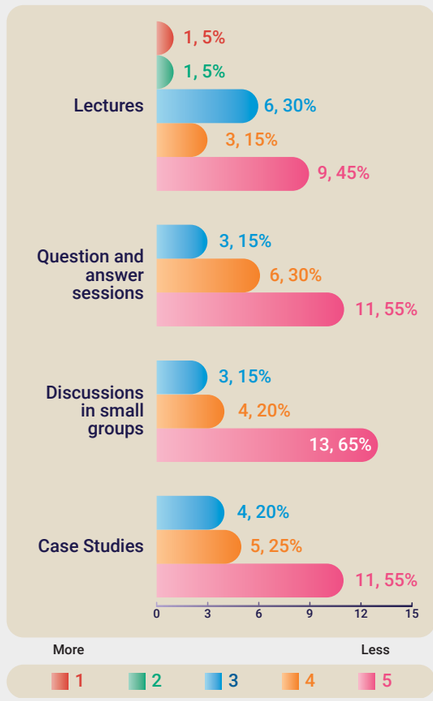
Q: WHAT WERE THE MAIN REASONS FOR YOUR PARTICIPATION IN THIS CAPACITY BUILDING WORKSHOP?



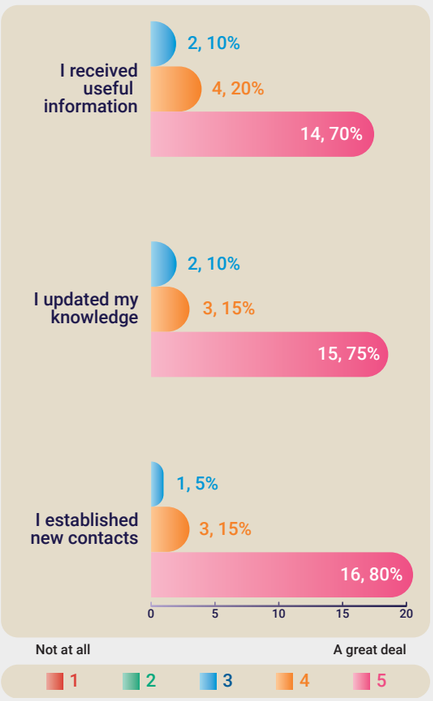
Q: HOW DID YOU FIND THE FOLLOWING?

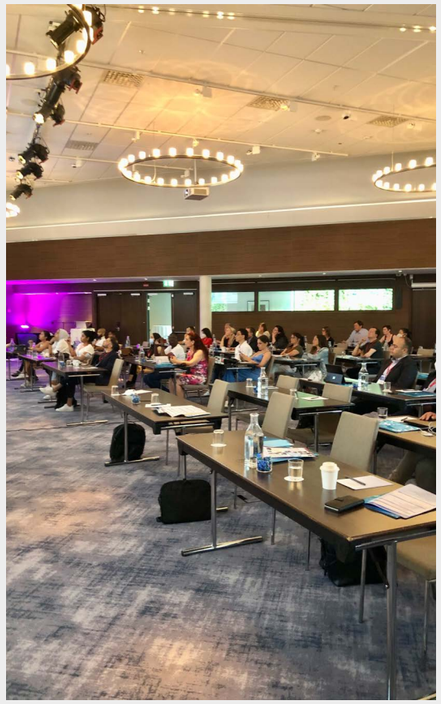


Q: WOULD YOU LIKE TO HAVE HAD MORE OR LESS OF THE FOLLOWING?

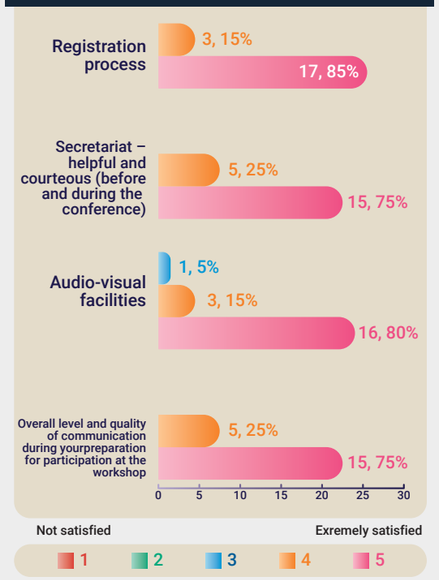


Q: DID THE WORKSHOP MEET YOUR EXPECTATIONS IN THE FOLLOWING WAYS?





Q: HOW SATISFIED WERE YOU WITH THE FOLLOWING ORGANISATIONAL ASPECTS?



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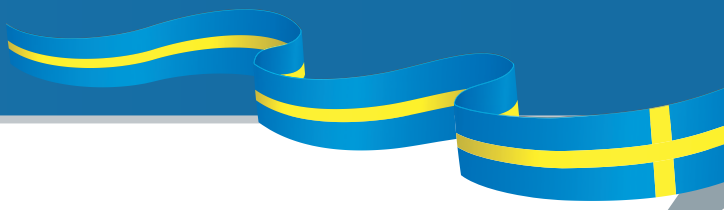

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