CAPACITY BUILDING WORKSHOP

for Thalassaemia Patients & Healthcare Professionals

& 1st RAIN Summit

for Patients with Rare Anaemias

16-19 May 2024

Venue: Grand Hotel Bucharest, Romania

THALASSAEMIA INTERNATIONAL FEDERATION

Organised by: Thalassaemia International Federation

In collaboration with: Asociatia Persoanelor cu Talasemie Majora - Romania

APTM

RAIN Rare Anaemias International Network

THALIA

Co-funded by the European Union

Capacity Building Workshop for Thalassaemia Patients & Healthcare Professionals and **1st RAIN Summit** for Patients with Rare Anaemias

HIGHLIGHTS

- The fundamentals of clinical management of Haemoglobin Disorders
- Multidisciplinary Care in Haemoglobin Disorders
- National Challenges in Addressing Haemoglobin Disorders
- New Era in the Care & Cure of Haemoglobin Disorders: Patient Testimonials
- Recently approved innovative therapies and medicinal products for Thalassaemia
- New Advances in the Pipeline for the Care & Cure of Haemoglobin Disorders
- Treatment Beyond the Haematologist & Paediatrician: Emerging Concerns
- Living with Thalassaemia - Opportunities, Challenges & Dilemmas
- Achieving Healthcare Reforms through Patient Advocacy

THALASSAEMIA INTERNATIONAL FEDERATION

Introduction

The Thalassaemia International Federation is delighted to welcome you to this ground-breaking Capacity Building Workshop for Thalassaemia Patients & Healthcare Professionals, organised in collaboration with Asociatia Persoanelor cu Talasemie Majora (APTM) and the Rare Anaemias International Network (RAIN). The Workshop held in Bucharest, Romania on 17 - 19 May 2024 promises to provide a unique platform for the exchange of knowledge, experiences and best practices among all attendees.

Organized in the context of TIF's internationally renowned educational programme, the Workshop' presents an excellent opportunity for participating doctors and patients to exchange knowledge on the latest scientific advances in the field, share concerns and challenges.

A unique occasion for a discussion that will cover the latest advances in research, recent regulatory authorisations of therapies for the care and cure of haemoglobin disorders and other rare anaemias. Simultaneously, there is a special focus on patients' disease education and capacity building. This will facilitate the empowerment of the patient/parent community, whose strong and competent representation and participation is extremely important at all levels of decision-making bodies and processes leading to more productive and successful outcomes.

Objectives:

1. **Empower and build capacities for patients and their families through strengthening their disease specific knowledge.**
2. **Inform the medical and patient communities about the state-of-art clinical management of haemoglobin disorders, the latest scientific advancements in the field, and regulatory developments for new drugs and therapies.**
3. **Extend the knowledge gained in experienced countries to other European countries through sharing experiences and best practices.**
4. **Develop the skills and capacities of patients for the advocacy and productive participation in decision making at the country and regional levels.**
5. **Educate and create core groups of patients in each country to support and strengthen their role in monitoring their own disease.**

ORGANISING COMMITTEE

Mr George Constantinou – Vice President of the Thalassaemia International Federation, UK

Mr Radu Ganescu – President, Asociatia Persoanelor cu Talasemie Majora (APTM)

SPEAKERS & CHAIRPERSONS

Mr Abdullah Al Fahdi – Member of Oman Hereditary Blood Disorders Association, Oman

Mrs Alejandra Watson – Founder and Board President of The Pyruvate Kinase Deficiency Foundation, USA

Mr Angelo Loris Brunetta – President of Associazione Ligure Thalassemici Italy, Treasurer of the Thalassaemia International Federation, Italy

Mr Anwar Ahmad – Member of Madinah Hereditary Blood Disorders Society, Saudi Arabia

Mrs Bessie Calabria – President of the Thalassemia Foundation of Canada, Board Member of the Thalassaemia International Federation, Canada

Mr Carl Lander – International Collaboration Director, Thrive with PK Deficiency, UK

Dr Carsten Werner Lederer – Senior Scientist/Head of Department, Molecular Genetics Thalassaemia Department, Associate Professor, The Cyprus Institute of Neurology & Genetics Nicosia, Cyprus

Prof. David Rees – Paediatric Haematologist at King's College Hospital, London, UK

Prof. Dimitrios Farmakis – Consultant Cardiologist, National and Kapodistrian University of Athens Medical School, Attikon University Hospital, Athens, Greece

Mr E. Hikmet Demiray, Germany

Dr Efthymia Vlachaki – Assistant Professor of Haematology-Hemoglobinopathies, Aristotle University, Thessaloniki, Greece, Adult Thalassemia Unit, Ippokration Hospital, Greece

Dr Evgenia Georganda – Psychologist, Psychotherapist, Athens, Greece

Mr Giorgio Pio Vindigni – Member of UNITED Onlus, Italy

Mr George Constantinou – Vice President of the Thalassaemia International Federation, UK

Prof. Joan-Lluis Vives Corrons – Emeritus Prof. University of Barcelona, Red Blood Cell Disorders and Rare Anaemias Unit, Institute for Leukaemia Research Josep Carreras, Ektacytometry Unit, Clinical Centre for Ambulatory Medicine, Spain

Mr John James – Chief Executive, UK Sickle Cell Society, London, United Kingdom

Prof. John Porter – Professor of Haematology, Head of the Joint Red Cell Unit for UCLH & Whittington Hospitals, Haematology Department, University College London, London, UK

Mrs Katia Pelides – Senior Educational Programme Officer, Thalassaemia International Federation, Cyprus

Prof. Kevin Kuo – Clinician-Investigator and Staff Haematologist, Red Blood Cell Disorders Program, Division of Medical Oncology and Haematology, Department of Medicine, University Health Network Assistant Professor, Division of Haematology, Department of Medicine, University of Toronto Assistant Professor (status), Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health, University of Toronto, Canada

Mrs Lily Cannon – Deputy Director, Thalassaemia International Federation, Cyprus

Mrs Maria Hadjidemetriou – Executive Board Member for the Cooley's Anemia Foundation, Board Member of the Thalassaemia International Federation, USA

Prof. Nicos Skordis – Paediatric Endocrinologist School of Medicine, University of Nicosia, Dept of Paediatric and Adolescent Endocrinology Paedi Centre for Specialized Paediatrics, Cyprus

Dr Panos Kottaridis – Consultant Haematologist at University College London Hospital, Medical Director at HCA Healthcare UK at UCLH, UK

Prof. Pietro Sodani – Scientific Consultant, Haemato- Oncology Paediatric Section, Bone Marrow Transplant Unit, Charitè Hospital Berlin Germany & Azienda Ospedali Riuniti Marche Nord, Pesaro, Italy

Dr Regine Grosse – Paediatric haematologist and oncologist at the University Hospital Hamburg Eppendorf, Germany

Mrs Shobha Tuli – Secretary of the Board, Thalassaemia International Federation (TIF), Thalassemics India, India

Dr Tamara Schryver – Co-Founder and President at Thrive with Pyruvate Kinase Deficiency Organization, USA

THALASSAEMIA INTERNATIONAL FEDERATION

DAY 1 THURSDAY 16 MAY 2024

15.00 – 16.30	Meeting of patient leaders - Central Europe (By invitation only)
17.00 – 18.30	Meeting of patient leaders - Eastern Europe (By invitation only)

DAY 2 FRIDAY 17 MAY 2024

ROOM Name:	**Ronda**
08.30 – 09.00	Registration
	OPENING CEREMONY
09.00 – 09.30	Welcome Messages Panos Englezos, President, Thalassaemia International Federation (TIF) - Pre-recorded George Constantinou, – Vice President of the Thalassaemia International Federation, UK Radu Ganescu, President, Asociatia Persoanelor cu Talasemie Majora Dr Horatiu-Remus Moldovan – Secretary of State at the Ministry of Health, Romania Prof. Dr. Daniel Coriu - Head of Department, Fundeni Clinical Institute, Hematology and Bone Marrow Transplantation, Bucharest.
Session 1	**Clinical Management for Thalassaemia – Progress & advances**
	Chairs: J. Porter & E. Vlachaki
09.30 – 09.55	Blood transfusion therapy – E. Vlachaki
09.55 – 10.20	Iron chelation Management – J. Porter
10.20 – 10.45	Iron Overload: the value of reliable monitoring and assessment – J. Porter
10.45 – 11.00	Q&A
11.00 – 11.30	**Coffee break**
Session 2	**Haemoglobin Disorders: New Developments**
	Chairs: D. Rees & R. Grosse
11.30 – 12.00	Authorized medicinal products – D. Rees
	Q&A
12.00 - 12.35	**The Patients' Perspective**
	Testimonial: Person with Thalassaemia – A. Ahmad Testimonial: Person with Rare Anaemia – T. Schryver Testimonial: Person with SCD – G. Vindigni Q&A

12.35 – 13.00	Overview of ongoing clinical trials in Haemoglobin Disorders – R. Grosse
13.00 – 14.00	**Lunch**
Session 3	**New Advances for the Cure of Haemoglobin Disorders**
	Chairs: P. Sodani & C. Lederer
14.00 – 14.25	Gene Editing for thalassaemia & SCD: where we stand today. Lecture: P. Kottaridis Patient Testimonial: E. Hikmet Demiray
14.25 – 14.50	Hematopoietic Stem Cell Transplantation in thalassaemia – Benefits & Complications – P. Sodani
14.50 – 15.15	Gene addition for thalassaemia & SCD: latest information – C. Lederer
15.15 – 15.30	Q&A
15.30 – 16.00	**Coffee break**
Session 4	**Multidisciplinary care: The third essential cornerstone in the management of Haemoglobin Disorders**
	Chairs: D. Farmakis & N. Skordis
16.00 – 16.25	Cardiac complications in thalassaemia – D. Farmakis
16.25 – 16.50	Impact of bone disease and pain in thalassaemia – N. Skordis
16.50 – 17.15	Management of Fertility and Pregnancy in thalassaemia – N. Skordis
17.15 – 17.30	Q&A
17.30 – 19.00	**Round-Table Discussion: Multi-Generational and Multi-Cultural Dynamics in Haemoglobin Disorders**
	Moderators: G. Constantinou & E. Georganda
	Global Panel of Patients and Healthcare Professionals

DAY 3 – SATURDAY 18 MAY 2024

Session 5	**Healthcare Professionals Session Case Presentations & Exchange of Best Practices**
ROOM Name:	**Rapsodia**
	Chairs: J. Porter & JL. Vives Corrons
09.30 – 09.45	Case presentation: E. Vlachaki
09.45 – 10.00	Q&A
10.00 – 10.15	Case presentation: D. Farmakis
10.15 – 10.30	Q&A
10.30 – 10.45	Case presentation: JL. Vives Corrons
10.45 – 11.00	Q&A

11.00 – 11.30	**Coffee break**
11.30 – 11.45	Case Presentation: D. Rees
11.45 – 12.00	Q&A
12.00 – 12.15	Case Presentation: N. Skordis
12.15 – 12.30	Q&A
12.30 – 12.45	Case Presentation: P. Kottaridis
12.45 – 13.00	Q&A
13.00 – 13.15	Case Presentation: K. Kuo
13.15 – 13.30	Q&A
13.30	**End of HCPs session**

1ST RAIN SUMMIT FOR PATIENTS WITH RARE ANAEMIAS

Joint Session with patient attendees of Capacity Building Workshop

Session 5	**Living with Rare Anaemias**
ROOM Name:	**Ronda**
	Chairs: T. Schryver & C. Ganescu
09.30 – 10.00	Patient Testimonials: Life with a rare anaemia Living with Thalassaemia – C. Ganescu Living with Rare Anaemia – A. Al Fahdi
10.00 – 10.20	Mental Health: Body image and relationships - E. Georganda
10.20 – 10.40	Nutrition in Thalassaemia & PKD - T. Schryver
10.40 – 11.00	How do I tell my child I have a rare anaemia and what it means - A. Watson
11.00 – 11.30	**Coffee break**
Session 6	**Capacity Building for Patient Associations - Breakout working groups**
	Moderator: S. Tuli & G. Constantinou
11.30 – 11.45	Intro presentation: K. Pelides
11.45 – 12.30	Table 1. Fundraising: How to gain supporters Facilitator: R. Ganescu
	Table 2. The Power of Collaboration: Building Effective Partner Networks Facilitator: L. Brunetta

	Table 3. Effective advocacy skills Facilitator: G. Constantinou
	Table 4. The importance of Communication and Visibility for Nonprofit Organizations Facilitator: B. Calabria
12.30 – 13.00	**Interaction & Reports**
13.00 – 14.30	**Lunch**
Session 7	**Management for Rare Anaemias: Guidelines**
	Chairs: D. Farmakis & G. Constantinou
14.30 – 15.00	Guidelines for the Management of Thalassaemia: History of development and methodology – M.D. Cappellini
15.00 – 15.30	New Guidelines for the Management of PKD: Unmet need & value – K. Kuo
15.30 – 16.00	Patients' involvement and perspective: Thal Patient: G. Constantinou PKD Patient: Carl Lander SCD Patient: J. James (Pre-recorded)

DAY 4 – SUNDAY 19 MAY 2024

ROOM Name:	**Rapsodia**
RAIN Members Strategy Session	
	Chairs: J. Porter & JL. Vives Corrons
09.30 – 10.00	Overview of RAIN accomplishments - L. Cannon (Pre-recorded)
10.00 – 10.30	Current Therapeutic Approaches for Rare Anaemias – J. Porter
10.30 – 11.00	New Development on diagnosis of Rare Anaemia and Thalassaemia – JL. Vives Corrons
11.00 – 11.30	**Coffee break**
11.30 – 12.00	Clinical Trials and New advances for the care and cure of Rare Anaemias – K. Kuo
12.00 – 13.00	Interactive Discussion: Future actions and activities **Moderators: G. Constantinou & JL. Vives Corrons**

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