EHA2026 Congress

June 11 - 14, 2026
Stockholm, Sweden

SPECIAL REPORT

The Thalassaemia International Federation (TIF) has successfully participated in the 31st EHA Annual Congress, held this year in a hybrid format between 11 - 14 June 2026 in Stockholm, Sweden.

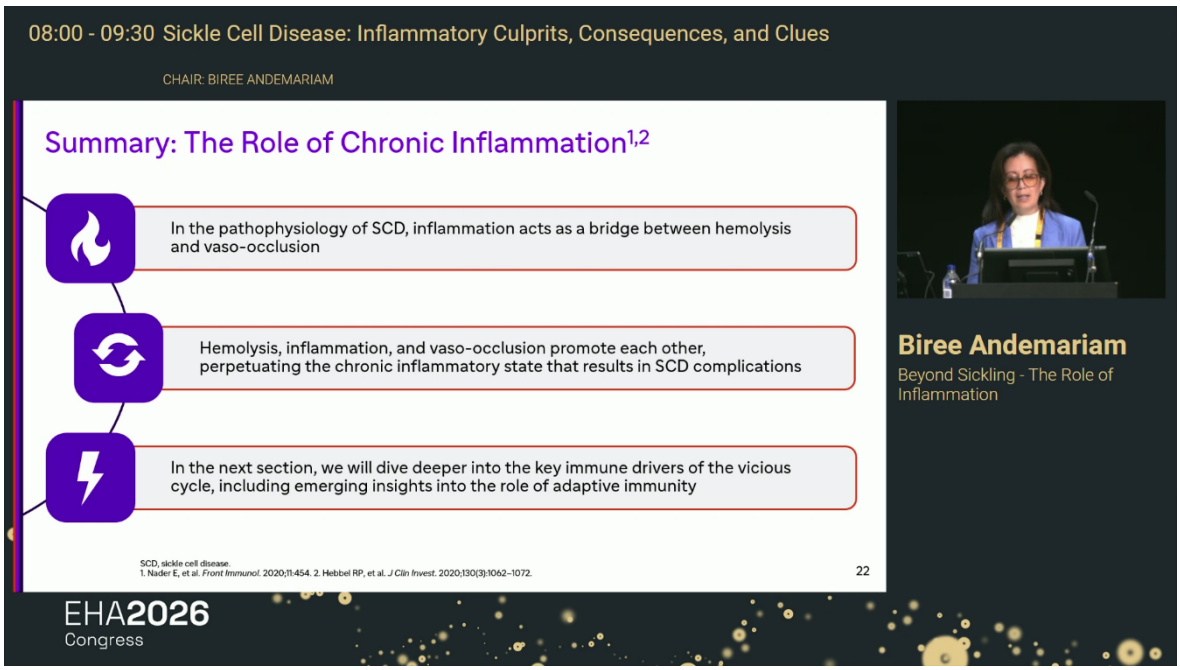
An annual milestone, EHA2026 gathered thousands of haematologists from across Europe and the world to discuss *hot topics* in research, management and cure of haematological diseases, including thalassaemia, sickle cell disease and other rare anaemias – all of which TIF supports the respective communities with its expertise in education and advocacy.

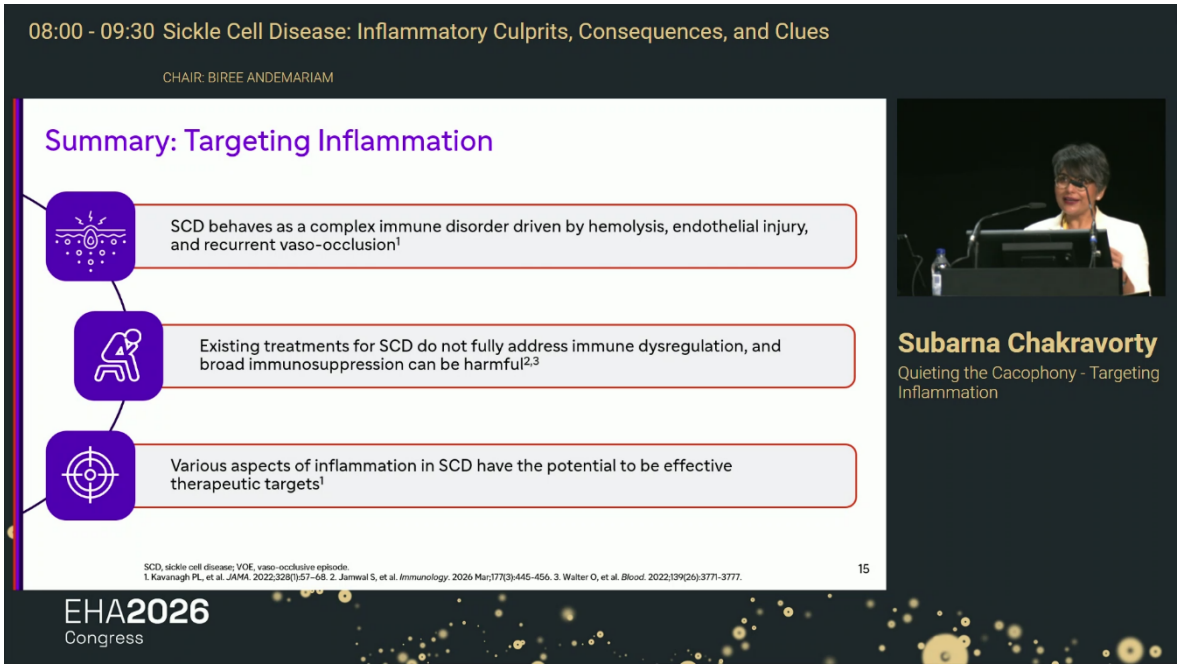
In this Special Report, we provide an overview of TIF's participation in this prestigious event.

Satellite Symposia

- **"Sickle Cell Disease: Inflammatory Culprits, Consequences, and Clues"**

The session, chaired by Dr Biree Andemariam (USA), focused on the role of inflammation in sickle cell disease, with speakers Dr Subarna Chakravorty (UK) and Dr Francesca Vinchi (USA) remarking how the key drivers and damage made to the immune system are not currently addressed by existing and available treatments options and proposing immune dysregulation and targeting inflammation as therapeutic targets of future therapies.

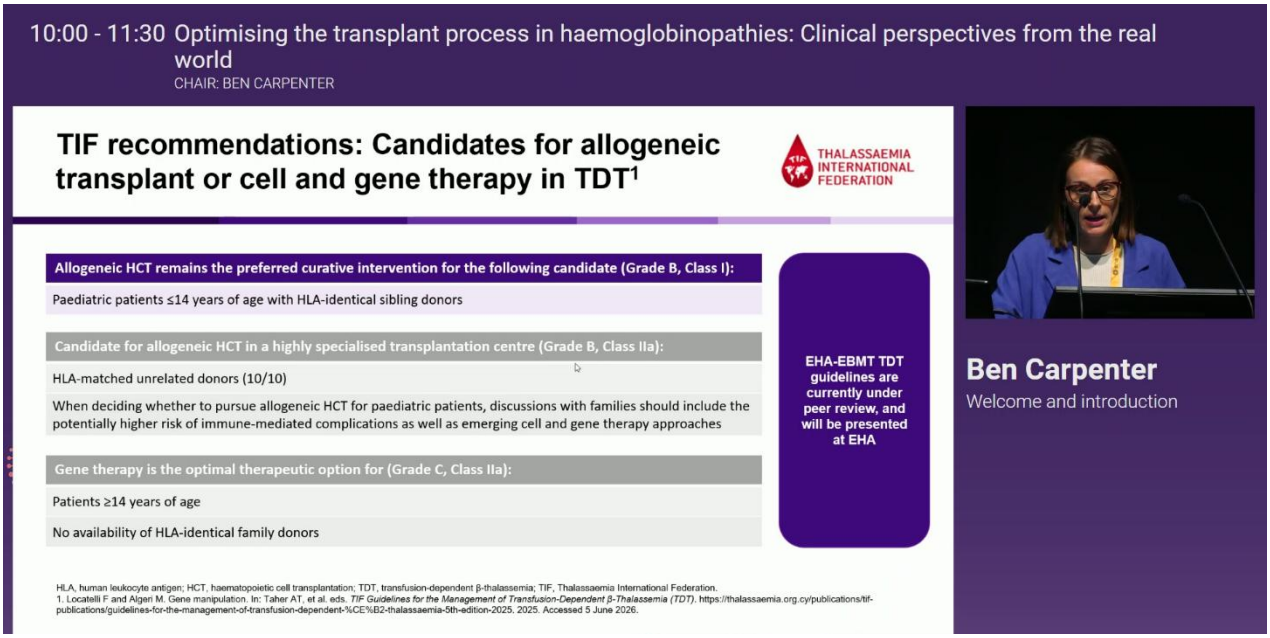


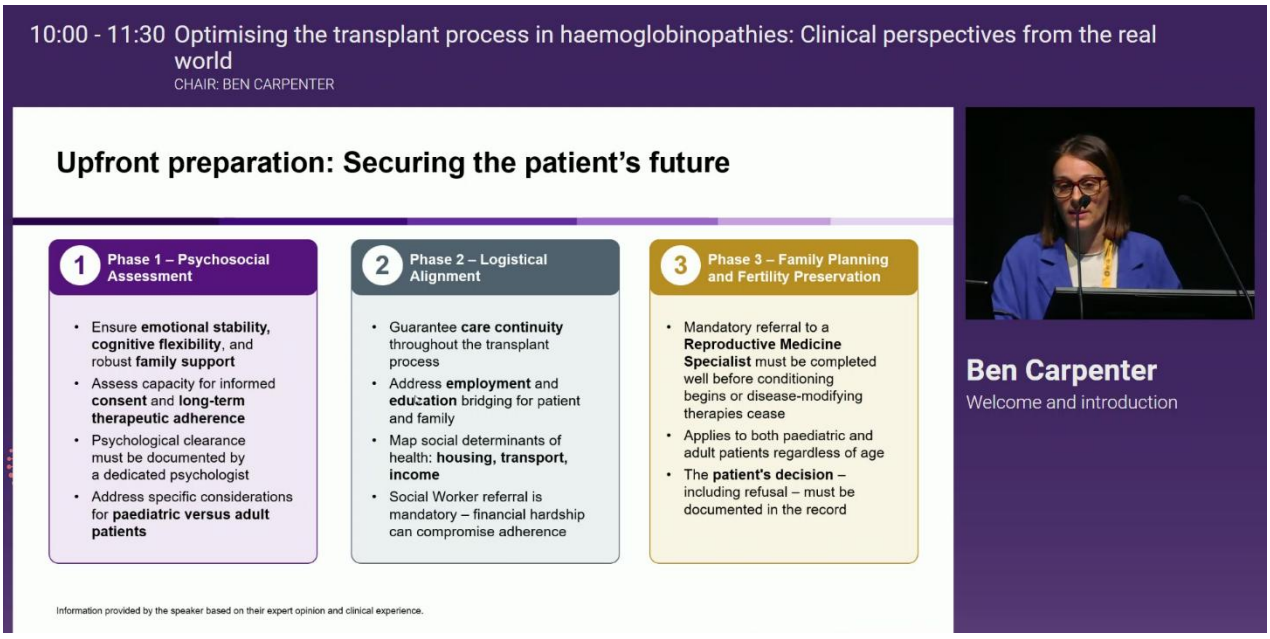


- **‘‘Optimising the transplant process in haemoglobinopathies: Clinical perspectives from the real world’’**

Practical considerations, evolving clinical challenges and social considerations in the pathway for curative treatment of haemoglobinopathies were discussed in a session chaired by Dr Ben Carpenter (UK). With a backdrop of increasing real-world evidence that has accumulated since the authorisation of gene editing for thalassaemia and sickle cell disease in the last few years, the expert panel featuring Dr Irene Motta (Italy), Dr Marco Zecca (Italy), Dr Sami Althubaiti (Saudi Arabia), and Dr Cecilia Langenskiöld (Sweden) highlighted the importance of knowledge exchange between the treating physician and the transplant team to achieve optimal outcomes.

The coordiantion of a multidisciplinary care team is non-negotiable, and must take into consideration to emotional well-being and stability of patients, regardless of age, as their lives transform and their role within the haemoglobinopathy community changes.





10:00 - 11:30 Optimising the transplant process in haemoglobinopathies: Clinical perspectives from the real world

CHAIR: BEN CARPENTER

The Referral Centre MDT: Roles and responsibilities

The Referral Centre MDT prepares patients for handover to the Transplant Centre by ensuring clinical, psychosocial, and logistical readiness

Figure created by the speaker based on their expert opinion and clinical experience. Not an exhaustive list. Additional specialties may be included per institutional standards
MDT, multidisciplinary team.

Ben Carpenter
Welcome and introduction

10:00 - 11:30 Optimising the transplant process in haemoglobinopathies: Clinical perspectives from the real world

CHAIR: BEN CARPENTER

The minimum shared dataset: Bridging both centres[a 1,2]

Genotype and phenotype	Precise genotype including alpha-gene status and exact variant mapping (β0, βE, etc.)
Organ baseline status	Validated cardiac T2* (ms), hepatic LIC (mg/g dw), LSM (kPa), spleen size and morphology, pulmonary function tests, etc.
Transfusion and immunology	Alloimmunisation history; all irregular antibodies; comprehensive anti-HLA antibody screening (DSA status)
Treatment and surgical history	Iron chelation history and tolerance; splenectomy status; prior significant infections; current medications, history of chronic pain
Life planning	Fertility preservation status and documentation; completed psychosocial assessment; family support mapping

[a]Information provided by the speaker based on their expert opinion and clinical experience. Not an exhaustive list. Additional assessments may be included per institutional standards.
DSA, donor-specific antibody; HLA, human leukocyte antigen; LIC, liver iron concentration; LSM, liver stiffness measurement.
1. Frangoul H, et al. *Transplant Cell Ther.* 2025;31(16):352.e1–352.e10; 2. Barba P and Rambaldi A. Evaluation and Counseling of Candidates. In: Sureda A, et al., eds. The EBMT Handbook, European Society for Bone and Marrow Transplantation; 2024:139.

Ben Carpenter
Welcome and introduction

10:00 - 11:30 Optimising the transplant process in haemoglobinopathies: Clinical perspectives from the real world

CHAIR: BEN CARPENTER

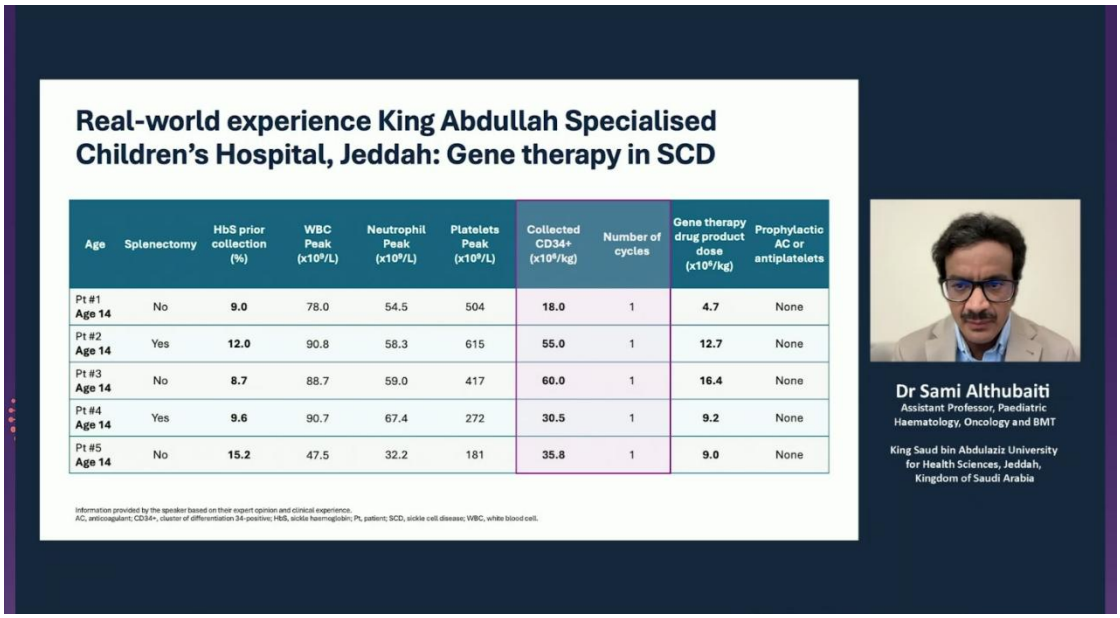
The interface: Co-leading the patient journey

Referral Centre	Shared responsibilities	Transplant Centre
Disease optimisation (chelation, transfusion management)	**Shared decision-making**	Conditioning precision and PK optimisation
Psychosocial grounding and support	**Continuous communication between centres**	Cellular engineering and graft processing
Upfront chelation and transfusion preparation	**Joint family counselling and patient education**	Acute toxicity and VOD management
Patient eligibility assessment	**Transfer of the minimum shared dataset**	Early post-transplant monitoring
Long-term surveillance post-transplant	**Coordinated timing of all pre-transplant steps**	Engraftment and chimerism assessment

Information provided by speaker based on their expert opinion and clinical experience.
PK, pharmacokinetic; VOD, veno-occlusive disease.

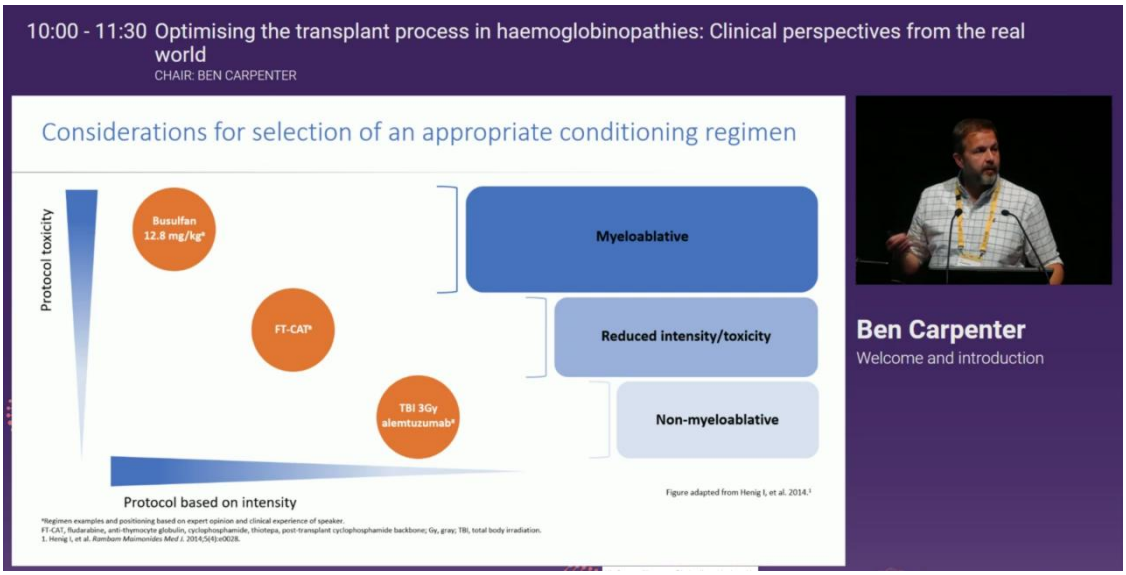
Ben Carpenter
Welcome and introduction

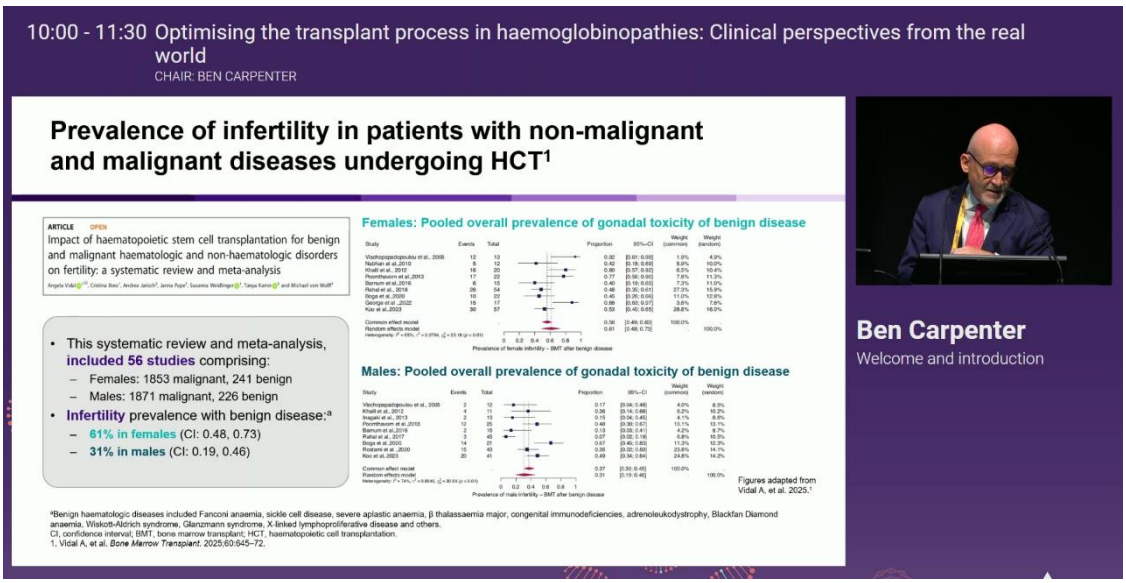
Real-world experience from Saudi Arabia demonstrated the variability in results of conditioning regimens while considerations for selecting approprate conditioning regimens were proposed by the UK. Implications of conditioning particularly in fertility were also presented.



Age	Splenectomy	HbS prior collection (%)	WBC Peak ($x10^9$/L)	Neutrophil Peak ($x10^9$/L)	Platelets Peak ($x10^9$/L)	Collected CD34+ ($x10^6$/kg)	Number of cycles	Gene therapy drug product dose ($x10^6$/kg)	Prophylactic AC or antiplatelets
Pt #1 Age 14	No	9.0	78.0	54.5	504	18.0	1	4.7	None
Pt #2 Age 14	Yes	12.0	90.8	58.3	615	55.0	1	12.7	None
Pt #3 Age 14	No	8.7	88.7	59.0	417	60.0	1	16.4	None
Pt #4 Age 14	Yes	9.6	90.7	67.4	272	30.5	1	9.2	None
Pt #5 Age 14	No	15.2	47.5	32.2	181	35.8	1	9.0	None

Information provided by the speaker based on their expert opinion and clinical experience.
AC, anticoagulant; CD34+, cluster of differentiation 34-positive; HbS, sickle haemoglobin; Pt, patient; SCD, sickle cell disease; WBC, white blood cell.

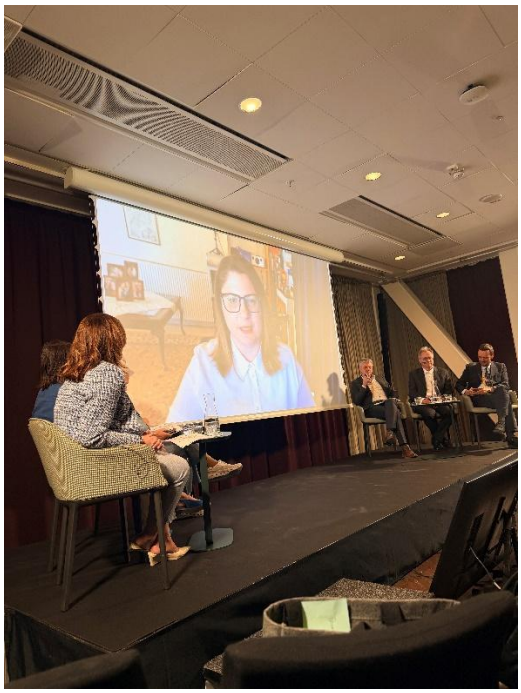




- **‘‘Clinical Decisions That Matter: Thalassaemia & Pyruvate Kinase Deficiency Through a Practical Lens’’**

Diagnosis and clinical management challenges were discussed in this session chaired by Prof. M.D. Cappellini (Italy) and Dr Richard van Wijk (Netherlands). Focusing on inherited haemolytic anaemias – pyruvate kinase deficiency and thalassaemia – speakers delivered compelling case presentations to highlight unmet medical needs and proposed new therapeutic pathways.

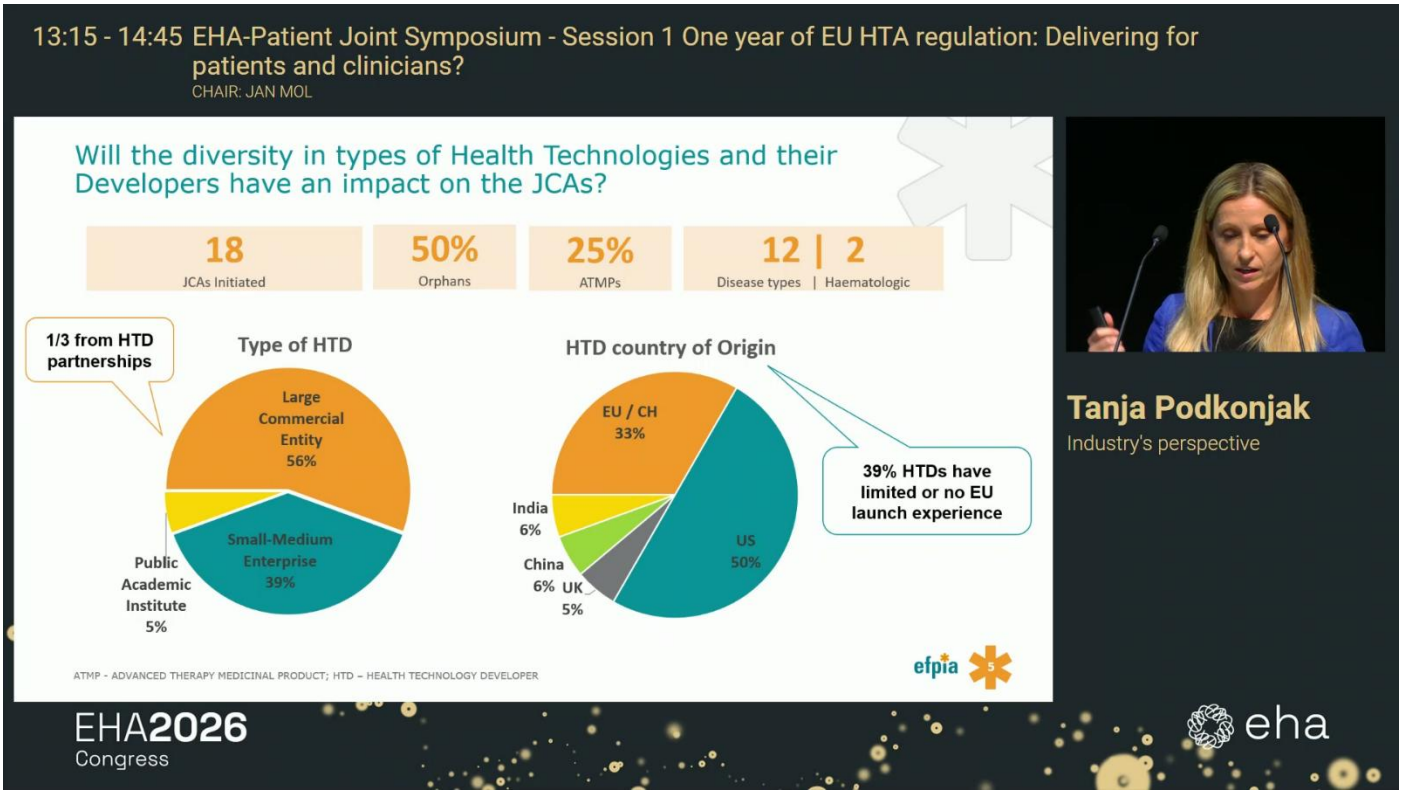
Ms Lily Cannon (TIF Deputy Director) participated with a video presentation which highlighted that thalassaemia care has been transformed by advances in treatment, multidisciplinary care, and stronger patient involvement, improving both survival and quality of life. However, patients continue to face significant treatment burdens and unequal access to care and innovation. Ms Cannon reiterated TIF’s commitment to strengthen and encourage collaboration, physician education, and meaningful patient partnerships, as essential components to achieving meaningful improvements for equitable, patient-centred outcomes.



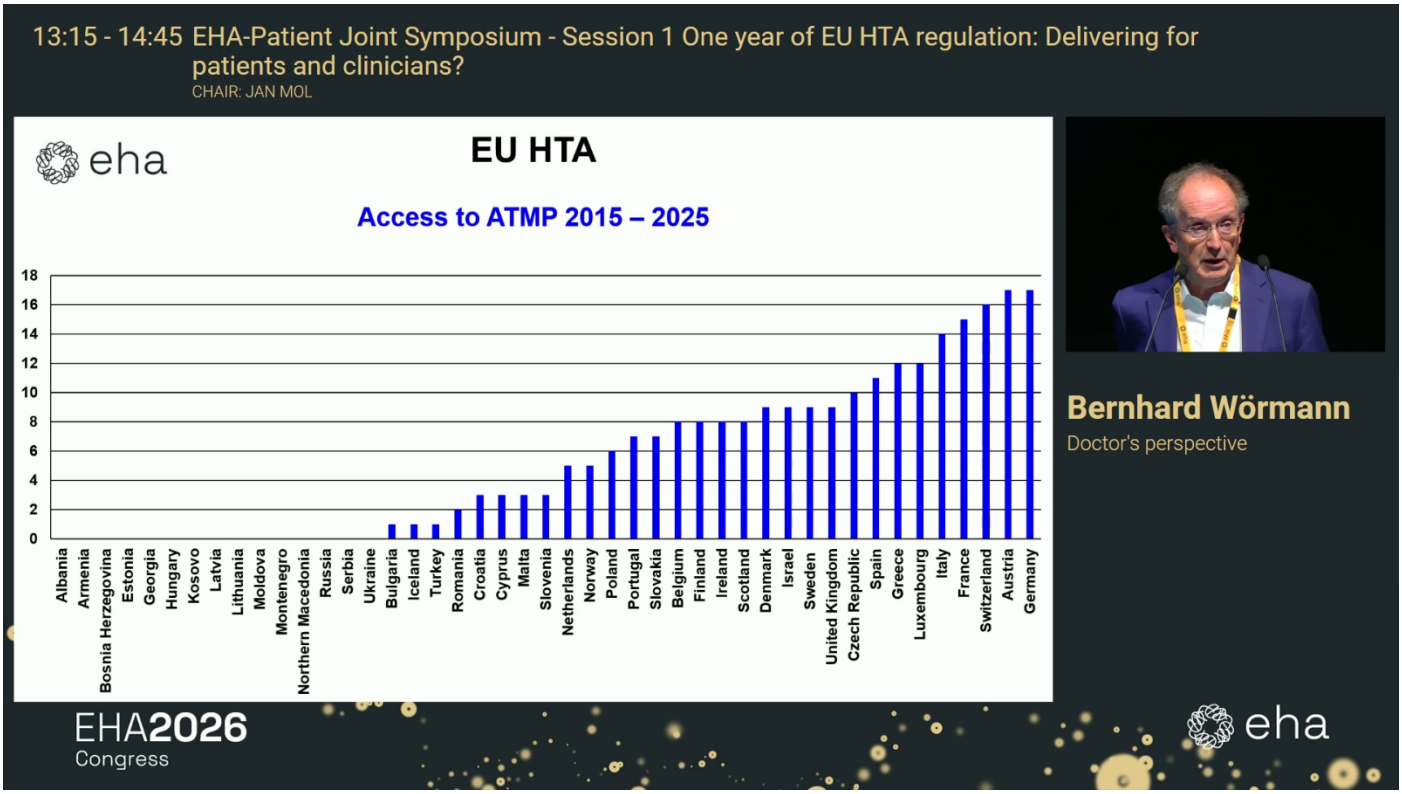
EHA-Patient Joint Symposium 1: One year of EU HTA regulation: Delivering for patients and clinicians?

The Symposium entitled *One year of EU HTA regulation: Delivering for patients and clinicians?* underlined the following:

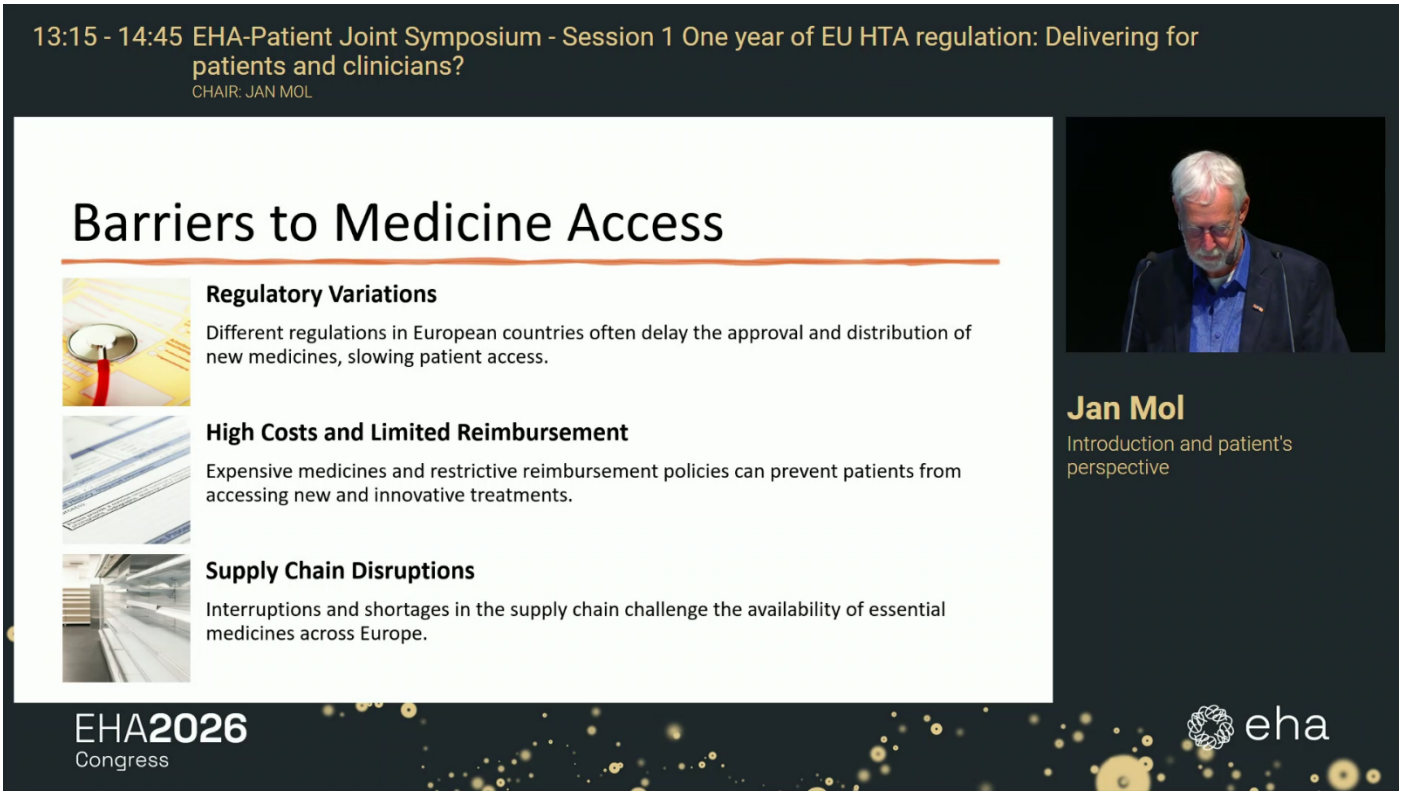
- **HTA cooperation in Europe is advancing rapidly, but implementation remains a work in progress.** The HTA Regulation was highlighted as a major milestone, with Joint Clinical Assessments (JCAs) progressing quickly—the first assessment was adopted just 49 days after marketing authorisation. To date, 18 JCAs have been initiated, including a high proportion of orphan products and ATMPs, but the real test will be how the system is applied at national level as stakeholders navigate a significant learning curve.



- **Innovation in haematology is accelerating, but patient access remains uneven.** The past decade has seen an unprecedented number of new haematology therapies approved, particularly for non-malignant disorders and rare anaemias. However, access to advanced therapies such as ATMPs remains highly limited across Europe, highlighting the gap between regulatory approval and patient availability.



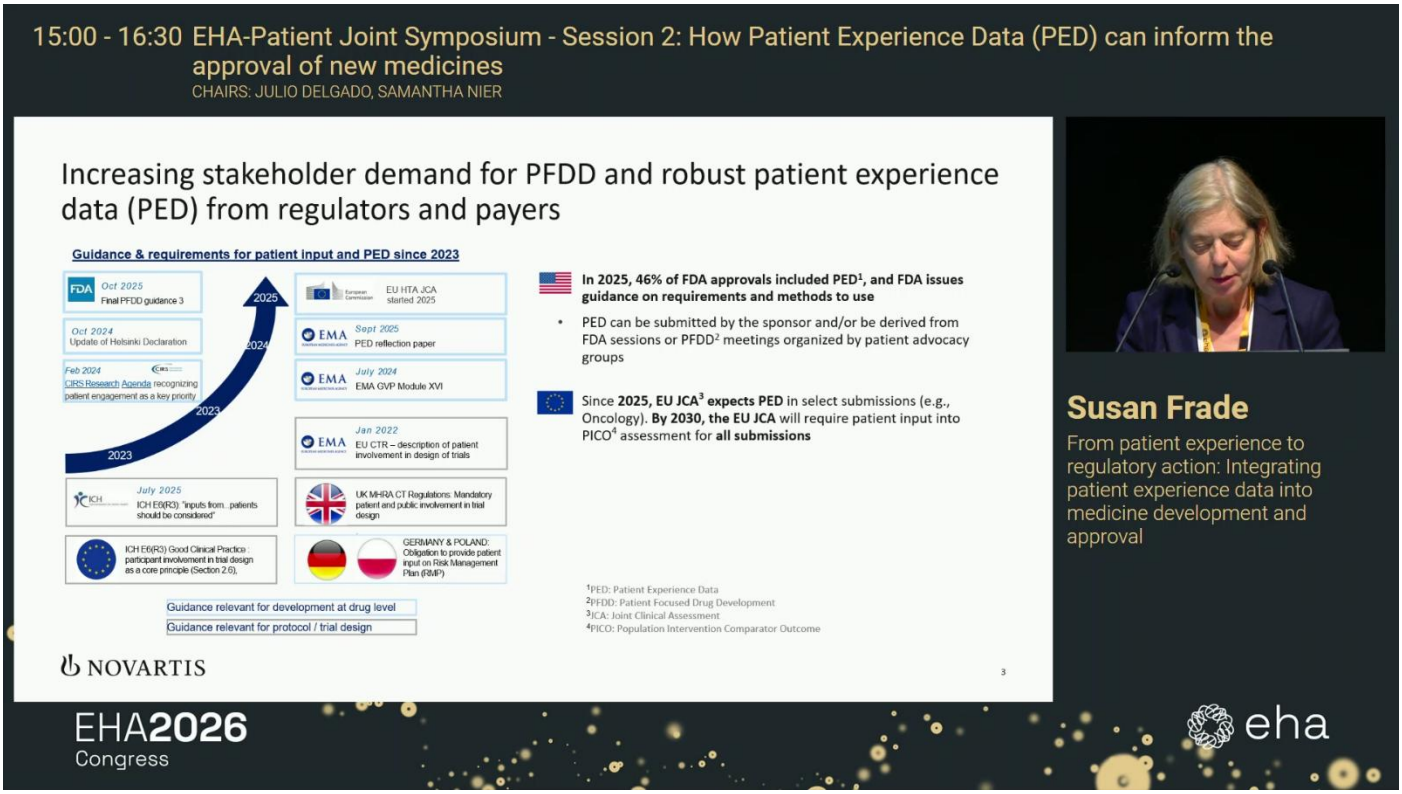
- **Meaningful patient and expert involvement is essential, yet transparency concerns persist.** Speakers stressed the value of patient participation in bringing real-world perspectives into HTA decision-making. At the same time, questions remain about how external expert and patient input is selected, considered, and reflected in assessments, particularly for ultra-rare diseases or conditions without established patient advocacy groups.



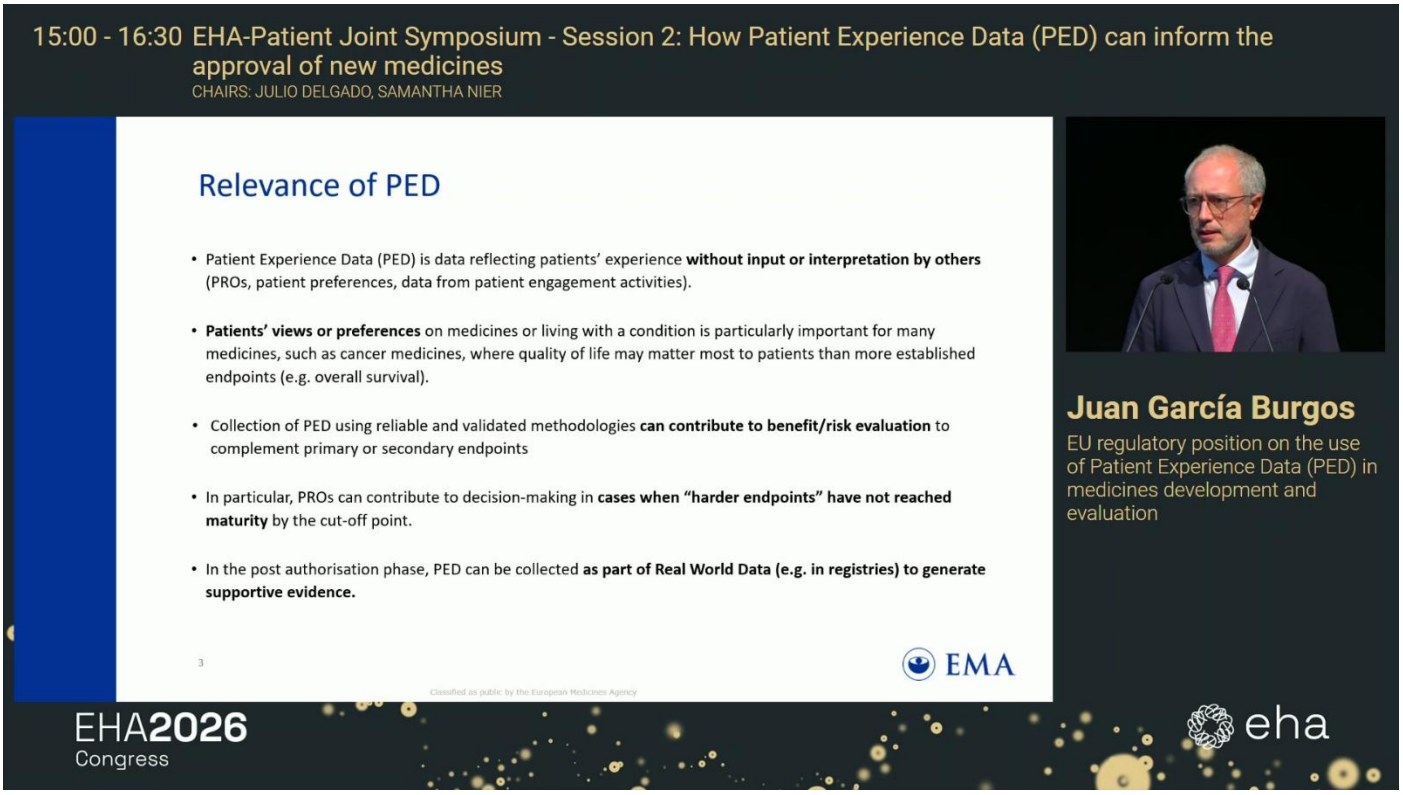
EHA-Patient Joint Symposium 2: How Patient Experience Data (PED) can inform the approval of new medicines

The Symposium entitled *How Patient Experience Data (PED) can inform the approval of new medicines* underscored that:

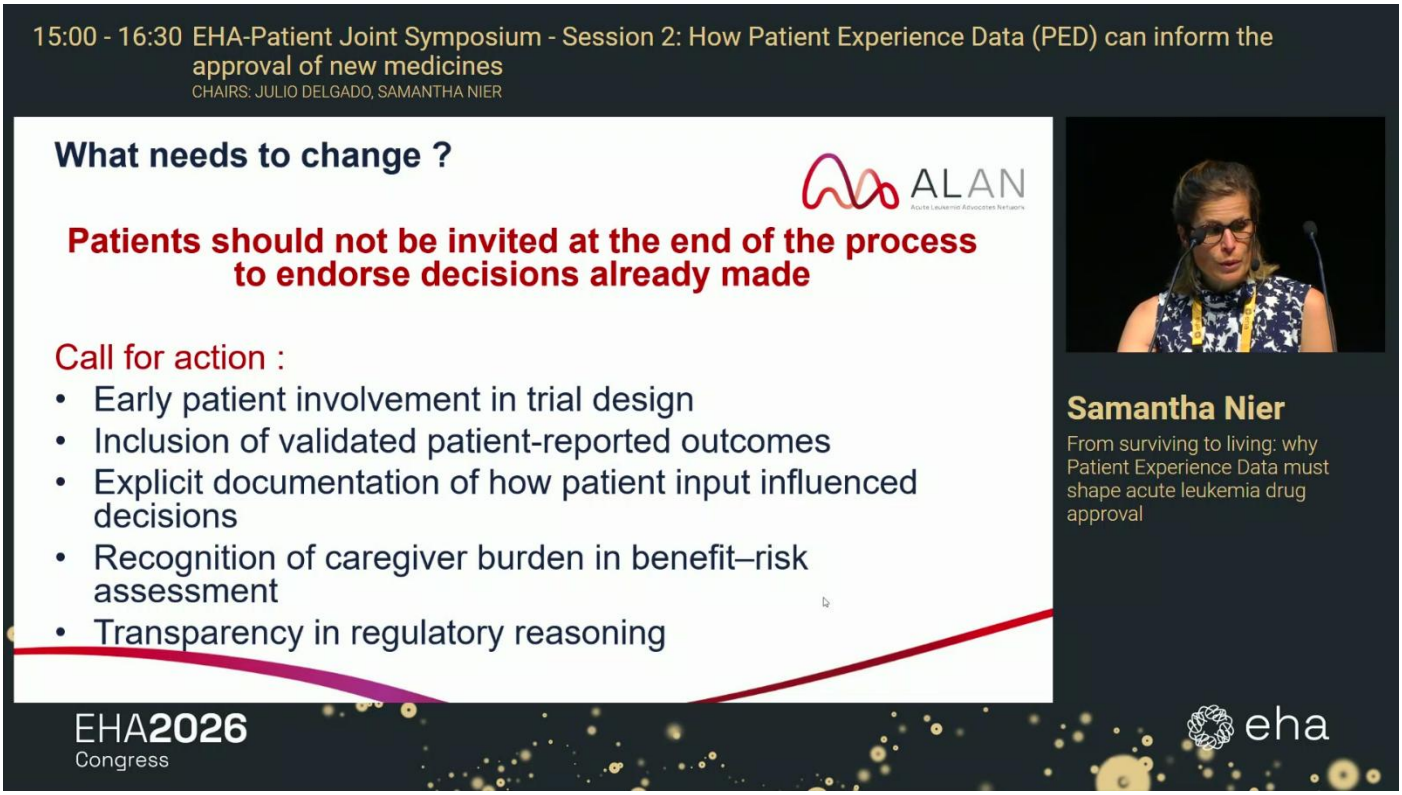
- **Patient Experience Data (PED) is increasingly recognised as a critical component of medicine development and evaluation.** Perspectives from patients, clinicians, regulators, and industry highlighted that while regulators assess evidence, HTA bodies evaluate value, and industry designs trials, patients ultimately live with the outcomes of treatment decisions



- **PED can strengthen regulatory decision-making and clinical research.** By identifying unmet needs, defining outcomes that matter most to patients, and improving trial design, PED helps ensure that new medicines are assessed on factors that reflect real-world patient priorities and experiences.



- **Embedding the patient voice helps ensure medicines deliver meaningful benefits.** Beyond demonstrating clinical effectiveness, PED supports the development and approval of treatments that have a tangible impact on patients' quality of life, with patient organisations such as TIF actively contributing to policy discussions, including recent feedback to the European Medicines Agency's Reflection Paper on PED.



EHA Patient Advocacy Committee

The EHA Patient Advocacy Committee meeting met on **11 June 2026** in the context of the EHA Annual Congress in Stockholm, Sweden. Mr Loris Brunetta (TIF Treasurer) represents TIF in the EHA PAC and currently services as PAC Vice-Chair.

In addition, Mr Brunetta participated in a meeting of the PAC with EHA Board Members on **12 June** and a debrief meeting of PAC members on **14 June**.

The meetings collectively highlighted the milestones accomplished and achievements of the PAC since its establishment in 2022, including:

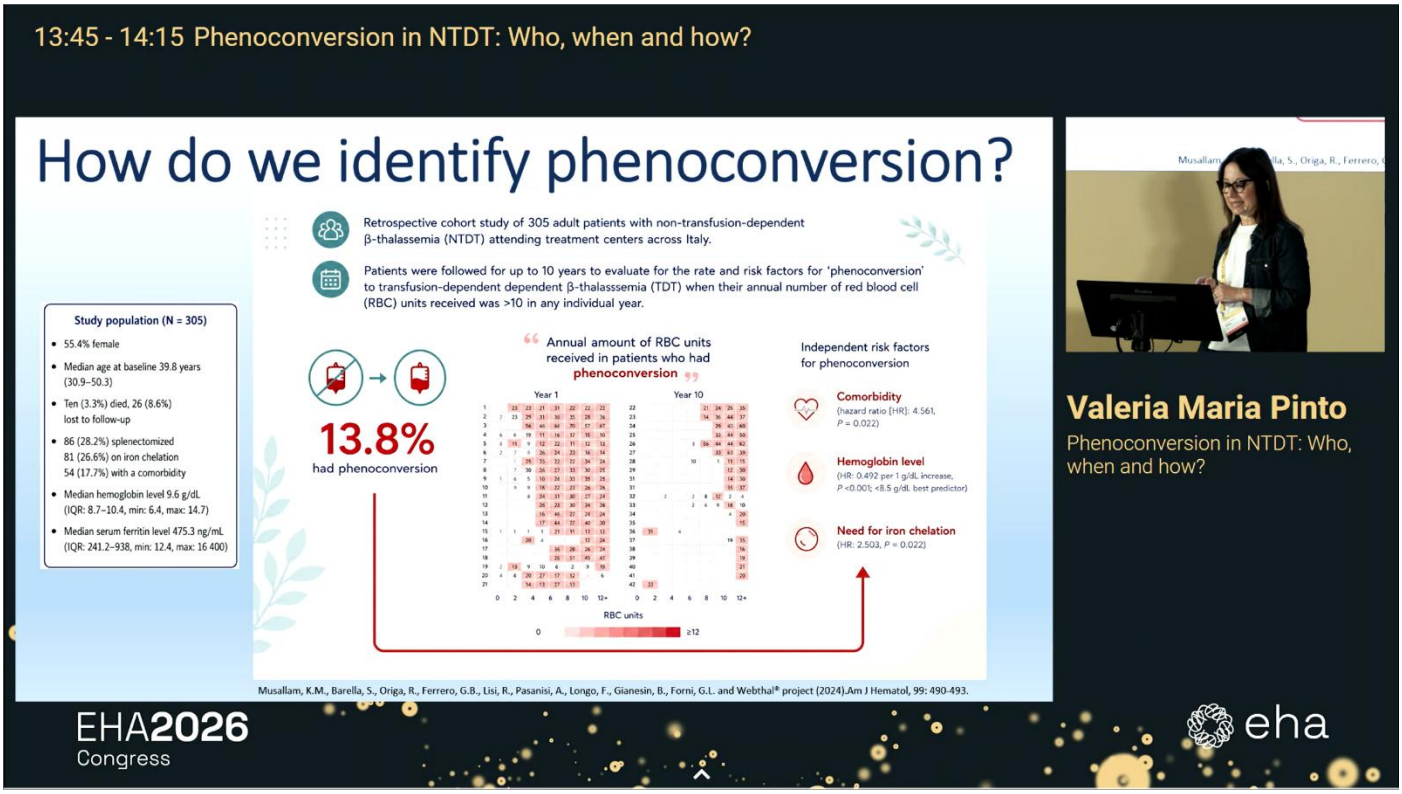
- Increasing number of attendees at the Patient Joint Symposiums at each years Congress
- Ever rising number of patient advocates that are participating as speakers and chairs throughout the congress sessions
- Presence of PAC members on EHA Committees

Furthermore, challenges were also identified including bureaucratic / administrative burdens on volunteer PAC members, limited resource (human, time, financial) availability for PAC activities etc.

Acknowledging the importance to maintain the willingness of the EHA to continue to support the PAC, the meetings provided an opportunity for reflection and set forward a pathway for strengthening and tightening the PAC to amplify its impact and actions.

12 JUNE

Spotlight Session: Phenoconversion in NTDT: Who, when and how?



Take Home Messages

Earlier Stage → Later Stage / Progression

NTDT is not a static disease. Its phenotype evolves over time.

Biological Transition Precedes Transfusion Dependency

Phenoconversion is a progressive process, not a single event. The biological transition often precedes transfusion dependency by years.

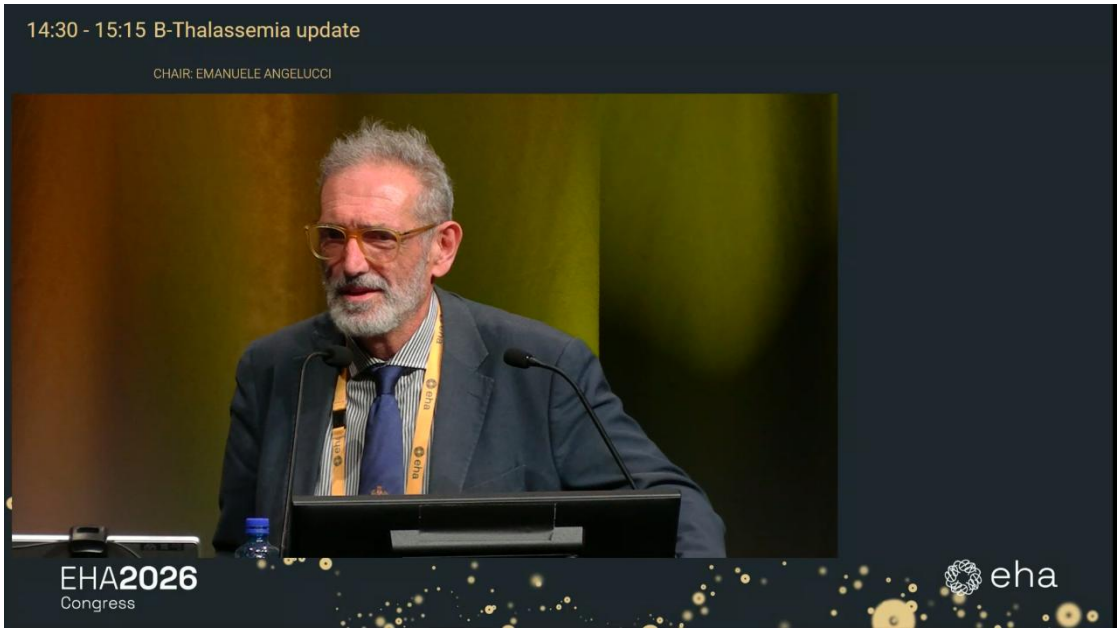
Earlier recognition creates therapeutic opportunities.

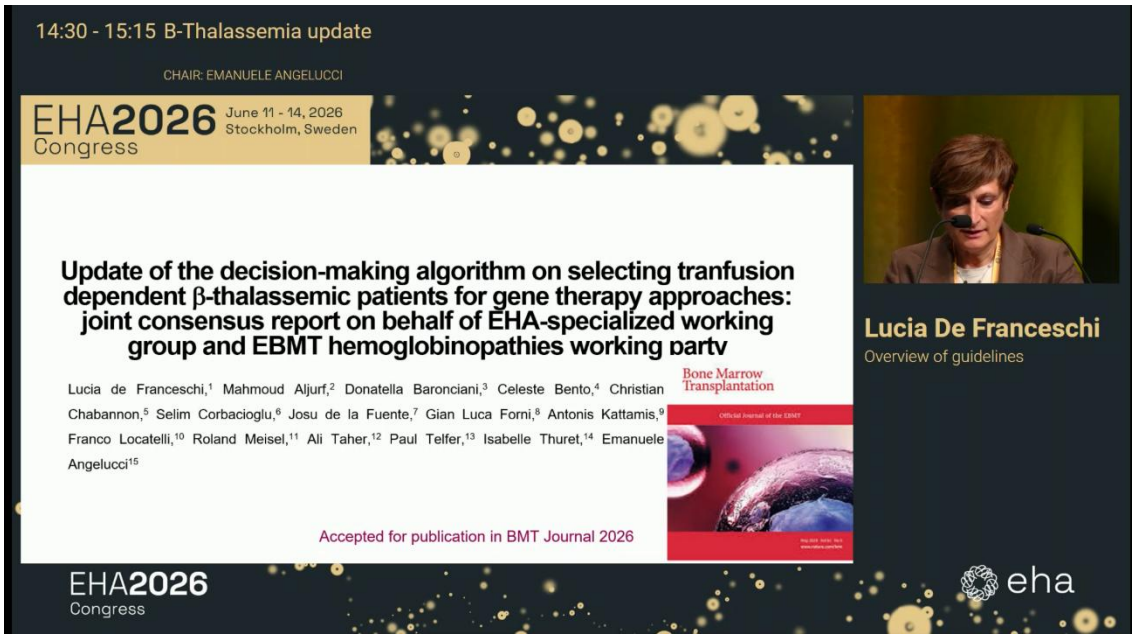
Prevention/Stopping Evolution

The future challenge may not be treating phenoconversion but preventing it.

Guidelines session: B-Thalassemia update

The session chaired by Dr Emanuele Angelucci (Italy), announced the new guidelines for selecting transfusion-dependent thalassaemia (TDT) patients for gene therapy approaches, presented by Dr Lucia de Franceschi (Italy). The updated EHA – EBMT decision-making algorithm that will be published in BMT Journal in 2026 was highlighted, particularly the importance of personalized assessment of eligible patients taking into account lifetime history of organ complications (especially liver) rather than a *snapshot* test. Long-term monitoring is required to ascertain durability as demonstrated by the clinical case presented by Dr Sandrine Visentin (France).





14:30 - 15:15 B-Thalassemia update

CHAIR: EMANUELE ANGELUCCI

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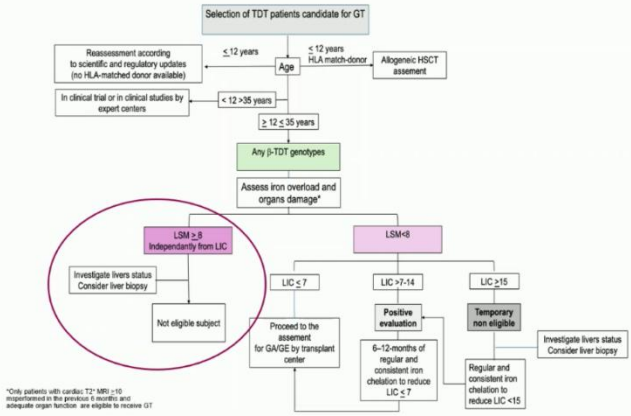
Algorithm for the selection of patients with transfusion dependent thalassemia (TDT), who are candidate for gene therapy approaches in Europe, excluding HSCT



Lucia De Franceschi

Overview of guidelines

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14:30 - 15:15 B-Thalassemia update

CHAIR: EMANUELE ANGELUCCI

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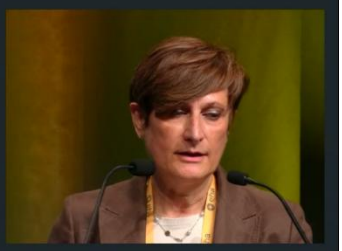
High priority patients	Ineligible patients	Assessable patients undergoing/ongoing changes to therapy (i.e. potential future candidates for gene therapy/gene editing approaches)

• Patients followed by specialized hemoglobinopathies centers or in centers ensuring traceable documentation of patient clinical history
• β-TDT patients aged:
  • ≥12 and < 35 years for GE
  • >=4 and < 35 years for GA
• Genotypes:
  • Any β-TDT genotype (GE/GA)
• patients eligible for allogeneic transplant with no HLA-identical donor
• patients with no significant iron overload (LIC<=7) and LMS<8Kpa
• patients with no evidence of organ damage
• patients who are registered in a qualified transplant center that has experience in hematopoietic stem cell transplant and is connected to a center specialized in the treatment of patients with β-TDT
• good compliance with treatment
• High level of motivation
	• patients not followed by specialized hemoglobinopathies centers or in centers ensuring traceable documentation of patient clinical history
	• β-TDT patients:
	  • age < 4 years (GA)
	  • aged <12 years and >35 years (GE) outside clinical trials or outside controlled studies by expert centers
	• patients with multiplication of alpha globin gene
	• patients with liver fibrosis and iron overload within the following characteristics:
	- LSM ≥8 kPa indipendantly from LIC
	- LSM <8 kPa and LIC > 7 -14 mg/dw without a 6-12-month period of regular and consistent iron chelation to decrease LIC ≤ 7 mg/g dw
	- LSM <8 kPa and LIC ≥15 mg/g dw (situation to be re-evaluated by liver biopsy, temporary not eligible)
	• patients with severe myocardial iron overload demonstrated by a T2* MRI <10 ms documented in the previous 6 months
	• Pulmonary hypertension (determined by cardiac catheterization)
	• patients with chronic organ damage, hepatopathy, insulin-dependent diabetes, nephropathy, positive thrombophilic status
		• patients followed by specialized hemoglobinopathies centers or in centers ensuring traceable documentation of patient clinical history
		• β-TDT patients aged:
		  • ≥12 and < 35 years for GE
		  • >=4 and < 35 years for GA
		• TDT patients with alpha globin gene deletion (GE) in clinical trials
		• patients with LSM =>8 kPa, independently from the LIC (see Figure 1)
		• patients with iron overload: LIC >15 mgFe/g Liver dw – cardiac MRI T2* < 10 ms in the previous 6 months
		• patients free from insulin-dependent diabetes
		• patients with slight and/or reversible cardiopathy
		• patients HCV-RNA/HBV-DNA* positive after achieving sustained viral clearance
		• good compliance with treatment



Lucia De Franceschi

Overview of guidelines

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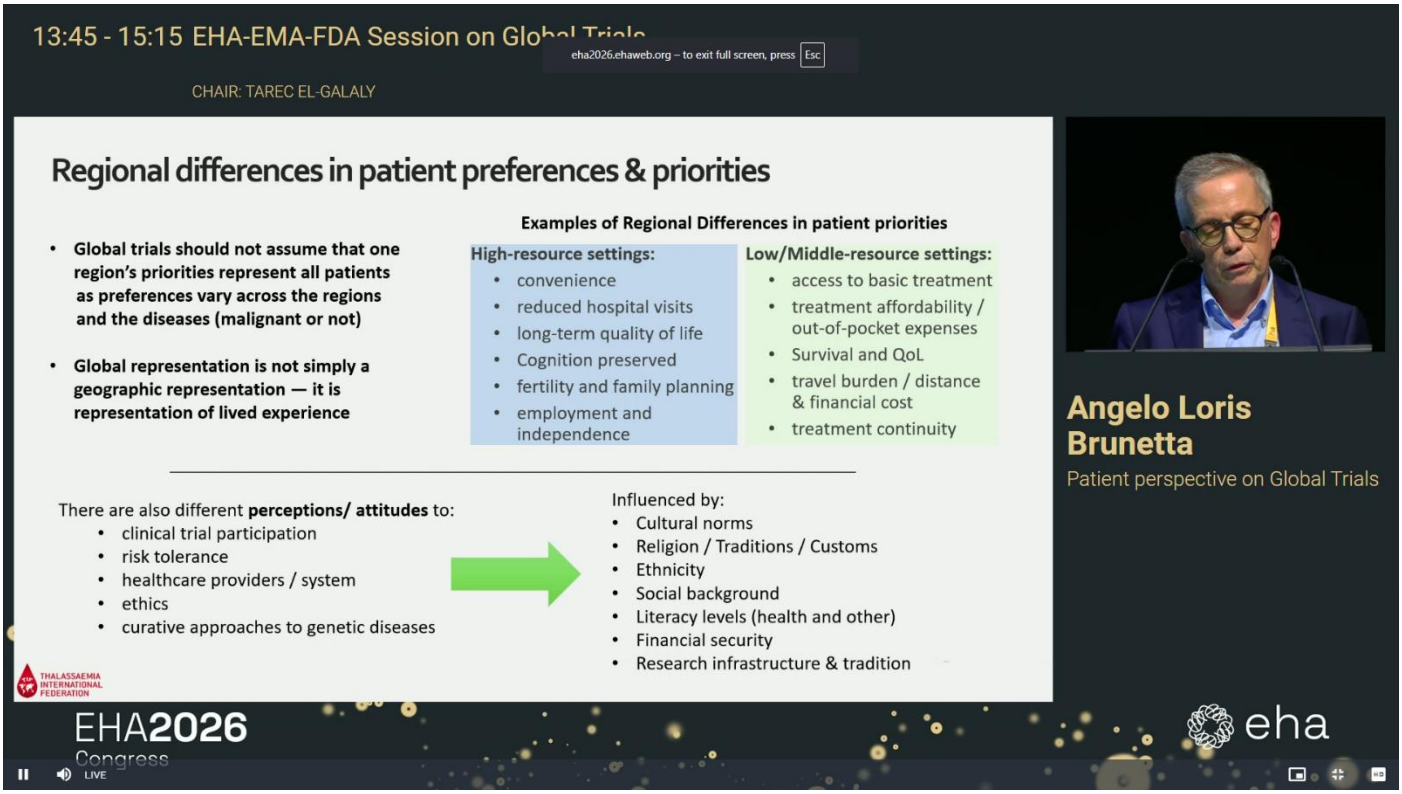
13 JUNE

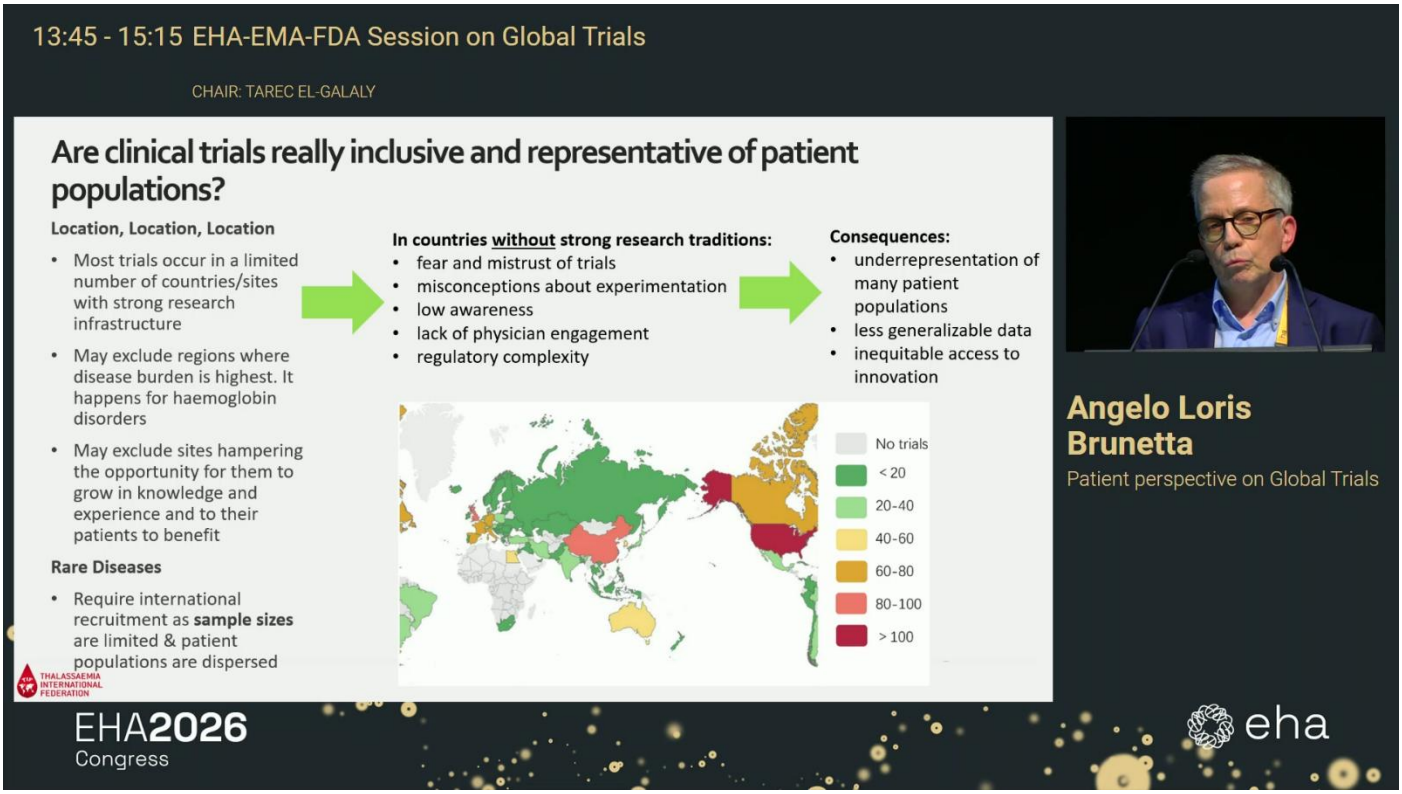
EHA-EMA-FDA Session on Global Trials

This session brought together representatives from the European Hematology Association (EHA), the regulatory agencies in the EU and USA, academia, industry, and patient organizations to discuss the scientific and regulatory considerations of multi-regional clinical trials.

The panel discussion explored the benefits and limitations of conducting trials across multiple regions versus generating globally relevant evidence from studies conducted in only a few countries. Real-world examples presented by representatives of academia, industry and patient organisations, highlighting differences in trial design requirements, legal frameworks, and regional variations in outcomes create challenges for regulatory decision-making and the global applicability of trial results. Representatives of the EMA and FDA noted how regional differences in data can influence regulatory assessments and decision-making.

TIF was represented by Mr Loris Brunetta (TIF Board Member) who emphasized the need to integrate the patient voice throughout the entire clinical development process. Patient involvement helps ensure that trials are inclusive, representative, and authentic by considering regional norms, cultural priorities, and outcomes that are meaningful to patients.





Overall, the discussion concluded that increased global harmonization is needed to develop clinical trials that are scientifically rigorous while remaining responsive to local patient needs, expectations, and regional contexts.

Topics in Focus Session: Hemoglobinopathies

The important advances and emerging challenges in the management of haemoglobin disorders, with a particular focus on thalassaemia were discussed in this session chaired by Dr Megane Brusson (France). Prof. Ali Taher (Lebanon) presented the evolving landscape of non-transfusion-dependent thalassaemia (NTDT), outlining key milestones that have transformed understanding and management of the disease. He highlighted the publication of the Thalassaemia International Federation (TIF) Guidelines since 2013, which have provided a framework for evidence-based care, and noted the significant achievement of dedicated therapies now being authorised specifically for NTDT patients. Prof. Maria Domenica Cappellini (Italy) focused on the changing morbidity profile of thalassaemia as improvements in treatment have resulted in a growing ageing patient population. She discussed emerging health challenges that are increasingly encountered in clinical practice, including cancer, atrial fibrillation, atherosclerosis, and clonal haematopoiesis. These developments underscore the need for long-term surveillance strategies and multidisciplinary care models tailored to the evolving needs of adults living with thalassaemia. Prof. Mariane de Montalembert, Chair of the EHA Hemoglobinopathies Group, presented recommendations on preconception and antenatal screening and prenatal diagnosis for haemoglobinopathies. She emphasised the importance of early identification of at-risk couples and the implementation of appropriate screening and counselling strategies to support informed reproductive choices and improve patient outcomes. Overall, the session demonstrated both the remarkable progress achieved in haemoglobinopathy care and the need to continue adapting clinical practice to address emerging challenges across the patient journey.

13:45 - 15:15 Hemoglobinopathies

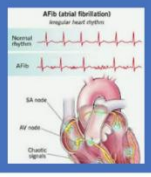
CHAIR: MEGANE BRUSSON

ATRIAL FIBRILLATION

Background

AF prevalence in thalassemia ranges between **2,6%** and **33,8%**

- Old studies
- Young mean age at enrollment



Multicentric observational retrospective study

Inclusione criteria: patients followed between January 1, 2003 and December 31st, 2023 with a diagnosis of β-TDT with at least one episode of AF

Primary Endpoint:
Prevalence of AF in TDT adult patients in Italy

Secondary Endpoints:
- Clinical management of AF in TDT patients in Italy with a focus on **catheter ablation**
- Evaluation of **thromboembolic risk** for AF in β-TDT patients measuring thromboembolic risk based on risk scores/thromboembolic events

9 centers, **1389** β-TDT

Valeria Di Stefano, MD

Di Stefano V, Blood Advances 2026

Maria Domenica Cappellini

Emerging morbidities in thalassemia: Is it a matter of aging?

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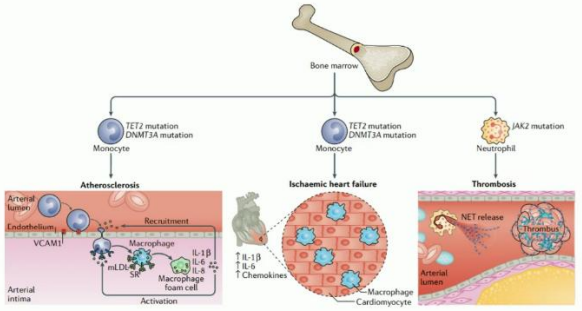
LIVE

13:45 - 15:15 Hemoglobinopathies

CHAIR: MEGANE BRUSSON

CLONAL HEMATOPOIESIS



Poster PS2379 : PREVALENCE AND CLINICAL IMPACT OF CLONAL HEMATOPOIESIS IN PATIENTS WITH BETA-THALASSEMIA", Saturday 13 june 2026

Silvio Loardi, MD

Simona Leoni MD

Claudio Tripodo Lab IFOM

Maria Domenica Cappellini

Emerging morbidities in thalassemia: Is it a matter of aging?

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13:45 - 15:15 Hemoglobinopathies

CHAIR: MEGANE BRUSSON

RECOMMENDATIONS FOR PRECONCEPTUAL AND ANTENATAL SCREENING AND PRENATAL DIAGNOSIS FOR HEMOGLOBINOPATHIES

European Hematology Association-Topic In Focus Hemoglobinopathies Group

Mariane de Montalembert, Maria Domenica Cappellini, Achille Iolascon, Lucia de Franceschi, Eda Ömur, Michele Abi Saad, Immacolata Andolfo, Celeste Bento, Maria Berghs, Doris Bonnet, Andreas Glenthoj, Beatrice Gulbis, Tuphan Kanti Dolai, Jorge Lima, Irene Motta, Roberta Russo, Ali Taher, Leon Tshilolo, Antonio Almeida, David Rees

Mariane de Montalembert

Recommendations for preconceptual and antenatal screening and prenatal diagnosis for hemoglobinopathies

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Oral Presentations & Abstracts

142 **abstracts on Haemoglobinopathies and their related topics, including new therapeutic and curative approaches (authorised and in the pipeline)**

16 **oral presentations relating to Thalassaemia, Sickle Cell Disease and haemoglobinopathies**

Read the scientific updates on new therapies on TIF's website here.