

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.

08:00 - 09:30 Sickle Cell Disease: Inflammatory Culprits, Consequences, and Clues


CHAIR: BIREE ANDEMARIAM

Summary: The Role of Chronic Inflammation^{1,2}

-  In the pathophysiology of SCD, inflammation acts as a bridge between hemolysis and vaso-occlusion
-  Hemolysis, inflammation, and vaso-occlusion promote each other, perpetuating the chronic inflammatory state that results in SCD complications
-  In the next section, we will dive deeper into the key immune drivers of the vicious cycle, including emerging insights into the role of adaptive immunity

SCD, sickle cell disease.
1. Nader E, et al. *Front Immunol*. 2020;11:454. 2. Hebbel RP, et al. *J Clin Invest*. 2020;130(3):1062-1072.

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Biree Andemariam
Beyond Sickling - The Role of Inflammation

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08:00 - 09:30 Sickle Cell Disease: Inflammatory Culprits, Consequences, and Clues


CHAIR: BIREE ANDEMARIAM

Summary: Targeting Inflammation

-  SCD behaves as a complex immune disorder driven by hemolysis, endothelial injury, and recurrent vaso-occlusion¹
-  Existing treatments for SCD do not fully address immune dysregulation, and broad immunosuppression can be harmful^{2,3}
-  Various aspects of inflammation in SCD have the potential to be effective therapeutic targets¹

SCD, sickle cell disease; VOE, vaso-occlusive episode.
1. Kavanagh PL, et al. *JAMA*. 2022;328(1):57-68. 2. Jamwal S, et al. *Immunology*. 2026 Mar;177(3):445-456. 3. Walter O, et al. *Blood*. 2022;139(26):3771-3777.

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Subarna Chakravorty
Quieting the Cacophony - Targeting Inflammation

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- **“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 coordination 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

TIF recommendations: Candidates for allogeneic transplant or cell and gene therapy in TDT¹

Allogeneic HCT remains the preferred curative intervention for the following candidate (Grade B, Class I):

- Paediatric patients ≤14 years of age with HLA-identical sibling donors

Candidate for allogeneic HCT in a highly specialised transplantation centre (Grade B, Class IIa):

- HLA-matched unrelated donors (10/10)

When deciding whether to pursue allogeneic HCT for paediatric patients, discussions with families should include the potentially higher risk of immune-mediated complications as well as emerging cell and gene therapy approaches

Gene therapy is the optimal therapeutic option for (Grade C, Class IIa):

- Patients ≥14 years of age
- No availability of HLA-identical family donors


EHA-EBMT TDT guidelines are currently under peer review, and will be presented at EHA

Ben Carpenter
Welcome and introduction

HLA, human leukocyte antigen; HCT, haematopoietic cell transplantation; TDT, transfusion-dependent β-thalassaemia; TIF, Thalassaemia International Federation.
1. Locatelli F and Algeri M. Gene manipulation. In: Taher AT, et al. eds. TIF Guidelines for the Management of Transfusion-Dependent β-Thalassaemia (TDT). <https://thalassaemia.org/cy/publications/tif-publications/guidelines-for-the-management-of-transfusion-dependent-ttce%2Fβ2-thalassaemia-5th-edition-2025>. 2025. Accessed 5 June 2026.

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

Upfront preparation: Securing the patient’s future



1 Phase 1 – Psychosocial Assessment

- Ensure **emotional stability, cognitive flexibility, and robust family support**
- Assess capacity for informed consent and **long-term therapeutic adherence**
- Psychological clearance must be documented by a dedicated psychologist
- Address specific considerations for **paediatric versus adult patients**

2 Phase 2 – Logistical Alignment

- Guarantee **care continuity** throughout the transplant process
- Address **employment and education** bridging for patient and family
- Map social determinants of health: **housing, transport, income**
- Social Worker referral is mandatory – financial hardship can compromise adherence

3 Phase 3 – Family Planning and Fertility Preservation

- Mandatory referral to a **Reproductive Medicine Specialist** must be completed well before conditioning begins or disease-modifying therapies cease
- Applies to both paediatric and adult patients regardless of age
- The **patient’s decision** – including refusal – must be documented in the record

Ben Carpenter
Welcome and introduction

Information provided by the speaker based on their expert opinion and clinical experience.

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

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

^aInformation 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 Frangou H et al. *Transplant Cell Ther*. 2023;21(10):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

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 appropriate conditioning regimens were proposed by the UK. Implications of conditioning particularly in fertility were also presented.

Real-world experience King Abdullah Specialised Children's Hospital, Jeddah: Gene therapy in SCD

Age	Splenectomy	HbS prior collection (%)	WBC Peak (x10 ⁹ /L)	Neutrophil Peak (x10 ⁹ /L)	Platelets Peak (x10 ⁹ /L)	Collected CD34+ (x10 ⁹ /kg)	Number of cycles	Gene therapy drug product dose (x10 ⁹ /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.



Dr Sami Althubaiti
Assistant Professor, Paediatric Haematology, Oncology and BMT
King Saud bin Abdulaziz University for Health Sciences, Jeddah, Kingdom of Saudi Arabia

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

CHAIR: BEN CARPENTER

Considerations for selection of an appropriate conditioning regimen

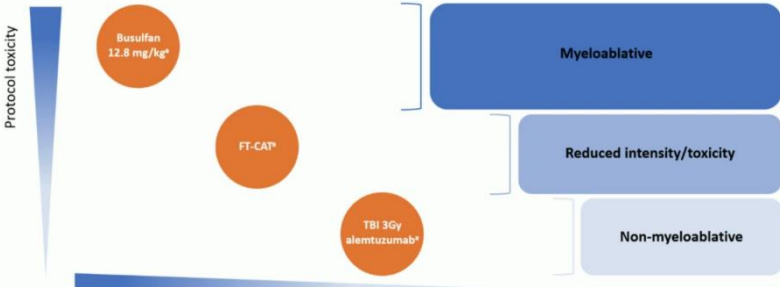



Figure adapted from Heng T, et al. 2014.¹

¹Regimen examples and positioning based on expert opinion and clinical experience of speakers: FT-CAT, Fludarabine, anti-thymocyte globulin, cyclophosphamide, thiotepa, post-transplant cyclophosphamide backbone; Gy, gray; TBI, total body irradiation.
1. Heng T, et al. *Bonmarrow Transplant*. 2014;5(4):e0026.



Ben Carpenter
Welcome and introduction

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

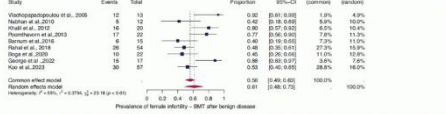
CHAIR: BEN CARPENTER

Prevalence of infertility in patients with non-malignant and malignant diseases undergoing HCT¹

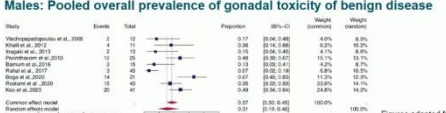
ARTICLE OPEN
Impact of haematopoietic stem cell transplantation for benign and malignant haematologic and non-haematologic disorders on fertility: a systematic review and meta-analysis
Angelo Vidal¹, Cosimo Bovi¹, Andrea Janak¹, Anna Papp¹, Susanna Weidinger¹, Tanya Karam¹ and Michael von Minckwitz¹

- This systematic review and meta-analysis, included 56 studies comprising:
 - Females: 1853 malignant, 241 benign
 - Males: 1871 malignant, 226 benign
- Infertility prevalence with benign disease:^a
 - 61% in females (CI: 0.48, 0.73)
 - 31% in males (CI: 0.19, 0.46)

Females: Pooled overall prevalence of gonadal toxicity of benign disease




Males: Pooled overall prevalence of gonadal toxicity of benign disease



Figures adapted from Vidal A, et al. 2025.¹

^aBenign haematologic diseases included Fanconi anaemia, sickle cell disease, severe aplastic anaemia, β thalassaemia major, congenital immunodeficiencies, adrenoleukodystrophy, Blackfan Diamond anaemia, Wiskott-Aldrich syndrome, Glanzmann syndrome, X-linked lymphoproliferative disease and others.
CI, confidence interval; BMCT, bone marrow transplant; HCT, haematopoietic cell transplantation.
1. Vidal A, et al. *Bone Marrow Transplant*. 2025;60:545-72.

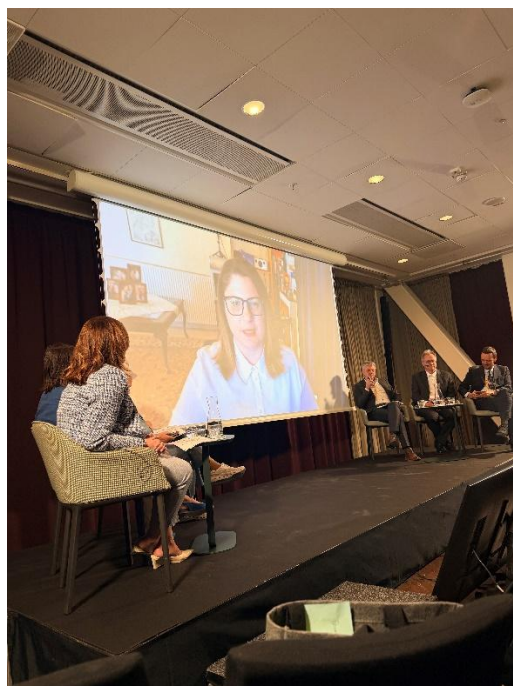


Ben Carpenter
Welcome and introduction

- **“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.

13:15 - 14:45 EHA-Patient Joint Symposium - Session 1 One year of EU HTA regulation: Delivering for patients and clinicians?
CHAIR: JAN MOL

Will the diversity in types of Health Technologies and their Developers have an impact on the JCAs?

18 JCAs Initiated 50% Orphans 25% ATMPs 12 | 2 Disease types | Haematologic

1/3 from HTD partnerships

Type of HTD

Type of HTD	Percentage
Large Commercial Entity	56%
Small-Medium Enterprise	39%
Public Academic Institute	5%


HTD country of Origin

Country	Percentage
EU / CH	33%
US	50%
India	6%
China	6%
UK	5%

39% HTDs have limited or no EU launch experience


ATMP - ADVANCED THERAPY MEDICINAL PRODUCT; HTD - HEALTH TECHNOLOGY DEVELOPER

efpia



Tanja Podkonjak
Industry's perspective

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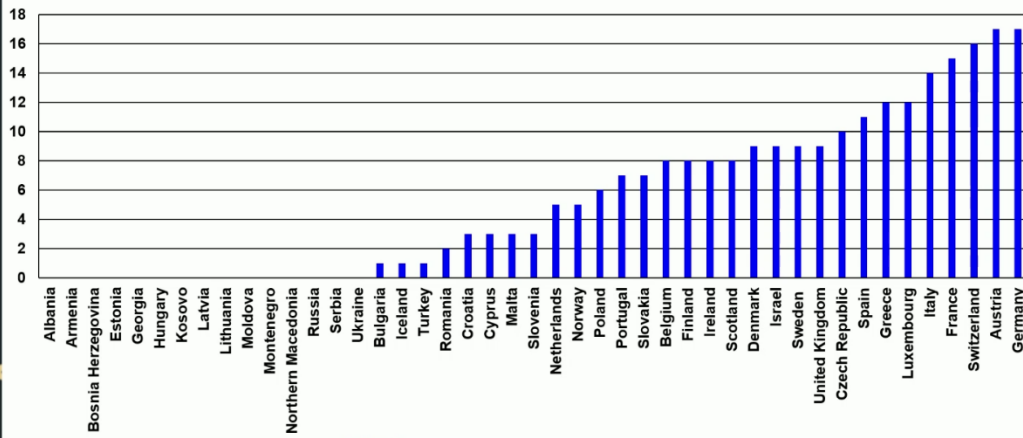


- **Innovation in haematology is accelerating, but patient access remains uneven.** The past decade has seen an unprecedented number of new haematology therapies approved, particularly for non-malignant disorders and rare anaemias. However, access to advanced therapies such as ATMPs remains highly limited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



EU HTA

Access to ATMP 2015 – 2025



Bernhard Wörmann
Doctor's perspective



- **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.

Barriers to Medicine Access



Regulatory Variations

Different regulations in European countries often delay the approval and distribution of new medicines, slowing patient access.



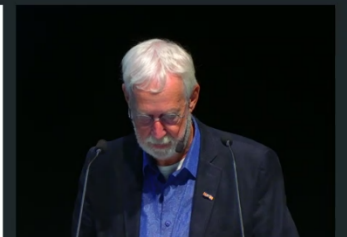
High Costs and Limited Reimbursement

Expensive medicines and restrictive reimbursement policies can prevent patients from accessing new and innovative treatments.



Supply Chain Disruptions

Interruptions and shortages in the supply chain challenge the availability of essential medicines across Europe.



Jan Mol
Introduction and patient's perspective



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

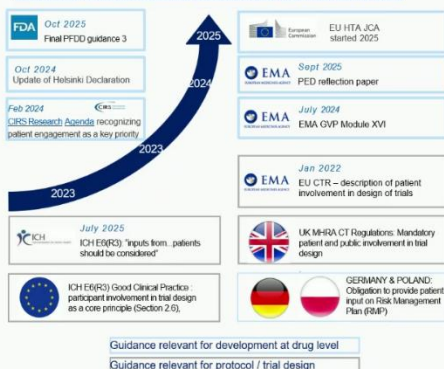
The Symposium entitled *How Patient Experience Data (PED) can inform the approval of new medicines* underscored that:

- **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

15:00 - 16:30 EHA-Patient Joint Symposium - Session 2: How Patient Experience Data (PED) can inform the approval of new medicines
CHAIRS: JULIO DELGADO, SAMANTHA NIER

Increasing stakeholder demand for PFDD and robust patient experience data (PED) from regulators and payers

Guidance & requirements for patient input and PED since 2023



In 2025, 46% of FDA approvals included PED¹, and FDA issues guidance on requirements and methods to use

- PED can be submitted by the sponsor and/or be derived from FDA sessions or PFDD² meetings organized by patient advocacy groups

Since 2025, EU JCA³ expects PED in select submissions (e.g., Oncology). By 2030, the EU JCA will require patient input into PICO⁴ assessment for all submissions

¹PED: Patient Experience Data
²PFDD: Patient Focused Drug Development
³JCA: Joint Clinical Assessment
⁴PICO: Population Intervention Comparator Outcome



Susan Frade

From patient experience to regulatory action: Integrating patient experience data into medicine development and approval

NOVARTIS

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- **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.

Relevance of PED

- Patient Experience Data (PED) is data reflecting patients' experience **without input or interpretation by others** (PROs, patient preferences, data from patient engagement activities).
- **Patients' views or preferences** on medicines or living with a condition is particularly important for many medicines, such as cancer medicines, where quality of life may matter most to patients than more established endpoints (e.g. overall survival).
- Collection of PED using reliable and validated methodologies **can contribute to benefit/risk evaluation** to complement primary or secondary endpoints
- In particular, PROs can contribute to decision-making in cases when **"harder endpoints" have not reached maturity** by the cut-off point.
- In the post authorisation phase, PED can be collected **as part of Real World Data (e.g. in registries) to generate supportive evidence.**

3



Classified as public by the European Medicines Agency



Juan García Burgos

EU regulatory position on the use of Patient Experience Data (PED) in medicines development and evaluation

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- **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.

What needs to change ?



Patients should not be invited at the end of the process to endorse decisions already made

Call for action :

- Early patient involvement in trial design
- Inclusion of validated patient-reported outcomes
- Explicit documentation of how patient input influenced decisions
- Recognition of caregiver burden in benefit–risk assessment
- Transparency in regulatory reasoning



Samantha Nier

From surviving to living: why Patient Experience Data must shape acute leukemia drug approval

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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

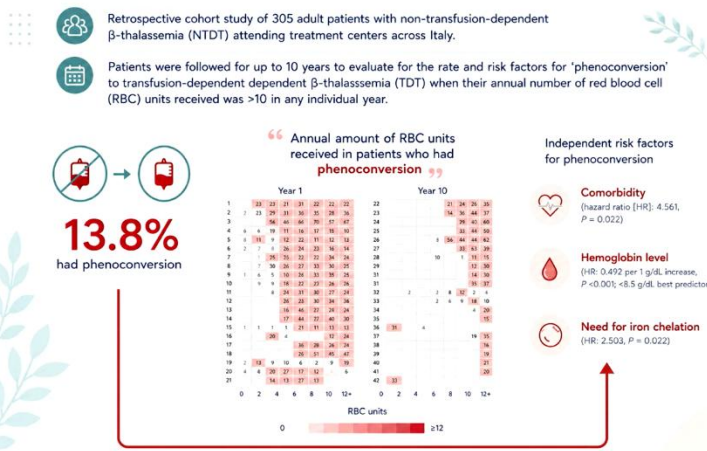
Furthermore, challenges were also identified including bureaucratic / administrative burdens on volunteer PAC members, limited resource (human, time, financial) availability for PAC activities etc.

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.

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

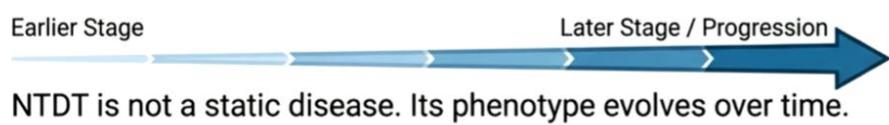
13:45 - 14:15 Phenoconversion in NTDT: Who, when and how?

How do we identify phenoconversion?



Valeria Maria Pinto
Phenoconversion in NTDT: Who, when and how?

Take Home Messages



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



Earlier recognition creates therapeutic opportunities.

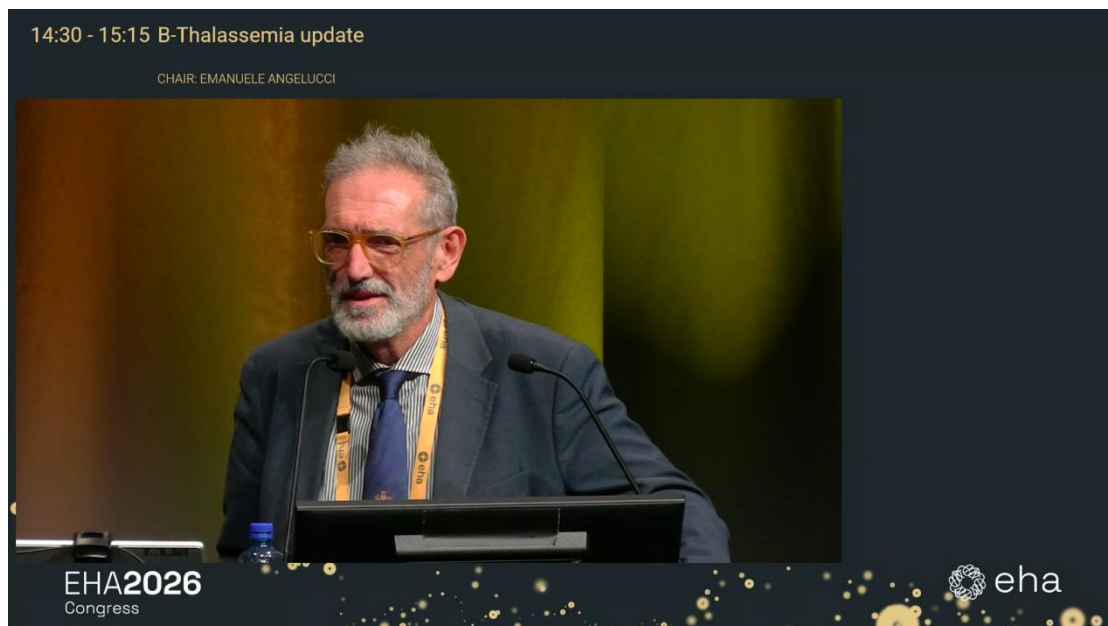


Prevention/Stopping Evolution

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 ANGIUCCI

EHA2026 Congress June 11 - 14, 2026 Stockholm, Sweden

Update of the decision-making algorithm on selecting tranfusion dependent β -thalassemic patients for gene therapy approaches: joint consensus report on behalf of EHA-specialized working group and EBMT hemoglobinopathies working partv

Lucia de Franceschi,¹ Mahmoud Aljurf,² Donatella Baronciani,³ Celeste Bento,⁴ Christian Chabannon,⁵ Selim Corbacioglu,⁶ Josu de la Fuente,⁷ Gian Luca Forni,⁸ Antonis Kattamis,⁹ Franco Locatelli,¹⁰ Roland Meisel,¹¹ Ali Taher,¹² Paul Telfer,¹³ Isabelle Thuret,¹⁴ Emanuele Angelucci¹⁵

Bone Marrow Transplantation
Official Journal of the EBMT

Accepted for publication in BMT Journal 2026

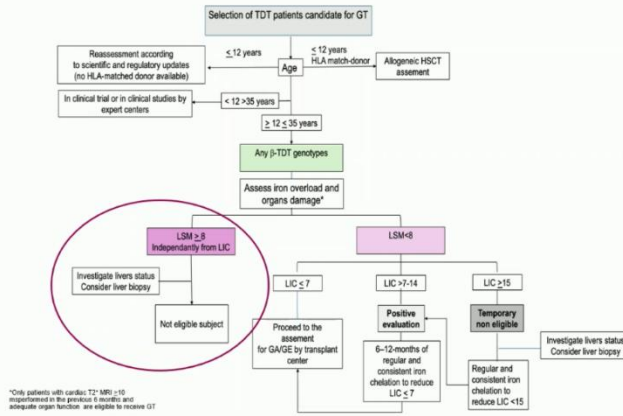
Lucia De Franceschi
Overview of guidelines

EHA2026 Congress

eha

The slide features a white background with black text. On the right side, there is a small inset photograph of Dr Lucia De Franceschi, a woman with short brown hair, wearing a brown jacket. The slide includes the EHA2026 Congress logo, the eha logo, and a small image of a bone marrow transplant slide. The text is centered and clearly legible.

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

High priority patients	Ineligible patients	Assessable patients undergoing/ongoing changes to therapy (i.e. potential future candidates for gene therapy/gene editing approaches)
<ul style="list-style-type: none"> Patients followed by specialized hemoglobinopathies centers or in centers ensuring traceable documentation of patient clinical history β-TDT patients aged: <ul style="list-style-type: none"> ≥12 and < 35 years for GE ≥4 and < 35 years for GA Genotypes: <ul style="list-style-type: none"> 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 LSM<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 	<ul style="list-style-type: none"> patients not followed by specialized hemoglobinopathies centers or in centers ensuring traceable documentation of patient clinical history β-TDT patients: <ul style="list-style-type: none"> 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 with the following characteristics: <ul style="list-style-type: none"> LSM ≥8 kPa independently 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 	<ul style="list-style-type: none"> patients followed by specialized hemoglobinopathies centers or in centers ensuring traceable documentation of patient clinical history β-TDT patients aged: <ul style="list-style-type: none"> ≥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

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.

13:45 - 15:15 EHA-EMA-FDA Session on Global Trials

CHAIR: TAREC EL-GALALY

Global trials – a necessity in hematology



Research progress in hematology can happen everywhere
->Global clinical trials ensures global access to innovation



Important differences may exist in disease biology, patient characteristics, and healthcare infrastructure
->Clinicians need to know that benefit/risk apply to their patients



Hematological diseases increasingly rare due to refined molecular subclassifications
->Global approach necessary for operational reasons



Tarec El-Galaly

Global clinical trials - a necessity for progress in hematology and a priority for EHA

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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.

Multiregional Clinical Trials



- Trials conducted in multiple countries, geographical, or regulatory regions

www.fda.gov

2

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Margret Merino
Multiregional Clinical Trials in Oncology: FDA Experience

Strengths

- Rapid accrual
- Generation of evidence to support use of the drug in multiple regions
- Identification of factors that may predict regional difference
- Improve the ability to conduct trials in rare diseases
- Promote clinical development efficiencies

Challenges

- Differences in patients, disease, medical practice across regions
- Satisfaction of multiple regulatory body requirements
- Applicability of results to local population

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.

How can global patient perspectives be incorporated into clinical trials?

Early Dialogue and Engagement

- Early engagement prevents protocols that are scientifically strong but practically unrealistic
- Patients / Patient Organisations should contribute to:
 - endpoint selection
 - administration models
 - trial burden assessment
 - visit schedules
 - informed consent design
 - acceptable risk-benefit trade-offs
 - geographic locations
- Patients should not only review trials after they are written, providing views that are often disregarded

How?

Structured consultation with mature, experienced and representative regional / international patient organizations.

Role of Patient Organisations



TIF's Expert Patients have participated in Community Advisory Boards, Focus Groups to offer patient perspective in:

- Administration method
- Meaningful end-points
- Quality of life improvement measures



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Regional differences in patient preferences & priorities

- Global trials should not assume that one region's priorities represent all patients as preferences vary across the regions and the diseases (malignant or not)
- Global representation is not simply a geographic representation — it is representation of lived experience

Examples of Regional Differences in patient priorities

High-resource settings:

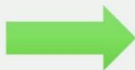
- convenience
- reduced hospital visits
- long-term quality of life
- Cognition preserved
- fertility and family planning
- employment and independence

Low/Middle-resource settings:

- access to basic treatment
- treatment affordability / out-of-pocket expenses
- Survival and QoL
- travel burden / distance & financial cost
- treatment continuity

There are also different perceptions/ attitudes to:

- clinical trial participation
- risk tolerance
- healthcare providers / system
- ethics
- curative approaches to genetic diseases



Influenced by:

- Cultural norms
- Religion / Traditions / Customs
- Ethnicity
- Social background
- Literacy levels (health and other)
- Financial security
- Research infrastructure & tradition



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LIVE



Are clinical trials really inclusive and representative of patient populations?

Location, Location, Location

- Most trials occur in a limited number of countries/sites with strong research infrastructure
- May exclude regions where disease burden is highest. It happens for haemoglobin disorders
- May exclude sites hampering the opportunity for them to grow in knowledge and experience and to their patients to benefit

Rare Diseases

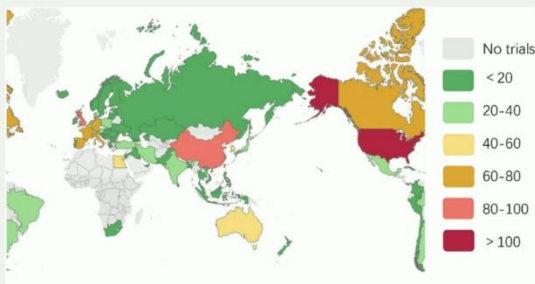
- Require international recruitment as sample sizes are limited & patient populations are dispersed

In countries without strong research traditions:

- fear and mistrust of trials
- misconceptions about experimentation
- low awareness
- lack of physician engagement
- regulatory complexity

Consequences:

- underrepresentation of many patient populations
- less generalizable data
- inequitable access to innovation



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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

Novel considerations for management of ineffective erythropoiesis and anemia in NTDT: TIF 2023 Guidelines

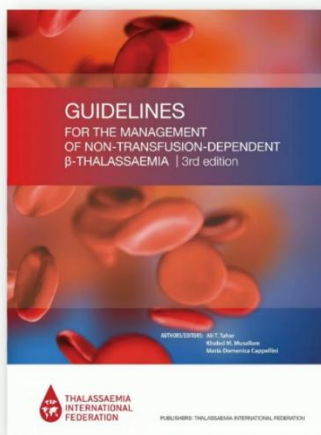


Table 2-1. Considerations for management of ineffective erythropoiesis and anemia in non-transfusion-dependent β -thalassaemia (NTDT).

Hemoglobin level	Ineffective erythropoiesis/ anemia-related symptoms or morbidity	Ineffective erythropoiesis/ anemia-related intervention considerations and treatment objectives
< 10 g/dL	No	<ul style="list-style-type: none"> Long-term intervention to raise hemoglobin level to ≥ 11 g/dL, and prevent symptoms or morbidity
	Yes	<ul style="list-style-type: none"> Short-term (6-12 months) intervention to ameliorate symptoms or morbidity, per physician judgment, and Long-term intervention to raise hemoglobin level to ≥ 11 g/dL, and prevent progression or recurrence of symptoms or morbidity
≥ 10 g/dL	No	None
	Yes	<ul style="list-style-type: none"> Short-term (6-12 months) intervention to ameliorate symptoms or morbidity, per physician judgment, and Long-term intervention to prevent progression or recurrence of symptoms or morbidity, per physician judgment

Grade
<ul style="list-style-type: none"> Long-term (in patients ≥ 18 years) Blood transfusion (a) careful consideration of secondary iron overload (especially in patients with iron-related morbidity such as hepatic and endocrine disease) with long-term intervention and risk of aluminum toxicity Hydroxyurea (in patients with blood pigmentation or leucopenia or β^0-thalassaemia, careful consideration of adverse events and loss of response with long-term intervention) Clinical trials
<ul style="list-style-type: none"> Short-term (2-6 months) objectives to be evaluated at 3-6 monthly intervals or per physician judgment Fatigue, dizziness, weakness, cramps of hands, joint space tenderness, poor development, poor performance at work or school, abnormal quality of life, diminished mental and bone Growth issues (weight or more indicative of growth pattern than weight), failure of secondary sexual development (in parallel with bone age), iron overload, diabetes, cardiovascular disease, pulmonary hypertension, or without secondary heart failure, extramedullary hematopoietic manifestations, hepatosplenomegaly, leg ulcers, osteopenia or osteoporosis, endocrine disease, liver disease, renal disease

In conjunction with other management and prevention strategies indicated for specific symptoms or morbidity.

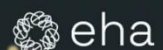


Ali T. Taher

From observation to intervention:
The evolving landscape of Non-Transfusion-Dependent Thalassaemia (NTDT)

Taher AT, Musallam KM, Cappellini MD. TIF NTDT Guidelines 2023.

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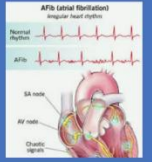


ATRIAL FIBRILLATION

Background

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

- Old studies
- Young mean age at enrollment



Multicentric observational retrospective study

Inclusion 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

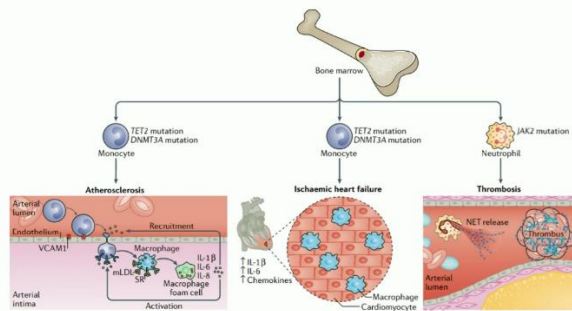
Di Stefano V, Blood Advances 2026



Maria Domenica Cappellini

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

CLONAL HEMATOPOIESIS



Poster P52379 : 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?

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 Glenthøj, 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

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](#).