EBMT

Haemoglobinopathies Working Party Educational Course

Regensburg, Germany
26-28 November 2025

WORD OF WELCOME

On behalf of the EBMT, we are pleased to invite you to the Haemoglobinopathy Meeting, an almost institutional event once again taking place in Regensburg, Germany, from Wednesday, November 26, to Friday, November 28. This meeting is organised as the annual educational event of the Haemoglobinopathy Working Party (HWP) of the EBMT.

The meetings feature a broad range of topics that address nearly all aspects of sickle cell disease and thalassemia. Its highly interactive format, which includes round tables and plenty of space for controversial discussions, enables young investigators to engage in a dynamic process where innovative approaches arise from the ideas and experiences of the world's leading experts in the field. Additionally, the informal atmosphere and enduring friendships within this group are almost palpable, offering an unparalleled experience that has persisted since the first meeting of this group in Regensburg in 2013.

The program has been designed to cover the main aspects of pathophysiology, diagnosis, and treatment of sickle cell disease and thalassemia. The meeting spans two days: the first half day will focus on a keynote lecture and new aspects of conventional therapies, along with the fascinating biology of the bone marrow niche. Thursday will mainly feature various aspects of gene therapy and transplantation, followed by a networking dinner. This day will also include the HWP business meeting, where proposals will be discussed and studies will be planned. Friday will conclude the program, primarily addressing alternative donor transplantation and the considerations of resource- limited countries.

In line with the scientific scope of the WPs and the educational goals of the courses, the meeting will provide participants with the opportunity to discuss and engage with all these experts during the sessions and social events. Furthermore, the meeting will include a one full day scientific programme for the nurses group on Thursday and Friday.

This engaging scientific program will unite the world's top experts in the field who are actively advancing our understanding of haemoglobinopathies.

Emanuele Angelucci - *Haemoglobinopathies Working Party Chair*
Josu de la Fuente - *Haemoglobinopathies Working Party Secretary*
Sandrine Brematha s - *Haemoglobinopathies Working Party Nurse Representative*
Selim Corbacioglu - *Local organiser*

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Wednesday, 26 November 2025

13:00 – 15:00	**Session I: The Bone Marrow Niche in Haemoglobinopathies**	
	Chairs: *Emanuele Angelucci (IT) & Selim Corbacioglu (DE)*	
13:05 – 13:25	Ferroptosis and Iron Haemostasis in BM and Inflammation	Federica Pilo (IT)
13:25 – 13:55	Is SCD a pre-cancerous condition: Clonality in SCD	Michael S. Chapman (UK)
13:55 – 14:15	Ineffective erythropoiesis	Lucia de Franceschi (IT)
14:15 – 14:35	Recent advances in iron homeostasis	John Porter (UK)
14:35 – 15:00	Round table discussion: *Are the intrinsic features of these disorders making transplantation difficult?*	
15:00 – 15:30	**Coffee Break**	
15:30 – 16:00	**Keynote Lecture**	
	Chair: *Josu de la Fuente (UK)*	
	Advances in non-cellular therapies	Kevin Kuo (CA)
16:00 – 17:30	**Session II: The problem of NTDT: an orphan haemoglobinopathy**	
	Chairs: *Federica Pilo (IT) & Miguel Abboud (LB)*	
16:00 – 16:20	Introduction and Natural History of NTDT	Nica Capellini (IT)
16:20 – 16:40	Mechanism of switch to transfusion dependence	Valeria Pinto (IT)
16:40 – 17:00	Modern treatment of NTDT	Donatella Baronciani (IT)
17:00 – 17:30	Round table discussion: *Is there a role for BMT or gene therapy (non-transplanter point of view)?*	
17:30 – 18:00	**Poster Walk**	

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Thursday, 27 November 2025
Main Track

09:00 – 10:30	**Session III (part I): Gene Editing, Gene Therapy, Gene Correction** **Chairs:** *Emanuele Angelucci (IT) & Josu de la Fuente (UK)*	
09:00 – 09:30	Update on Zynteglo for TDT	Mattia Algeri (IT)
09:30 – 10:00	Update on Lytfia for SCD	Julie Kanter (US)
10:00 – 10:30	Update on Casgevy for TDT	Josu de la Fuente (UK)
10:30 – 11:00	**Coffee Break**	
11:00 – 12:30	**Session III (part II): Gene Editing, Gene Therapy, Gene Correction** **Chairs:** *Emanuele Angelucci (IT) & Josu de la Fuente (UK)*	
11:00 – 11:30	Update on Casgevy for SCD	Selim Corbacioglu (DE)
11:30 – 12:00	Update on BEACON study for SCD	Matthew Heeney (US)
12:00 – 12:30	Roundtable discussion: *Are all approaches the same?*	
12:30 – 13:15	**Lunch Break**	
13:15 – 14:15	**Industry Symposium kindly sponsored by Vertex** **Panel perspectives: Learnings from clinical experience across the patient journey**	
13:15 – 13:25	Welcome and introduction	Roland Meisel (DE)
13:25 – 13:45	Optimising the patient journey for gene therapies: Lessons from clinical practice	Rabi Hanna (US)
13:45 – 14:15	Panel discussion and clinical perspectives	All
14:15 – 15:45	**Session III (part III): Gene Editing, Gene Therapy, Gene Correction** **Chairs:** *Rabi Hanna (US) & Josu de la Fuente (UK)*	
14:15 – 14:40	Strategies for successful apheresis collection and post dosing care	Akshay Sharma (US)
14:40 – 15:05	Getting to the 'sweet spot' exposure of Busulfan; minimal toxicity/optimal effect	Jaap Jan Boelens (US)
15:05 – 15:35	Real-world experience: REGENT	Sonali Choudhury (US)
15:35 – 15:45	Round table discussion: *What are the priorities for a successful programme?*	
15:45 – 16:15	**Coffee Break**	
16:15 – 17:00	**Industry Symposium kindly sponsored by Miltenyi** **Alpa-beta T-cell-depleted haploidentical stem cell transplantation, a valid alternative for adults and children suffering from hemoglobinopathies worldwide?**	
16:15 – 16:30	aß T cell-depleted grafts for adults and children with sickle cell disease	Selim Corbacioglu (DE)
16:30 – 16:45	TCR alpha/beta depleted haplo-identical transplants in transfusion dependent thalassemia: experience from India	Sunil Bhat (IN)
16:45 – 17:00	Discussion	Jaap Jan Boelens (US)
17:00 – 18:00	**Session IV: Alternative Donor Transplantation** **Chairs:** *Fabio Giglio (IT) & Matthew Heeney (US)*	
17:00 – 17:20	PTCy RIC transplantation (for adults & children)	Adetola Kassim (US)
17:20 – 17:40	Managing mixed chimerism in aß-depleted tx	Selim Corbacioglu (DE)
17:40 – 18:00	Patient selection for curative therapies	Monica Bathia (US)

For further information, please contact education.events@ebmt.org

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Thursday, 27 November 2025
Nurses' Track

09:30 – 11:00	**Session I: Pre-transplant care**	
	Chairs: *Hilda Mekelenkamp (NL) & Sandrine Brematthas (UK)*	
09:30 – 09:40	Introductions	
09:40 – 10:00	Supportive care in sickle cell disease	Catherine Mkandawire (UK)
10:00 – 10:20	Supportive care in thalassaemia	Majken Høeg Olsen (DK)
10:20 – 10:40	Indication and outcomes of HSCT	Liesbeth Suijk (NL)
10:40 – 11:00	Preparation for standard HSCT	Hilda Mekelenkamp (NL)
11:00 – 11:30	**Coffee Break**	
11:30 – 12:30	**Session II: Conditioning approaches, alternative donor transplantation & patient experience**	
	Chairs: *Marjola Gjergji (IT) & Majken Høeg Olsen (DE)*	
11:30 – 11:50	Use of haploidentical donors for transplants in haemoglobinopathies	Adetola Kassim (US)
11:50 – 12:10	Use of pre transplant immunosuppression (PTIS) in TDT	Donatella Baronciani (IT)
12:10 – 12:20	Having a fully matched sibling bone marrow transplant: my story	Angela Diasivi (UK)
12:20 – 12:30	Discussion	
12:30 – 13:15	**Lunch Break**	
13:15 – 14:15	**Industry Symposium kindly sponsored by Vertex (*Main Room*)**	
14:15 – 15:45	**Session III: Gene Editing, Gene Therapy, Gene Correction**	
	Chairs: *Liesbeth Suijk (NL) & Catherine Mkandawire (UK)*	
14:15 – 14:35	Gene therapy: is this the new way forward?	Marjola Gjergji (IT)
14:35 – 14:55	Gene therapy preparation: how does it differ to HSCT preparation?	Sandrine Brematthas (UK)
14:55 – 15:15	Mobilisation and optimising stem cell collections	Kelly Hennessy (UK)
15:15 – 15:35	Updates from the clinical trials & future trials	Miguel Abboud (LB)
15:35 – 15:45	Case study	Abdul-Qadeer Akhtar (UK)
15:45 – 16:15	**Coffee Break**	
16:15 – 17:00	**Industry Symposium kindly sponsored by Miltenyi (*Main Room*)**	
17:00 – 18:00	**Session IV: The role of MDT members and patient perspective**	
	Chairs: *Hilda Mekelenkamp (NL) & Sandrine Brematthas (UK)*	
17:00 – 17:20	Role of the clinical psychologist for patients with haemoglobinopathies	Becky Armstrong (UK)
17:20 – 17:40	Nutrition in patients with sickle cell disease	Claudine Matthews (UK)
17:40 – 18:00	Patient perspective (TIF)	Loris Angelo Brunetta (IT)

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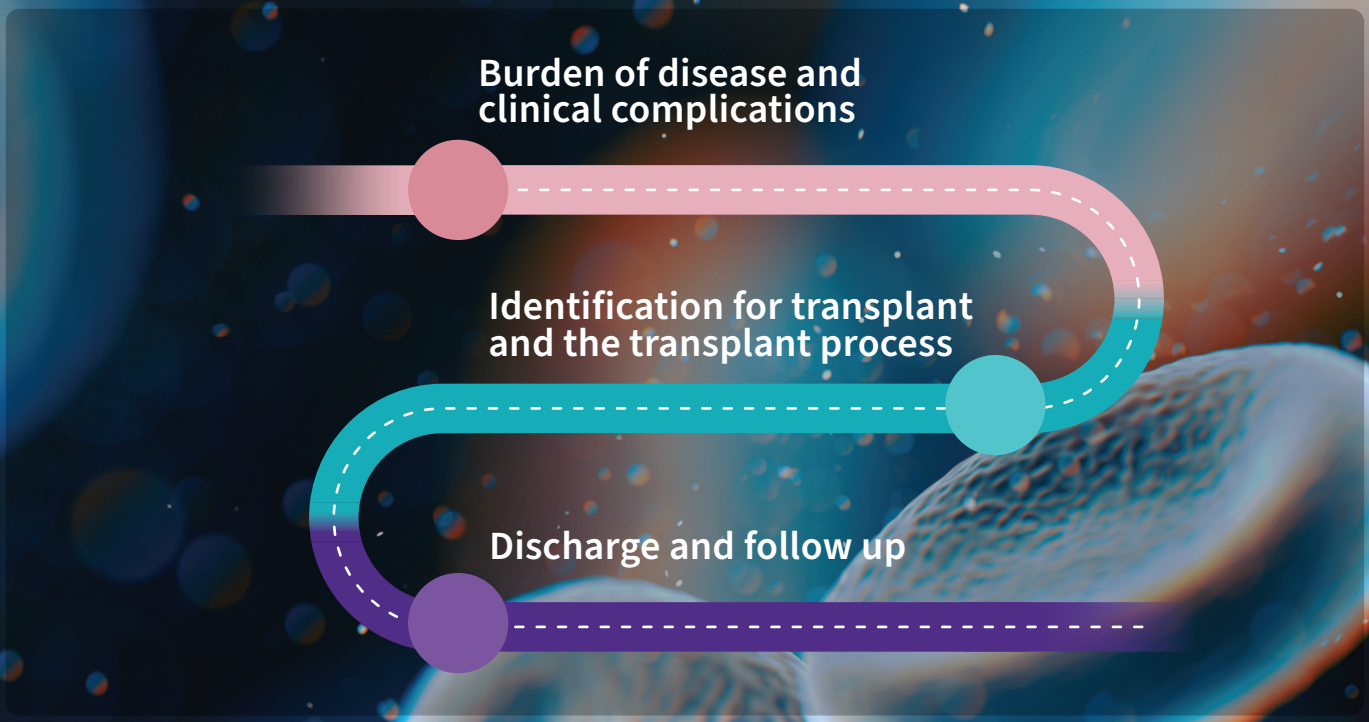
Friday, 28 November 2025

08:00 – 09:30	**Haemoglobinopathies Working Party Annual Meeting Business Meeting** *Open to all.*	
09:30 – 11:00	**Session V: Resource Limited Setting** **Chairs:** *Fabio Gilgio (IT) & Miguel Abboud (LB)*	
09:30 – 09:45	Challenges & priorities of allogeneic transplantation in Tanzania	Stella Malangahe (TZ)
09:45 – 10:00	Challenges & priorities of allogeneic transplantation in Nigeria	Ugonna Fakile (NG)
10:00 – 10:15	Challenges & priorities of allogeneic transplantation in unequal settings	Lawrence Faulkner (IT)
10:15 – 10:30	Does gene therapy have a role in resource limited setting	Miguel Abboud (LB)
10:30 – 11:00	Roundtable discussion: *Is the role for curative therapies in resource limited settings?*	
11:00 – 11:30	**Coffee Break**	
11:30 – 13:00	**Session VI: Alternative Donor Transplantation-Improving Outcomes** **Chairs:** *Monica Bhatia (US)*	
11:30 – 11:55	Cerebrovascular disease: reversibility or stabilisation?	Francoise Bernaudin (FR)
11:55 – 12:15	Utility and outcomes of transplantation of psychometric tests	Becky Armstrong (UK)
12:15 – 12:40	Reversibility of iron damage	Emanuele Angelucci (IT)
12:40 – 13:00	Effect of BMT on sickle cell disease-related organ complications	Erfan Nur (NL)
13:00 – 13:30	**Farewell Lunch**	

GLOBEIN

PURSUING EDUCATIONAL EXCELLENCE IN HAEMOGLOBINOPATHIES

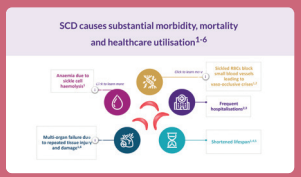
Learning through the patient journey: Haemoglobinopathies in focus



Burden of disease and clinical complications

E-LEARNING

DISEASE PATHOPHYSIOLOGY AND BURDEN OF SICKLE CELL DISEASE



Identification of the transplant and the transplant process

VIDEOS & PODCASTS

HOLISTIC CONSIDERATIONS IN HAEMOGLOBINOPATHIES: THE LIVED PATIENT PERSPECTIVE

Scan the QR code to access the GlobeIN website or visit www.globeinportal.com

The content enclosed is intended only for healthcare professionals interested in haemoglobinopathies.

GlobeIN is an educational programme organised and funded entirely by Vertex Pharmaceuticals (Europe) Limited. All content is developed by the scientific committee and speakers for each meeting, with support from a medical education agency acting as Secretariat. Vertex Pharmaceuticals (Europe) Limited has had an opportunity to review the materials for scientific accuracy and fair balance.

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THE SCIENCE *of* POSSIBILITY

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