



Paediatric Diseases Working Party Educational Meeting on Haemoglobinopathies 2022

Regensburg, Germany
10-12 November 2022

WELCOME

The hemoglobin related genetic disorders, namely sickle cell disease (SCD) and thalassemia (TDT), are by far the most frequent monogenetic disorder worldwide affecting several million worldwide, mostly in low and middle income countries. During the last decades, the overall survival has improved significantly predominantly due to education and supportive measures. Nevertheless, despite optimal care the overall survival has not reached average standards.

Curative options such as stem cell transplantation remained reserved for patients with well-matched siblings in developed countries. Only in the last decade significant advancement in molecular science, advancements in drug development, stem cell therapy and gene manipulation technologies started to allow an opportunity for almost all patients with these diseases to improve their quality of life or achieve even cure.

Due to this highly relevant content, the **PDWP** of the **EBMT** decided to continue the tradition of the '**Regensburg Meetings**' and dedicate another scientific meeting exclusively to this area of research, which will gain increasingly importance in the upcoming years.

During three days, all relevant aspects of SCD and TDT will be presented by international experts from all over the world in a **live** format with **in-person** attendance availability. Each session will offer the opportunity to interact directly with the expert to discuss the presentation and to ask questions from your clinical practice. Participation is limited to encourage lively interaction and discussion among all participants.

Selim Corbacioglu, PDWP Chair
Josu de la Fuente, PDWP Vice Chair
Katharina Kleinschmidt, PDWP Secretary
Hilda Mekelenkamp, NG Paediatric Committee Chair

Scientific Programme Thursday, 10th November 2022

12:00 – 13:00	Registration and Welcome Coffee	
13:00 – 14:30	Session I: The Genomics of Sickle Cell Disease (KNL) Chair: Lakshmanan Krishnamurti	
13:00 – 13:10	Current natural history of haemoglobinopathies and outcomes of transplantation	Josu de la Fuente
13:10 – 13:30	Genetic modifiers of HbF	Swee Lay Thein (Video)
13:30 – 13:50	Long-term outcome in SCD	Lakshmanan Krishnamurti
13:50 – 14:10	Clonal evolution in SCD	Courtney Fitzhugh (Video)
14:10 – 14:30	Q&A	
14:30 – 15:00	Coffee Break	
15:00 – 15:30	Keynote Lecture: Genomic control of haemoglobin switching Chair: Josu de la Fuente	Douglas Higgs
15:30 – 16:55	Session II: Targeted Treatment Options for Hemoglobinopathies Chair: Fabio Ciceri	
15:30 – 15:50	Novel Targeted Therapeutic Options beyond Transfusion and Chelation	Maria Cappellini (Video)
15:50 – 16:35	Novel targeted agents in development for Sickle Cell Disease	Miguel Abboud

Friday, 11th November 2022

08:30 – 10:15	Session III (part 1): Gene Editing, Gene Therapy, Gene Correction Chair: Mathew Porteus	
08:30 – 08:50	Gene Correction: The Gold Standard of Gene Therapy for Sickle Cell Disease?	Mathew Porteus
08:50 – 09:05	Update on Gene Editing for HGB	Josu de la Fuente
09:05 – 09:25	Update Gene Therapy with BBB	Julie Kanter-Washko
09:25 – 09:40	Update on Gene Editing for TDT CrispR	Selim Corbacioglu
09:40 – 10:15	Roundtable discussion	
10:15 – 10:45	Coffee Break	
10:45 – 11:50	Session III (part 2): Gene Editing, Gene Therapy, Gene Correction Chair: Josu de la Fuente	
10:45 – 11:10	Nanotechnology for gene correction	Stavros Loukogeorgakis
11:10 – 11:30	Development of base editing approaches for haemoglobinopathies and characterisation off-target effects	James Davies
11:30 – 11:50	Tiget B-Thal gene therapy trial: 5 years follow-up	Fabio Ciceri
11:50 – 12:50	Industry Symposium by Vertex Roundtable discussion	
12:50 – 13:30	Lunch	

13:30 – 14:50

Session IV (part 1): Alternative Approaches for HSCT in HGB
Chair: Selim Corbacioglu

13:30 – 13:50
13:50 – 14:10
14:10 – 14:30
14:30 – 14:50

Alternative Donor: Haploidentical HSCT in Thalassemia (PTCY)
Curing Adult Sickle Cell Patient: A Realistic Proposition
Alternative Donor HSCT in SCD: PTCY
Q&A

Suradej Hongeng
Erfan Nur
Adetola Kassim (Video)

14:50 – 15:15

Coffee Break

15:15 – 17:30

Session IV (part 2): Alternative Approaches for HSCT in HGB
Chair: Katharina Kleinschmidt

15:15 – 15:35
15:35 – 15:55
15:55 – 16:15
16:15 – 16:35

16:35 – 16:55
16:55 – 17:15
17:15 – 17:30
17:30 – 17:45

Alternative Donor HSCT in SCD: Alpha beta depleted Haplo HSCT
Unrelated BMT in SCD
Cord Blood transplantation in SCD
Pretransplant Immunosuppression enabling transplantation in immunized patients
Non-myeloablative HSCT in SCD in Children
Reduced intensity conditioning in hemoglobinopathies
NIH reduced intensity approach in related haploidentical HSCT with PTCY
Roundtable discussion

Selim Corbacioglu
Shalini Shenoy
Suhag Parikh

Satya Yadav
Greg Guilcher
Akshay Sharma
Matthew Hsieh (Video)

19:30 – 22:00

Networking Dinner

Saturday, 12th November 2022

08:30 – 10:10

Session V (part 1): Global Curative Approaches for HGB
Chair: Shalini Shenoy

08:30 – 08:50
08:50 – 09:10
09:10 – 09:30
09:30 – 09:50

09:50 – 10:10

HSCT in pediatric SCD: Saudi Arabian Experience
PBSC use with PTCY: Indian Experience
Development of a SCD Transplant Program in Subsahara-Africa
The cure of severe hemoglobinopathies as an opportunity to expand global access to bone marrow transplantation
Haploidentical Transplantation with PTCY: Brazil experience within VGC2

Abdullah Aljefri
Gaurav Kharya
Siana Nkya

Lawrence Faulkner
Carmen Bonfim

10:10 – 10:30

Coffee Break

10:30 – 12:00

Session V (part 2): Global Curative Approaches for HGB
Chair: Greg Guilcher

10:30 – 10:50
10:50 – 11:10
11:10 – 11:30
11:30 – 12:00

CIBMTR Data on HSCT in SCD
HSCT in Thalassemia: Turkish Experience
HSCT in patients with Thalassemia: experience in France
Ethical and organizational challenges of SCD research in low and middle-income countries

Kristin Page
Akif Yesilipek
Isabelle Thuret

Miguel Abboud

12:00 – 12:40

Session VI: advances in transplant technology
Chair: Selim Corbacioglu

12:00 – 12:20
12:20 – 12:40

Correction of Vascular Complications
MAS in haploidentical HSCT for HGB

Francoise Bernaudin
Sarita Jaiswal (Video)

12:40 – 14:00

Lunch & Farewell

Nurses Group Programme

Friday, 11th November 2022

08:30 – 08:45	Session I (Part 1): Pre-transplant care Chair: Hilda Mekelenkamp	
08:45 – 10:15	Session I (Part 1): Pre-transplant care Chair: Hilda Mekelenkamp	
08:45 – 09:05	Supportive care Sickle Cell disease	Kelly Hennessy
09:05 – 09:25	Pain Management in Sickle Cell disease	Regina Kulzer
09:25 – 09:45	Supportive care Thalassemia	Lisbeth Andersson Lund
09:45 – 10:05	Indications for HSCT	Lawrence Faulkner
10:05 – 10:15	Q&A – discussion	
10:15 – 10:45	Coffee Break	
10:45 – 12:35	Session I (Part 2): Pre-transplant care Chair: Marjola Gjerjgi	
10:45 – 11:00	HSCT Decision-making for hemoglobinopathy patients	Hilda Mekelenkamp
11:00 – 11:20	Preparation for HSCT	Sandrine Bremathas
11:20 – 11:40	Donor choice – Donor care	Daphna Hutt
11:40 – 11:50	Q&A - Discussion	
11:50 – 12:50	Industry Symposium by Vertex Roundtable discussion (plenary room)	
12:35 – 13:30	Lunch	
13:30 - 14:50	Session II: Research session Chair: Daphna Hutt	
13:30 -13:50	Evidence based nursing: is there evidence to provide the best care?	Ida Ophorst-Bremer
13:50 – 14:10	How to write an abstract	Hilda Mekelenkamp
14:10 – 14:30	How to present your research/project	Valentina Biagioli
14:30 – 14:50	Interactive Session	
14:50 – 15:15	Coffee Break	
15:15 – 17:30	Session III: Clinical transplant care Chair: Ida Ophorst-Bremer	
15:15 – 15:35	Pain management during HSCT	Thaïsa Zendath
15:35 – 15:55	Skin care during HSCT	Judith Timmermans
15:55 – 16:15	Psycho-social development/support	Elisabeth Kuehn-Wolff
16:15 – 16:35	Nursing care in genetherapy	Matteo Amicucci
16:35 – 16:55	Case presentation gene therapy in Thalassemia	Caroline Aumeier & Ann Katrin Lang
16:55 – 17:15	Family perspectives on genetherapy for thalassemia	Marjola Gjerjgi (Video)
17:15 – 17:30	Q&A	



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Networking Dinner

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08:30 – 10:10

Session IV: Post-transplant care

Chair: Christoph Bauer

08:30 – 08:50

The nurses' role in late effects

08:50 – 09:10

Transition to adult care

09:10 – 09:50

Communication challenges in pediatric nursing (debate)

Julia Ruiz (Video)

Trude Minee

Marjola Gjergji

Hilda Mekelenkamp

Eugenia Trigos

09:50 – 10:10

Q&A

10:10 – 10:40

Coffee Break

10:40 – 12:40

Session V: Nursing Challenges

Chair: Sandrine Bremathas

10:40 – 11:00

Outreach program

11:00 – 11:20

Quality management in nursing

11:20 – 11:40

Differences in nurses' training in Europe

Eugenia Trigos

Eugenia Trigos

Christoph Bauer

& Tanja Kremer

11:40 – 12:20

Round table session on ethical issues

12:40 – 14:00

Lunch & Farewell



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