



Paediatric Diseases Working Party Educational Meeting on Haemoglobinopathies 2022

Regensburg, Germany
10-12 November 2022

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 treatment options: Crizanlizumab & Voxelotor	Kenneth Ataga
16:35 – 16:55	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 – 11:30	Nanotechnology for gene correction	Stavros Loukogeorgakis
11:30 – 11:50	Development of base editing approaches for haemoglobinopathies and characterisation off-target effects	James Davies
11:50 – 12:50	Industry Symposium by Vertex Roundtable discussion	
12:50 – 13:30	Lunch	



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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 – 17:30

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
Roundtable discussion

Selim Corbacioglu
Shalini Shenoy
Suhag Parikh

Satya Yadav
Greg Guilcher
Akshay Sharma

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



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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
09:05 – 09:25
09:25 – 09:45
09:45 – 10:05
10:05 – 10:15

Supportive care Sickle Cell disease
Pain Management in Sickle Cell disease
Supportive care Thalassemia
Indications for HSCT
Q&A – discussion

Kelly Hennessy
Regina Kulzer
Lisbeth Andersson Lund
Lawrence Faulkner

10:15 – 10:45

Coffee Break

10:45 – 12:35

Session I (Part 2): Pre-transplant care
Chair: Marjola Gjerjgi

10:45 – 11:00
11:00 – 11:20
11:20 – 11:40
11:40 – 11:50

HSCT Decision-making for hemoglobinopathy patients
Preparation for HSCT
Donor choice – Donor care
Q&A - Discussion

Hilda Mekelenkamp
Sandrine Bremathas
Daphna Hutt

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

Evidence based nursing: is there evidence to provide the best care?
How to write an abstract
How to present your research/project
Interactive Session

Ida Ophorst-Bremer
Hilda Mekelenkamp
Valentina Biagioli

14:50 – 15:15

Coffee Break

15:15 – 17:30

Session III: Clinical transplant care
Chair: Ida Ophorst-Bremer

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

Pain management during HSCT
Skin care during HSCT
Psycho-social development/support
Nursing care in genetherapy
Case presentation gene therapy in Thalassemia
Family perspectives on genetherapy for thalassemia
Q&A

Thaisa Zendath
Judith Timmermans
Elisabeth Kuehn-Wolff
Matteo Amicucci
Caroline Aumeier & Ann Katrin Lang
Marjola Gjerjgi (Video)

19:30 – 22:00

Networking Dinner



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08:30 – 10:10

Session IV: Post-transplant care
Chair: Christoph Bauer

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

The nurses' role in late effects
Transition to adult care
Communication challenges in pediatric nursing (debate)

Julia Ruiz (Video)
Trude Minee
Marjola Gjergji
Hilda Mekelenkamp
Eugenia Trigoso

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
11:00 – 11:20
11:20 – 11:40

Outreach program
Quality management in nursing
Differences in nurses' training in Europe

Eugenia Trigoso
Eugenia Trigoso
Christoph Bauer
& Tanja Kremer

11:40 – 12:20

Round table session on ethical issues

12:40 – 14:00

Lunch & Farewell