



# Paediatric Diseases Working Party Educational Meeting on Haemoglobinopathies 2022

Regensburg, Germany  
10-12 November 2022

## Scientific Programme Thursday, 10<sup>th</sup> November 2022

<b>12:00 – 13:00</b>	<b>Registration and Welcome Coffee</b>	
<b>13:00 – 15:00</b>	<b>Session I: The Genomics of Sickle Cell Disease (KNL)</b>	
	Genetic modifiers of HbF Clonal evolution in SCD Long-term outcome in SCD HbB beta Thalassemia: An increased risk of endothelial complications? Natural history of both SCD and Thalassaemia	Swee Lay Thein Courtney Fitzhugh To be announced To be announced To be announced
<b>15:00 – 15:30</b>	<b>Coffee Break</b>	
<b>15:30 – 16:10</b>	<b>Keynote Lecture: Genomic control of hemoglobin switching</b>	<b>Douglas Higgs</b>
<b>16:10 – 17:30</b>	<b>Session II: Targeted Treatment Options for Hemoglobinopathies</b>	
	Novel Targeted Therapeutic Options beyond Transfusion and Chelation Novel targeted treatment options: Crizanlizumab & Voxelotor	To be announced To be announced



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## Friday, 11<sup>th</sup> November 2022

<b>08:30 – 10:10</b>	<b>Session III (part 1): Gene Editing, Gene Therapy, Gene Correction</b>  Gene Correction: The Gold Standard of Gene Therapy for Sickle Cell Disease? Update on Gene Editing for HGB Update on Gene Editing for SCD CrispR Update Gene Therapy with BBB	Mathew Porteus To be announced Josu de la Fuente Julie Kanter-Washko
<b>10:10 – 10:40</b>	<b>Coffee Break</b>	
<b>10:40 – 12:40</b>	<b>Session III (part 2): Gene Editing, Gene Therapy, Gene Correction</b>  Nanotechnology for gene correction In vivo correction approaches In-utero approaches for transplantation and gene correction Update on Gene Editing for Thal CrispR Tiget B-Thal gene therapy trial: 5 years follow-up	To be announced James Davies To be announced Selim Corbacioglu Fabio Ciceri
<b>12:40 – 14:00</b>	<b>Lunch Symposium by Vertex   Roundtable discussion</b>	
<b>14:00 – 15:20</b>	<b>Session IV (part 1): Alternative Approaches for HSCT in HGB</b>  Alternative Donor: Haploidentical HSCT in Thalassemia (PTCY) Haplo with PTCY for Thalassemia: US and UK Experience Curing Adult Sickle Cell Patient: A Realistic Proposition Alternative Donor HSCT in SCD: PTCY	Suradej Hongeng Kenneth Cooke Erfan Nur Adetola Kassim
<b>15:20 – 16:20</b>	<b>Coffee Break + Poster Walk</b>	
<b>16:20 – 18:40</b>	<b>Session IV (part 2): Alternative Approaches for HSCT in HGB</b>  Alternative Donor HSCT in SCD: T-Haplo NIH reduced intensity approach in related haploidentical HSCT with PTCY Unrelated BMT in SCD Cord Blood transplantation in SCD Haplo HSCT Pretransplant Immunosuppression enabling transplantation in immunized patients Non-myeloablative HSCT in SCD in Children Reduced intensity conditioning in hemoglobinopathies	Selim Corbacioglu Matthew Hsieh Shalini Shenoy Suhag Parikh To be announced  Satya Yadav Greg Guilcher Akshay Sharma
<b>20:00 – 23:00</b>	<b>Networking Dinner</b>	



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## Saturday, 12<sup>th</sup> November 2022

08:30 – 10:10

### Session V (part 1): Global Curative Approaches for HGB

PBSC use with PTCY: Indian Experience  
Development of a SCD Transplant Program in Sub-Sahara-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  
HSCT in pediatric SCD: Saudi Arabian Experience

Gaurav Kharya  
Siana Nyka

Lawrence Faulkner

Carmen Bonfim  
Abdullah Aljefri

10:10 – 10:40

### Coffee Break

10:40 – 11:40

### Session V (part 2): Global Curative Approaches for HGB

CIBMTR Data on HSCT in SCD  
HSCT in Thalassemia: Turkish Experience  
Thalassemia Treatment Experience in France

Kristin Page  
Akif Yesilipek  
To be announced

11:40 – 12:20

### Session VI: Ethical and Legal Aspects of Gene Correction

Ethical Aspects of Embryonic Gene Editing  
Legal Aspects of Embryonic Gene Editing

To be announced  
To be announced

12:20 – 13:40

### Session VII: advances in transplant technology

Non-Chemotherapy based conditioning regimens  
Correction of Vascular Complications  
MAS in haploidentical HSCT for HGB  
PK and Antigen target differences of variant ATGs and (potential) implications for HCT in Hemoglobinopathies

Fabio Ciceri  
Francoise Bernaudin  
Sarita Jaiswal

Jaap Jan Boelens

13:40 – 15:00

Lunch & Farewell



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## Nurses Group Programme Friday, 11<sup>th</sup> November 2022

<b>08:50 – 10:10</b>	<b>Session I (Part 1): Pre-transplant care</b> <b>Chair:</b> Hilda Mekelenkamp	
	Supportive care Sickle Cell disease Pain Management in Sickle Cell disease	Kelly Hennessy Katharina Geiling & Regina Kulzer Lisbeth Andersson Lund
	Supportive care Thalassemia Q&A – discussion	
<b>10:10 – 10:40</b>	<b>Coffee Break</b>	
<b>10:40 – 12:40</b>	<b>Session I (Part 2): Pre-transplant care</b> <b>Chair:</b> Hilda Mekelenkamp	
	Indications for HSCT HSCT Decision-making for hemoglobinopathy patients Preparation for HSCT Donor choice – Donor care Q&A - Discussion	Lawrence Faulkner Hilda Mekelenkamp Sandrine Bremathas Daphna Hutt
<b>12:40 – 14:00</b>	<b>Lunch Symposium</b>	
<b>14:00 - 15:20</b>	<b>Session II: Research session</b> <b>Chair:</b> Marjola Gjergji	
	Evidence based nursing/ From a clinical problem to a research proposal How to write an abstract How to present your research/project Interactive Session	Ida Ophorst-Bremer Hilda Mekelenkamp Valentina Biagioli
<b>15:20 – 16:20</b>	<b>Coffee Break</b>	
<b>16:20 - 18:40</b>	<b>Session III: Clinical transplant care</b> <b>Chair:</b> Daphna Hutt	
	Pain management during HSCT Skin care during HSCT Psycho-social development/support Communication challenges in pediatric nursing (discussion or talk) Nursing care in genetherapy Case presentation gene therapy in Thalassemia	Thaisa Zendath Judith Timmermans Elisabeth Kuehn-Wolff Marjola Gjergji Matteo Amicucci Caroline Aumeier & Ann Katrin Lang Marjola Gjergji (Video)
	Family perspectives on genetherapy for thalassemia Q&A	
<b>20:00 – 23:00</b>	<b>Networking Dinner</b>	



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## Saturday, 12<sup>th</sup> November 2022

<b>08:30 – 10:40</b>	<b>Session IV: Post-transplant care</b> <b>Chair:</b> Ida Ophorst-Bremer  The nurses' role in late effects Transition to adult care Communication challenges in pediatric nursing (debate) Q&A	Julia Ruiz Trijntje Guijt Marjola Gjergji
<b>10:40 – 11:00</b>	<b>Coffee Break</b>	
<b>11:00 – 12:40</b>	<b>Session V: Nursing Challenges</b> <b>Chair:</b> Christoph Bauer  Outreach program Quality management in nursing Differences in nurses' training in Europe  Round table session on ethical issues	Eugenia Trigos Eugenia Trigos Christoph Bauer & Tanja Kremer
<b>12:40 – 14:00</b>	<b>Lunch &amp; Farewell</b>	