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

VERTEX

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VERTEX ORGANISED AND SPONSORED SATELLITE SYMPOSIUM AT PDWP 2022

A roundtable discussion: Considering treatment decisions for patients with haemoglobinopathies

Friday 11 November 2022 | 11:50—12:50 CET

Panorama 2, Parkside Events, Prüfeninger Str. 20, 93049 Regensburg, Germany

Vertex invites you to join Prof. Roland Meisel, Prof. Selim Corbacioglu and a faculty of leading European experts as they discuss treatment decisions for patients with haemoglobinopathies.

TOPIC	SPEAKER
Welcome and introduction	Prof. Roland Meisel & Prof. Selim Corbacioglu (Chairs)
Treatment perspectives in β-Thalassaemia: *a case study in TDT*	Panel: Josu de la Fuente, Erfan Nur & Holger Cario
Treatment perspectives in Sickle Cell Disease: *a case study in SCD*	
Chair's close of symposium	Prof. Roland Meisel & Prof. Selim Corbacioglu

Company overview

Vertex is a global biotechnology company that invests in scientific innovation with the aim to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a pipeline of investigational small molecule, cell and genetic therapies aimed at other serious diseases where it has deep insight into causal human biology, including sickle cell disease, β-thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 12 consecutive years on Science magazine's Top Employers list and one of the 2022 Seramount 100 Best Companies.

This symposium is for healthcare professionals only, specifically for those involved in the management of SCD and TDT. This will be an interactive meeting, with dedicated time for scientific exchange. It is encouraged to fully partake in the discussion and Q&A throughout the symposium to ensure lively debate. Please note the event will be live and a recording will not be available.

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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**	

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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	Alternative Donor: Haploidentical HSCT in Thalassemia (PTCY)	Suradej Hongeng
13:50 – 14:10	Curing Adult Sickle Cell Patient: A Realistic Proposition	Erfan Nur
14:10 – 14:30	Alternative Donor HSCT in SCD: PTCY	Adetola Kassim (Video)
14:30 – 14:50	Q&A	
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	Alternative Donor HSCT in SCD: Alpha beta depleted Haplo HSCT	Selim Corbacioglu
15:35 – 15:55	Unrelated BMT in SCD	Shalini Shenoy
15:55 – 16:15	Cord Blood transplantation in SCD	Suhag Parikh
16:15 – 16:35	Pretransplant Immunosuppression enabling transplantation in immunized patients	Satya Yadav
16:35 – 16:55	Non-myeloablative HSCT in SCD in Children	Greg Guilcher
16:55 – 17:15	Reduced intensity conditioning in hemoglobinopathies	Akshay Sharma
17:15 – 17:30	NIH reduced intensity approach in related haploidentical HSCT with PTCY	Matthew Hsieh (Video)
17:30 – 17:45	Roundtable discussion	
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	HSCT in pediatric SCD: Saudi Arabian Experience	Abdullah Aljefri
08:50 – 09:10	PBSC use with PTCY: Indian Experience	Gaurav Kharya
09:10 – 09:30	Development of a SCD Transplant Program in Subsahara-Africa	Siana Nkya
09:30 – 09:50	The cure of severe hemoglobinopathies as an opportunity to expand global access to bone marrow transplantation	Lawrence Faulkner
09:50 – 10:10	Haploidentical Transplantation with PTCY: Brazil experience within VGC2	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	CIBMTR Data on HSCT in SCD	Kristin Page
10:50 – 11:10	HSCT in Thalassemia: Turkish Experience	Akif Yesilipek
11:10 – 11:30	HSCT in patients with Thalassemia: experience in France	Isabelle Thuret
11:30 – 12:00	Ethical and organizational challenges of SCD research in low and middle-income countries	Miguel Abboud
12:00 – 12:40	**Session VI: advances in transplant technology** **Chair: Selim Corbacioglu**	
12:00 – 12:20	Correction of Vascular Complications	Francoise Bernaudin
12:20 – 12:40	MAS in haploidentical HSCT for HGB	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	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	Thaisa 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	
19:30 – 22:00	**Networking Dinner**	

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Saturday, 12th November 2022

08:30 – 10:10	**Session IV: Post-transplant care** **Chair: Christoph Bauer**	
08:30 – 08:50	The nurses' role in late effects	Julia Ruiz (Video)
08:50 – 09:10	Transition to adult care	Trude Minee
09:10 – 09:50	Communication challenges in pediatric nursing (debate)	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 Brematthas**	
10:40 – 11:00	Outreach program	Eugenia Trigoso
11:00 – 11:20	Quality management in nursing	Eugenia Trigoso
11:20 – 11:40	Differences in nurses' training in Europe	Christoph Bauer & Tanja Kremer
11:40 – 12:20	Round table session on ethical issues	
12:40 – 14:00	**Lunch & Farewell**	

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