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 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.

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<th>TOPIC</th>
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<td>Welcome and introduction</td>
<td>Prof. Roland Meisel &amp; Prof. Selim Corbacioglu (Chairs)</td>
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<tr>
<td>Treatment perspectives in β-Thalassaemia: a case study in TDT</td>
<td>Panel: Josu de la Fuente, Erfan Nur &amp; Holger Cario</td>
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<tr>
<td>Treatment perspectives in Sickle Cell Disease: a case study in SCD</td>
<td>Prof. Roland Meisel &amp; Prof. Selim Corbacioglu</td>
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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.

V1.0 INT-70-2200180 | 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

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

16:35 – 17:00
Q&A

17:00 – 18:00
Coffee Break

18:00 – 19:00
Dinner

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

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<td>Curing Adult Sickle Cell Patient: A Realistic Proposition</td>
<td>Erfan Nur (Video)</td>
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<td>14:10 – 14:30</td>
<td>Alternative Donor HSCT in SCD: PTCY</td>
<td>Adetola Kassim (Video)</td>
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<td>14:30 – 14:50</td>
<td>Q&amp;A</td>
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### Session IV (part 2): Alternative Approaches for HSCT in HGB
**Chair:** Katharina Kleinschmidt

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<td>Alternative Donor HSCT in SCD: Alpha beta depleted Haplo HSCT</td>
<td>Selim Corbacioglu</td>
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<td>15:35 – 15:55</td>
<td>Unrelated BMT in SCD</td>
<td>Shalini Shenoy</td>
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<td>15:55 – 16:15</td>
<td>Cord Blood transplantation in SCD</td>
<td>Suhag Parikh</td>
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<td>16:15 – 16:35</td>
<td>Pretransplant Immunosuppression enabling transplantation in immunized patients</td>
<td>Satya Yadav</td>
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<td>16:35 – 16:55</td>
<td>Non-myeloablative HSCT in SCD in Children</td>
<td>Greg Guilcher</td>
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<td>16:55 – 17:15</td>
<td>Reduced intensity conditioning in hemoglobinopathies</td>
<td>Akshay Sharma</td>
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<td>17:15 – 17:30</td>
<td>NIH reduced intensity approach in related haploidentical HSCT with PTCY</td>
<td>Emily Limerick (Video)</td>
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<td>17:30 – 17:45</td>
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<td>19:30 – 22:00</td>
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**Saturday, 12th November 2022**

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

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<td>HSCT in pediatric SCD: Saudi Arabian Experience</td>
<td>Abdullah Aljefri</td>
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<td>08:50 – 09:10</td>
<td>PBSC use with PTCY: Indian Experience</td>
<td>Gaurav Kharya</td>
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<td>09:10 – 09:30</td>
<td>Development of a SCD Transplant Program in Subsahara-Africa</td>
<td>Siana Nkya</td>
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<td>09:30 – 09:50</td>
<td>The cure of severe hemoglobinopathies as an opportunity to expand global access to bone marrow transplantation</td>
<td>Lawrence Faulkner</td>
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<td>09:50 – 10:10</td>
<td>Haploidentical Transplantation with PTCY: Brazil experience within VGC2</td>
<td>Carmen Bonfim</td>
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### Session V (part 2): Global Curative Approaches for HGB
**Chair:** Greg Guilcher

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<td>CIBMTR Data on HSCT in SCD</td>
<td>Kristin Page</td>
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<td>HSCT in Thalasemia: Turkish Experience</td>
<td>Akif Yeslipek</td>
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<td>HSCT in patients with Thalasemia: experience in France</td>
<td>Isabelle Thuret</td>
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<td>Ethical and organizational challenges of SCD research in low and middle-income countries</td>
<td>Miguel Abboud</td>
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<td>12:00 – 12:40</td>
<td>Session VI: advances in transplant technology</td>
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<td>Correction of Vascular Complications</td>
<td>Francoise Bernaudin</td>
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<td>12:20 – 12:40</td>
<td>MAS in haploidentical HSCT for HGB</td>
<td>Sarita Jaiswal (Video)</td>
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<td>12:40 – 14:00</td>
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Paediatric Diseases Working Party Educational Meeting on Haemoglobinopathies 2022

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  
Chair: Hilda Mekelenkamp
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
**Saturday, 12th November 2022**

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<td>The nurses’ role in late effects</td>
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<td>Transition to adult care</td>
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<td>09:10 – 09:50</td>
<td>Communication challenges in pediatric nursing (debate)</td>
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<td><strong>Chair:</strong> Sandrine Bremathas</td>
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<td>Outreach program</td>
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<td>11:00 – 11:20</td>
<td>Quality management in nursing</td>
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<td>11:20 – 11:40</td>
<td>Differences in nurses’ training in Europe</td>
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<td>11:40 – 12:20</td>
<td>Round table session on ethical issues</td>
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<td>12:40 – 14:00</td>
<td><strong>Lunch &amp; Farewell</strong></td>
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**Notes:**
- Julia Ruiz (Video)
- Trude Minee
- Marjola Gjergji
- Hilda Mekelenkamp
- Eugenia Trigoso
- Eugenia Trigoso
- Christoph Bauer
- Tanja Kremer
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