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General information

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Registration
Online registration under:
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Congress Fee

Both days	100 €
Conference Dinner	50 €

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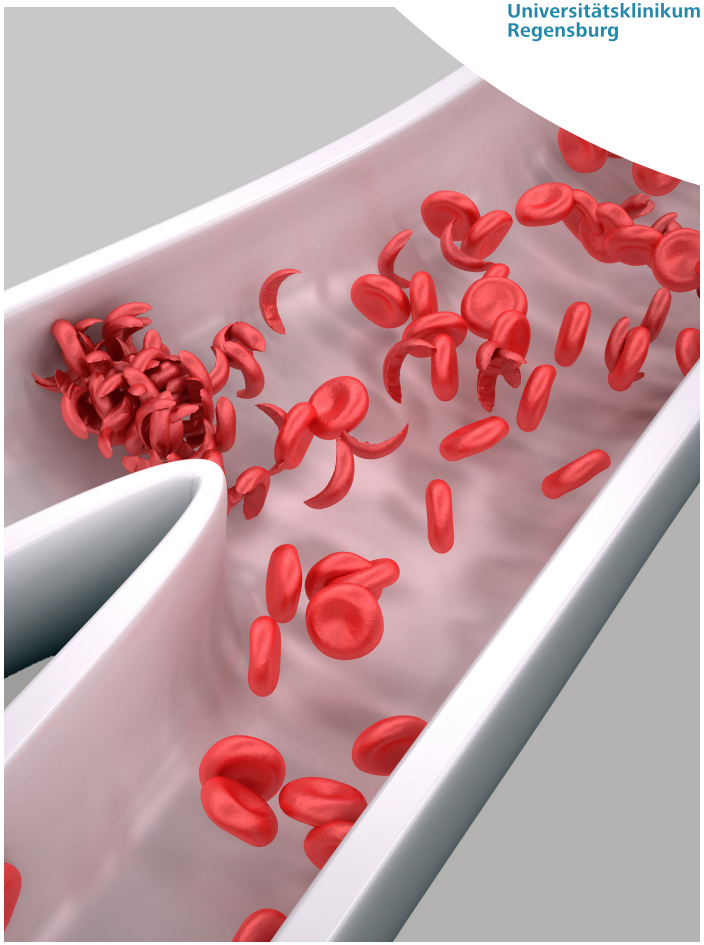

Department of Pediatric Hematology, Oncology and Stem Cell Transplantation

4. INTERNATIONAL MEETING ON SICKLE CELL DISEASE - CURE OR PROCRASTINATION?

Auktionshaus Keup, Haidplatz 7, 93047 Regensburg, Germany

29 - 30 June 2017

25 JAHRE UKR Stationäre Universitätsmedizin

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Dear colleagues and friends,

I would like to invite you to the 4th international meeting on sickle cell disease.

Sickle cell disease is one of the most prevalent, debilitating, enigmatic diseases worldwide. In developing countries, the mortality in infancy and childhood remains persistently high. In developed countries, despite optimal medical care, a prolonged average overall survival can be achieved but the quality of life for most patients remains dismal. Sickle cell disease is, however, curable.

Stem cell transplantation is currently the only curative option, but is offered to only a few patients. The reasons are lack of knowledge and donor availability according to established criteria. Haploidentical stem cell transplantation has advanced enormously within the last decade, making a donor available for almost every patient. Safety and wide applicability has rendered this procedure increasingly a standard of care for many diseases. Its broader application in sickle cell disease must therefore be considered for obvious reasons.

Gene therapy, on the other hand is an intriguing option for a monogenetic disease but has not yet reached sufficient safety and stability to hit 'mainstream'. Additionally, many novel agents are in development to manipulate hemoglobin expression and polymerization and promise a simple cure. The long-term efficacy of chronic medication on systemic organ damage inflicted by sickle cell disease is unsure.

In the end the question is cure or procrastination. Both options bare risks and promises.

This meeting will provide us an overview of a variety of sickle related topics, including current treatment strategies and an outlook on future options to tackle this highly debilitating disease.

It is my pleasure to welcome you to Regensburg, a medieval city of a notable history, spanning two millennia, from ancient Roman history to today, and experience Bavarian hospitality in a UNESCO world heritage site.

Selim Corbacioglu
Professor of Pediatrics
KUNO Children's Hospital, University Hospital Regensburg

Thursday, 29.06.2017

11:30 **Lunch**

12:40 **Welcome Reception**
Selim Corbacioglu, Regensburg & Dietger Niederwieser, Leipzig on behalf of the WBMT

Session 1

13:00 **What is the optimal medical care for patients with SCD? Do your ‚homework' prior to transplantation.**
Roswitha Dickerhoff, Düsseldorf

13:20 **Impact of iron chelation therapy in stem cell transplantation.**
Andrea Jarisch, Frankfurt

13:40 **Transition and adult SCD patients: A different challenge.**
Anette Hoferer, Stuttgart

14:00 **Non-transplant alternatives: new and developing therapies for sickle cell disease.**
Miguel Abboud, Beirut

14:20 **What's new in the detection and the management of cerebral vasculopathy in sickle cell anemia?**
Francoise Bernaudin, Créteil

14:40 **How to avoid alloimmunization in SCD?**
Volker Witt, Vienna

15:00 **Sickle cell disease: a risk factor for post-transplant endothelial complications?**
Enric Carreras, Barcelona

15:20 **Coffee Break**

Session 2

15:50 **Haploidentical stem cell transplantation – State of the Art.**
Rupert Handgretinger, Tübingen

16:10 **Overview of the current indication and results of hematopoietic stem cell transplantation in patients with with sickle cell disease.**
Eliane Gluckman, Paris

16:30 **What are the options for bone marrow transplant for sickle cell disease in Africa: Experience from Tanzania?**
Julie Makani, Tanzania

16:50 **BMT for hemoglobinopaties in lower income settings: opportunities and challenges.**
Lawrence Faulkner, Bangalore

17:10 **Haploidentical BMT to expand donor availability: The post-transplantation cyclophosphamide experience.**
Josu de la Fuente, London

17:30 **Haploidentical SCT in SCD: the CD3/CD19 experience.**
Selim Corbacioglu, Regensburg

17:50 **How to bridge the gap in haploidentical SCT? Means of accelerated immune reconstitution.**
Stephan Mielke, Stockholm

18:10 **Fertility preservation during Stem Cell Transplantation in Semi-elective/non-malignant Diseases for all ages? State of the art and beyond.**
Jean-Hugues Dalle, Paris

18:30 **Hepatic VOD despite targeted PK-based Busulfan administration in high-risk patients.**
Tayfun Güngör, Zurich

20:00 **Conference Dinner**

Friday, 30.06.2017

Session 3

09:00 **Clinical outcomes of gene therapy with BB305 lentiviral vector for SCD and β-Thalassemia.**
Jean-Antoine Ribeil, Paris

09:20 **Targeting BCL11A using shRNA embedded miRNAs (shRNA^{miRs}) in Lentiviral vectors for treatment of β-hemoglobinopathies.**
David Williams, Boston

09:40 **Autologous HSC-based gene therapy in beta-thalassemia and SCD: Current status and perspectives.**
Fabio Ciceri, Milan

10:10 **Coffee Break**

10:30 – 12:00 **Discussion and Conclusions**

- What is the role of modern conventional treatment options?
- Is SCT the best curative option for SCD patients?
- What can be optimized prior to HSCT to reduce TRM?
- How much fertility preservation?
- When is the best time point for HSCT in SCD patients? Should the current indications for HSCT in SCD patients be maintained?
- Is the current donor selection process still state of the art?
- What should be considered for conditioning?
- How to manage post-SCT immune suppression for SCD patients?
- Gene therapy: What to expect when we are expecting?
- Et al.

12:00 **Lunch**