EBMT
European Society
for Blood and Marrow Transplantation

Pediatric Disease Working Party Educational Course on. "Sickle Cell Disease" 2019, Regensburg

(16.5.- 17. 5.2019)

Thursday, 16.05.19

11:30 – 12:30 Lunch & Registration

Thursday, 16.05.19 Session I: 13:00 – 14:40

Session Chairs: Krishnamurti Lakshmanan
Josu de la Fuente

12:45 – 13:00	**Welcome Reception**
13:00 – 13:20	**Miguel Abboud** An update on conventional therapy
13:20 – 13:40	**Paul Telfer** Medical treatments available for non-cerebrovascular complications
13:40 – 14:00	**Julie Makani** Approaches in developing countries
14:00 – 14:20	**Mahmoud Aljurf** HSCT for Patients with Sickle Cell Disease in the Eastern Mediterranean
14:20 – 14:40	**Lawrence Faulkner** How to setup a successful transplant program for hemoglobinopathies in developing countries: The Cure2Children approach

14:40 – 15:10 Coffee Break

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Thursday, 16.05.19 Session II: 15:10 – 18:10

Session Chairs: **Miguel Abboud**
Lawrence Faulkner

15:10 – 15:30	**Krishnamurti Lakshmanan** Should young children without severe disease with a matched sibling be offered BMT?
15:30 – 15:50	**Francoise Bernaudin** What's the place of HSCT in the management of cerebral vasculopathy in children with sickle cell anemia?
15:50 – 16:10	**Josu de la Fuente** Should children/young adult with non-severe disease be offered an alternative donor BMT?
16:10 – 16:30	**Pietro Sodani** Is a MUD HSCT still part of the algorithm for transplantation of SCD patients?
16:30 – 16:50	**Jean-Hugues Dalle** Hematologists and transplant physicians: Where do we meet?
16:50 – 18:10	**Round Table Discussion** • Can optimal conventional care provide a long-term cure? • To transplant or not to transplant: What are the minimal SCD related complications to offer a HSCT in 2019? • Would we still make differences between donor choice? • Is there an optimal conditioning or how much conditioning do we need? • Et al

20:00 - 23:00 **Dinner**

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Friday, 17.05.19 Session III: 08:30 – 10:10

Session Chairs: John Tisdale
Fabio Ciceri

08:30 – 08:50	**Eliane Gluckman** Alternative donor hematopoietic stem cell transplantation for sickle cell disease in Europe.
08:50 – 09:10	**Adetola Kassim** Alternative Donor: Post-Transplant Cyclophosphamide
09:10 – 09:30	**Selim Corbacioglu** Alternative Donor: aß/CD3 T-cell depleted Haplo HSCT
09:30 – 09:50	**Arjan Lankester** What is the impact of ATG Pharmakokinetik on haploidentical HSCT in SCD?
09:50 – 10:10	**Enrique Carreras/Marta Palomo** Is sickle cell disease related NT, a systemic endotheliopathy?
10:10 – 10:20	**Adetola Kassim** Oxygen extraction in SCD?

10:20 – 10:40 Coffee Break

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Friday, 17.05.19 Session IV: 10:40 – 13:30

Session Chairs: **Mahmoud Aljurf**
Julie Makani

10:40 – 11:00	**Marina Cavazzana** Gene Therapy for S/S, where do we stand?
11:00 – 11:20	**John Tisdale** Genetic strategies for the cure of SCD
11:20 – 11:40	**David Williams** Gene Therapy: BCL11A as a Therapeutic Target in Gene Therapy for Sickle Cell Disease
11:40 – 12:00	**Fabio Ciceri** Gene Therapy: Hematopoietic stem cell lentiviral gene therapy for adult and pediatric patients affected by hemoglobinopathies: the Milano experience
12:00 – 12:20	**TBC** Gene Therapy: CrispRCas based Gene Therapy for SCD
12:20 – 13:30	**Round Table Discussion** • Pro's and Con's: gene therapy vs. HSCT? • Is there an upper or lower age limit for gene therapy or HSCT? • What are the pro's and con's of the different gene therapeutic approaches? • Et al