

Paediatric Diseases Working Party

Wednesday, 8 July 2020

16:00	OPENING	N. KROEGER (DE) R. PEFFAULT DE LATOUR (FR) A. LANKESTER(NL) S. CORBACIOGLU (DE) H. MEKELENKAMP (NL)
16:05	KEYNOTE IRON METABOLISM	Discussion, Q&A J. PORTER(UK)
16:35	THALASSEMIA	Discussion, Q&A A. PIGA(IT) S.MARKTEL(IT) M.ZECCA (IT) F. LOCATELLI (IT) M. WALTERS(US)
17:45	Break Visit Virtual Exhibition	
18:05	SICKLE CELL DISEASE: EXPERIMENTAL AND ALTERNATIVE TREATMENTS	Discussion, Q&A J. DE LA FUENTE (UK) S. CORBACIOGLU(DE)
18:35	SICKLE CELL DISEASE: CURRENT STANDARD OF CARE	Discussion, Q&A B. ARMSTRONG(UK)

18:55 **END OF SESSIONS**

Chairs:

J. de la Fuente (UK) & S. Corbacioglu(DE)



virtual

Midterm Focus Meeting on Non-Malignant Diseases

8-10 July 2020

Paediatric Diseases Working Party

Lectures On-Demand

KEYNOTE LECTURE

Iron metabolism | J. PORTER | United kingdom

THALASSEMIA

Luspertacept | A. PIGA | Italy

MUD vs Haplo | S. MARKTEL | Italy

Gene therapy thalassemia | F. LOCATELLI | Italy

SICKLE CELL DISEASE: EXPERIMENTAL AND ALTERNATIVE TREATMENTS

MUD HSCT | M. ZECCA | Italy

Haplo HSCT | J. DE LA FUENTE | United Kingdom

Gene editing/gene therapy/gene editing 2.0 | M. WALTERS | United States

SICKLE CELL DISEASE: CURRENT STANDARD OF CARE

Psychological aspects of SCD during HSCT | B. ARMSTRONG | United Kingdom

Inborn Errors Working Party

Thursday, 9 July 2020

16:00	NON-SCID PRIMARY IMMUNODEFICIENCIES Chair: D. Moshous(FR)& M. Albert (DE)	Discussion, Q&A E. MORRIS(UK) T. GUNGOR (CH) D. MOSHOUS(FR) A. GENNERY (UK)
16:50	Break Visit Virtual Exhibition	
17:00	IMMUNE DYSREGULATION Chairs: A. Lankester (NL)& E. Gambineri (IT)	Discussion, Q&A E. GAMBINERI(IT) J. LEIDING(US) I. ANDRE(FR)
17:50	Break Visit Virtual Exhibition	
18:00	INHERITED METABOLIC DISEASES Chairs: ME. Bernardo(IT) & R. Wynn(UK)	Discussion, Q&A R. WYNN(UK) J. J. BOELENS(US) J-S. KUEHL (DE) A. GHOSH(UK) ME. BERNARDO(IT)
18:50	END OF SESSIONS	



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NON-SCID PRIMARY IMMUNODEFICIENCIES

Role of HSCT in AYA-PID population | E. MORRIS | United Kingdom

Indication and outcome of stem cell therapy in CGD | T. GUNGOR | Switzerland

Antibody-based treatment in HLH: cure or bridge to HSCT | D. MOSHOUS | France

Alternative conditioning in special cases | A. GENNERY | United Kingdom

IMMUNE DYSREGULATION

PIRDs: diseases with many faces (talk on basics of disease) | S. HAMBLETON | United Kingdom

Tregopathies: HSCT Indications and outcomes | E. GAMBINERI | Italy

Role of biologicals in management of PIRDs (bridge to HSCT) | J. LEIDING | United States

Gene therapy in Tregopathies | I. ANDRE | France

INHERITED METABOLIC DISEASES

Lysosomal Storage Disease, Current Paediatric Perspectives | R. WYNN | UK

Leukodystrophies and HSCT | J.J. BOELEN | United States

IEM in Adults | J-S. KUEHL | Germany

Complementary and competing therapies in inherited metabolic diseases | A. GHOSH |
United Kingdom

Stem Cell GT in IEM | ME. BERNARDO | Italy

Severe Aplastic Anaemia Working Party

Friday, 10 July 2020

16:00	AA DIAGNOSIS ALGORITHM AND DBA Chairs: R. Peffault de Latour(FR) & T. Leblanc(FR)	Discussion, Q&A R. PEFFAULT DE LATOUR(FR) T. LEBLANC (FR) J-H. DALLE(FR)
16:45	Break Visit Virtual Exhibition	
17:05	CURRENT AND FUTURE TREATMENTS IN FA Chairs: A. Risitano (IT) & R. Peffault de Latour (FR)	Discussion, Q&A C. DUFOUR(IT) C. BONFIM(BR) J. BUEREN(SP)
17:50	CLOSING	R. PEFFAULT DE LATOUR(FR) A. LANKESTER(NL) S. CORBACIOGLU(DE) H. MEKELENKAMP(NL)



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Severe Aplastic Anaemia Working Party

Lectures On-Demand

AA DIAGNOSIS ALGORITHM AND DBA

When should we search for germline mutations in aplastic anemia? | R. PEFFAULT DE LATOUR | France

DBA outside BMT: an update | T. LEBLANC | France

DBA and BMT | J-H. DALLE | France

CURRENT AND FUTURE TREATMENTS IN FA

BMT (outside alternative) in FA | C. DUFOUR | Italy

Alternative BMT | C. BONFIM | Brazil

Gene therapy in FA | J. BUEREN | Spain

Wednesday, 8 July 2020

Nurses Group

16:05 **CARE FOR THE DONOR** Discussion, Q&A
D. HUTT(IL)
Chair:
H. Mekelenkamp (NL)

16:50 Break | Visit Virtual Exhibition

17:10 **THE INFORMATION
ROADMAP DURING
HSCT** Discussion, Q&A
S. VALENTA(CH)
S. SLADE(UK)
Chair:
T. Schroder(FI)

17:50 **END OF SESSIONS**

Thursday, 9 July 2020

16:00 **EMOTIONAL (AND
PHYSICAL) PREPARATION
FOR HSCT** Discussion, Q&A
S. CHATURVEDI(UK)
R. GREGSON(NL)
Chair:
M. Gjergi(IT)

16:45 Break | Visit Virtual Exhibition

17:05 **HOW TO SURVIVE
ISOLATION DURING HSCT** Discussion, Q&A
M. BAKKER(NL)
M. VAN DER HOLST (NL)
Chair:
D. Hutt(IL)

17:45 **END OF SESSIONS**

Friday, 10 July 2020

16:00 **LOOKING BACK ON HSCT** Discussion, Q&A
T. NEUMAN(NL)
J.E. BENSE (NL)
A. SCHERWATH(DE)
Chair:
H. Mekelenkamp(NL)

16:45 Break / Visit Virtual Exhibition

17:05 **SUPPORTIVE CARE** Discussion, Q&A
J. LARGE(UK)
D. WRIGHT(UK)
Chair:
S. Slade(UK)

17:40 **CLOSING REMARKS** H. MEKELENKAMP(NL)

Nurses Group

Lectures On-Demand

CARE FOR THE DONOR

Being an adult donor for your sibling | A. KISCH | Sweden

Being a pediatric donor for your sibling | D. HUTT | Israel

THE INFORMATION ROADMAP DURING HSCT

Information session pre-HSCT? | S. SLADE | United Kingdom

Using E-health | S. VALENTA | Switzerland

EMOTIONAL (AND PHYSICAL) PREPARATION FOR HSCT

Adult (holistic needs assessment) | S. CHATURVEDI | United Kingdom

Pediatric | R. GREGSON | The Netherlands

HOW TO SURVIVE ISOLATION DURING HSCT

Surviving physically | M. BAKKER & M. VAN DER HOLST | The Netherlands

Patient experiences | P. VAN LEEUWEN | The Netherlands

LOOKING BACK ON HSCT

Value Based Healthcare | T. NEUMAN & J.E. BENSE | | The Netherlands

Surviving Cognitively | A. SCHERWATH | Germany

SUPPORTIVE CARE

Pre-BMT considerations for patients with PNH clone | J. LARGE | United Kingdom

Understanding needs of AYA's | D. WRIGHT | United Kingdom