

Scientific Programme

Friday - October 11th, 2019

10.00-11.00	Registration & Welcome Coffee	
11.00-11.10	Welcome	<i>A. Lankester, P. Veys</i>
11.10-11.20	Introduction – 40 years of SCT & Gene Therapy at GOSH	<i>P. Veys</i>
11.20-11.45	From TALENS and CRISPR to base conversion: Cells for ALL?	<i>W. Qasim</i>
11.45-12.45	GENE THERAPY VS SCT FOR WAS AND CGD	
	<i>Chairs: A. Lankester, P. Veys</i>	
	Allo-HSCT in CGD	<i>R. Chiesa</i>
	GT in CGD	<i>C. Booth</i>
	Inflammation in CGD	<i>A. Magnani</i>
	Discussion HSCT vs GT in CGD	<i>Moderated by A. Lankester</i>
	Allo-HSCT in WAS	<i>M. Albert</i>
	GT in WAS	<i>A. Aiuti</i>
	Discussion HSCT vs GT in WAS	<i>Moderated by P. Veys</i>
12.45-13.45	Lunch Break	
13.45-14.35	NEWBORN SCREENING AND THEN...	
	<i>Chairs: B. Gaspar, S. Pai, G. Lucchini</i>	
	Conditioning regimen for SCID in the NBS era	<i>C. Dvorak</i>
	Mind the gap between NBS and definitive treatment: how to keep the patient safe	<i>J. Heimall</i>
	Gene therapy for SCIDs, where do we stand	<i>C. Booth</i>
	Impact of genetic diagnosis in the SCID NBS era	<i>A. Gennery</i>

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14.35-15.45 NEW GENE THERAPIES

Chairs: A. Fischer, C. Booth

ART-SCID	<i>M. Cowan</i>
LAD	<i>E. Almarza</i>
Fanconi Anaemia	<i>J. Sevilla, E. Almarza</i>
p47 CGD	<i>G. Santilli</i>

15.45-16.00 Coffee Break

16.00-16.45 GENOME EDITING – EMERGING NEW OPPORTUNITIES

Chairs: W. Qasim

WAS	<i>A. Cavazza</i>
Genome edited T-cells	<i>C. Georgiadis</i>
XLP	<i>B. Houghton</i>

16.45-18.00 HLH – WHAT'S NEW?

Chair: K. Rao

Alemtuzumab	<i>D. Moshous</i>
Emapalumab	<i>A. Rao</i>
Future targets for combination therapy in HLH	<i>H. Decaluwe</i>
HLH registry	<i>S. Ehl</i>
Perforin GT	<i>C. Booth</i>
Munc GT	<i>T. Cathomen</i>

18.00 Close

19.30 Networking Dinner

Saturday - October 12th, 2019

09.00-09.15	Welcome and IEWP Report	<i>A. Lankester</i>
09.15-10.30	STEM CELL GENE THERAPY (GT) IN METABOLIC DISEASES	
	<i>Chairs: R. Wynn, M.E. Bernardo</i>	
	GT in MLD	<i>V. Calbi</i>
	GT in MPS1/Hurler	<i>M.E. Bernardo</i>
	GT in MPSIII1/Sanfilippo	<i>R. Wynn</i>
	Debate: SCT vs GT for X-ALD	<i>T. Lund, C. Sevin</i>
10.30-11.00	Coffee Break	
11.00-12.00	ALL THINGS RAG	
	<i>Chairs: M. Hoenig, A. Lankester</i>	
	RAG deficiency: new insights into inflammation and thymic defects	<i>L. Notarangelo</i>
	Hypomorphic RAG SCT study	<i>C. Schuetz, B. Neven</i>
	RAG1 gene therapy	<i>B. Berghuis, A.Lankester</i>
12.00-13.00	HB PATHY SESSION	
	<i>Chairs: J. de la Fuente, P. Amrolia</i>	
	Stem cell therapy in b-thalassemia	<i>S. Marktel</i>
	Stem cell therapy in b-thalassemia	<i>F. Locatelli</i>
	Discussion and debate	
13.00-14.00	Lunch Break	
14.00-15.00	THYMIC ISSUES	
	<i>Chairs: A. Worth, G. Hollander</i>	
	Novel insight into thymus development: new tools and new complexities	<i>G. Hollander</i>

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	Thymic stromal cell defects: clinical aspects	<i>F. Dhalla</i>
	Approaches in correcting athymia	<i>A. Worth, P. Bonfanti</i>
15.00-15.30	Coffee Break	
15.30-16.30	HSCT OR NOT?	
	<i>Chairs: A. Worth, B. Neven</i>	
	LRBA deficiency: Does the immune deficiency and dysregulation activity (IDDA) score facilitate the decision, whom and when to transplant?	<i>V. Tesch, B. Shadur, P. Stepensky</i>
	LV_FOXP3 Treg-like cells in IPEX	<i>R. Bacchetta</i>
	Preclinical studies on CD4 gene therapy in IPEX	<i>M. Cavazzana</i>
	Role of HSCT in Schimke syndrome	<i>B. Neven</i>
16.30-17.30	IEWP STUDY REPORTS/PROPOSALS AND INTERNATIONAL REPORTS	
	<i>Chairs: A. Lankester, M. Albert, S. Hazelaar</i>	
	Haplo PT-CY vs TCR $\alpha\beta$	<i>M. Slatter, M. Albert, B. Neven, A. Bertaina</i>
	Adult PID	<i>E. Morris</i>
	Interaction with CIBMTR/PIDTC	<i>L. Griffith</i>
	Japanese experience with UCBT in SCID	<i>S. Miyamoto</i>
	Others	
17.30	Close	
18.30	Recital kindly sponsored by GOSHCC	
19.30	Social Dinner	

Sunday - October 13th, 2019

09.00-10.00 **IEWP Renewed Guidelines**

Chairs: A. Lankester, M. Albert

10.00-10.15 **Coffee Break**

10.15-11.15 **PK/INDIVIDUALIZED CONDITIONING AS A TOOL TO IMPROVE OUTCOME**

Chairs: R. Chiesa, T. Güngör, R. Bredius

Introduction to PK/PD modelling *J. Standing*

Busulfan PK *M. Hauri-Hohl*

Treosulfan PK *R. Chiesa*

ATG/Alemtuzumab PK *R. Bredius*

The future of TDM in HCT *J. Standing*

Discussion

11.15-12.30 **SPECIAL CHALLENGES AND NOVEL INDICATIONS**

Chairs: M. Slatter, S. Bakhtiar

Ataxia Telangiectasia (AT) and HSCT experience *S. Bakhtiar*

AT in NBS, a clinical dilemma *D. Berghuis*

HSCT in VEO-IBD (London experience) *A. Worth*

HSCT in XLA (who benefits) *B. Shillitoe*

12.30 **Final Remarks** *P. Veys, A. Lankester*

13.00 **Farewell Lunch**