



Inborn Errors Working Party  
Annual Conference  
24<sup>th</sup> – 26<sup>th</sup> September 2021  
Milan, Italy

## Scientific Programme Friday, 24th of September 2021

<b>11.00 – 12.00</b>	<b>Registration and Welcome Coffee</b>	
<b>12.00 – 13.30</b>	<b>Welcome</b> Chairs: Bénédicte Neven and Claire Booth	
	Welcome Message	Francesca Pasinelli and Fabio Ciceri
	Overview of Sr-TIGET gene therapy clinical trials New perspectives for gene therapy and gene editing	Alessandro Aiuti Luigi Naldini
<b>13.30 – 14.30</b>	<b>Lunch Break</b>	
<b>14:30 – 16.15</b>	<b>Stem cell therapy in metabolic diseases</b> Chairs: Maria Ester Bernardo and Caroline Lindemans	
	Use of Rituximab in the preparation of CBT Gene therapy in MLD Gene therapy in MPS IIIA and future trial in MPS II GT for MPSIH Pathophysiological mechanism of bone damage and bone cross correction in MPSIH gene therapy	Rob Wynn Valeria Calbi Claire Horgan Maria Ester Bernardo Ludovica Santi
<b>16.15 – 16.45</b>	<b>Coffee Break</b>	
<b>16.45 – 17.30</b>	<b>New IEWP HSCT guidelines</b> Chairs: Tayfun Güngör and Emma Morris	
	Time to start working on the next version? Discussion	Arjan Lankester
<b>17.30 – 17.40</b>	<b>Closing</b>	
<b>20.00</b>	<b>Networking Dinner</b>	



## Scientific Programme

### Saturday, 25th of September 2021

<b>09.00 – 09.15</b>	<b>IEWP news and updates</b>	Bénédicte Neven
<b>09.15 – 10.45</b>	<b>SCID</b> Chairs: Arjan Lankester and Manfred Hoenig	
	Impact of naive CD4 on outcome, SCETIDE study Consequences of absent B-cell function long-term Retroviral GT for ADA-SCID LV GT for ADA SCID GT trials available in Europe for PID ARTEMIS GT HTLP (human T lymphocyte progenitor)	Arjan Lankester Bénédicte Neven Maria Pia Cicalese Claire Booth Claire Booth Marina Cavazzana Marina Cavazzana
<b>10.45 – 11.15</b>	<b>Coffee Break</b>	
<b>11.15 – 12.15</b>	<b>Novel indications/future approaches</b> Chairs: Austen Worth	
	GT trials for X-SCID Stem cell expansion Gene editing for HIGM	Sung-Yun Pai Bernhard Gentner Daniele Canarutto
<b>12.15 – 13.00</b>	<b>Keynote Lecture I</b> Chair: Luigi Naldini	
	Advances and pitfalls in the curative treatment of PID	Alain Fischer
<b>13.00 – 14.00</b>	<b>Lunch Break</b>	
<b>14.00 – 15.00</b>	<b>Optimizing alternative donor outcome in IE ‘haplo approaches’</b> Chairs: Michael Albert and Bénédicte Neven	
	TCRa/b results Newcastle PTCY results Brazil PTCY results Paris IEWP haplo study – preliminary results	Su Han Lum Carmem Bonfim Bénédicte Neven Mary Slatter
<b>15.00 – 16.15</b>	<b>Weighing the pros and cons of HSCT or not in non-SCID PID</b> Chairs: Carsten Speckmann and Isabelle Meyts	
	TTC7A working group initiative JAKinibs in STAT1/STAT3 GOF	Giovanna Lucchini Carsten Speckmann and Bénédicte Neven
	HSCT in DADA2? No Yes Discussion	Amanda Ombrello Giorgia Bucciol
<b>16.15 – 16.45</b>	<b>Coffee Break</b>	



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## Scientific Programme Saturday, 25th of September 2021

**16.45 – 17.30**

**Keynote Lecture II**

Chair: Alessandro Aiuti

Treg-based therapies for autoimmune disorders

Maria Grazia Roncarolo

**17.30 – 19.00**

**IEWP studies and proposals**

Chairs: Arjan Lankester and Stephan Ehl

AYA – final results

Porphyria – final results

WAS – final results

ICF – results

hypoRAG – results

IFNGR – results

2<sup>nd</sup>/3<sup>rd</sup> HSCT – proposal

Emma Morris

Despina Moshous

Michael Albert

Arjan Lankester

Catharina Schütz

and Julia Gerke

Manfred Hoenig

Zohreh Nademi

**20.00**

**Social Dinner**



## Scientific Programme Sunday, 26th of September 2021

**09.00 – 10.15**     **Stem cell therapy in hemoglobinopathy**

Chair: Sarah Marktél and Marco Zecca

Best candidates for cellular therapy  
Progress in HSCT  
Challenges in thalassemia GT: update from TIGET  
Gene-editing in hemoglobinopathies  
Update on bbb studies

Nica Cappellini  
Marco Zecca  
Giuliana Ferrari  
Selim Corbacioglu  
Franco Locatelli

**10.15 – 11.00**     **Coffee Break**

**11.00 – 12.00**     **What's new in conditioning?**

Chairs: Andrew Gennery and Persis Amrolia

Anti-CD66 RIT - update from UK RIT study  
Novel anti-CD45 drug conjugates as targeted conditioning  
for SCT and GT  
Anti c-kit conditioning

Robert Chiesa  
Persis Amrolia

Rajni Agarwal

**12.00 – 13.20**     **Clinical and other challenges in HSCT for PID**

Chairs: Jana Pachlopnik Schmid and Fabio Candotti

Results of NBS in Germany and situation in other  
European countries  
ZNF1-deficiency  
HLH treated by Campath  
SHP2 inhibitors for SAP deficiency  
Emapalumab for prevention of GF

Carsten Speckmann

Jana Pachlopnik Schmid  
Despina Moshous  
Claire Booth  
Pietro Merli

**13.20 – 13.35**     **Final Remarks**

Bénédicte Neven