

FRIDAY, 23 SEPTEMBER 2022

13.00 – 13.30 Registration and Welcome Coffee

13.30 – 14.15 Welcome

Chairs: Bénédicte Neven and Despina Moshous

HSCT in PIDs: from 1968 to 2022 and beyond

Alain Fischer

14:15 – 15.00 Stem cell therapy in metabolic diseases (1)

Chairs: Maria Ester Bernardo and Robert Wynn

GT for MLD: post-marketing data (initial results from US, France, Germany)

LV-GT in MPS IIIA

Early clinical outcome after HSCT-GT in MPSIH

Valeria Calbi

Rob Wynn

Maria Ester Bernardo

15.00 – 15.30 Coffee Break

15:30 – 16:45 Stem cell therapy in metabolic diseases (2)

Chairs: Maria Ester Bernardo and Robert Wynn

UCBT + in vivo GT for Krabbe disease

Novel GT approaches/new indications:

MPSII

Development of an ex-vivo GT platform for LSDs with skeletal involvement

Maria Escolar

Robert Wynn

Alessandro Aiuti

16.45 – 17.15 Keynote Lecture

How to improve immune reconstitution after HSCT

Marina Cavazzana

17.15 – 18.00 IEWP studies committee meeting

18.00 – 23:00 Networking Event

SATURDAY, 24 SEPTEMBER 2022

9.00 – 10.30 SCID

Chairs: Bénédicte Neven and Manfred Höning

Newborn Screening:

Pitfalls of NBS
US experience and SCID definition (PIDTC revision)
Update on NBS in Europe

Mirjam van der Burg
Chris Dvorak
Carsten Speckmann

Long-term outcome:

When is the time for a second procedure in SCID?
Case Presentation
How to retransplant
Gene therapy as a second procedure for X-SCID

Manfred Höning
Bénédicte Neven
Bénédicte Neven
Suk See De Ravin

10.45 – 11.15 Coffee Break

11:15 – 11:25

Gene therapy in RAG1 SCID

Arjan Lankester

11.25 – 11.55

IEWP news and updates

Chairs: Bénédicte Neven and Michael Albert

11.55 – 12.25

Round table discussion: Access to gene therapy

Claire Booth, Arjan Lankester, Alain Fischer, Alessandro Aiuti

12.25 – 13:15

Keynote Lecture:

Multi-omics dissection of the immunopathology of RAG defects

Luigi Notarangelo

13.15 – 14.15 Lunch Break

14.15 – 15.45

Conditioning for HSCT in IEI

Chairs: Persis Amrolia and Michael Albert

Randomized Treo vs Bu (medac trial)
ARTIC trial
MGTA-117 as conditioning prior to HSCT
JSP 191 clinical trial update
Bu-vs-Treo exposure in relation to gonadal toxicity

Karl Walter Sykora
Arjan Lankester
Shawn Rose
Rajni Agarwal
Eileen vd Stoep

15.45 – 16.15 Coffee Break

16.15 – 17.30

Stem cell therapy in hemoglobinopathies

Chairs: Marco Zecca and Franco Locatelli

Perspective of treatment in thalassemia (Iuspatcept)

Olivier Hermine

Haplo HSCT in hemoglobinopathies:

PTCY
TCRab
Gene therapy in hemoglobinopathies
Gene therapy in hemoglobinopathies – future approaches

Arjan Lankester
Franco Locatelli
Franco Locatelli
Annarita Miccio

17.30 – 19.25

IEWP studies and proposals

Chairs: Su Han Lum and Arjan Lankester

Haplo TCRa/b and PTCY: final results
Second allo HSCT for IEI: preliminary results
TTC7A
STATIGOF
Haplo HSCT in WAS
ARCIB Proposal

Su Ham Lum
Zohreh Nademi
Giovanna Lucchini
Catharina Schuetz
Carmem Bonfim
Gianluca Dell'Orso

20.00

Networking dinner



Inborn Errors Working Party Annual Conference

Paris, France
23-25 September 2022

SUNDAY, 25 SEPTEMBER 2022

9.00 – 10.30 **Emerging indications**

Chairs: Emma Morris and Andrew Gennery

HSCT in XLA
Gene editing in XLA
HSCT in CTLA4 def.
Gene editing in CTLA4 def.
CARMIL2
HSCT for CSFR1

Andrew Gennery
Georgia Santilli
Mary Slatter
Emma Morris
Romain Levy
Caroline Lindemans

10.30 – 11.00 **Coffee Break**

11.00 – 12.15 **Challenges in inflammatory conditions**

Chairs: Despina Moshous, Stephan Ehl

STAT1GOF genotype/phenotype correlation
JAKinibs for STAT1/3GOF
CGD and inflammation

Siobhan Burns
Carsten Speckmann
Emmanuelle Six

12.15 – 12.30 **Final Remarks**