Dear Colleagues,

This year, the annual conference of the Inborn Errors Working Party (IEWP) will be organised in Graz, Austria, on 22-24 September 2023. It will be a great pleasure to welcome you on behalf of EBMT.

As always, the meeting will feature comprehensive sessions on stem cell transplantation and gene therapy in inborn errors of immunity, inborn errors of metabolism, hemoglobinopathies, as well as new indications.

This year’s special sessions will focus on the pathophysiology of IEI, on hematopoietic defects (telomeropathies, rasopathies, bone marrow failures), the use of serotherapy, and how to make better use of existing registries. A keynote presentation will be given by Elie Haddad (Montreal, CA).

We look forward to welcoming you in Graz.

Bénédicte Neven, IEWP Chair
Michael Albert, IEWP Secretary
Markus Seidel, Local Organiser
Friday, 22 September 2023

13:00 – 13:45  Registration & Welcome Coffee

13:45 – 14:00  Welcome
   Chairs: Michael Albert (DE), Markus Seidel (AT)

14:00 – 15:45  Session I - Stem cell therapy in metabolic diseases
   Chairs: Maria Ester Bernardo (IT), Rob Wynn (UK)

  14:00 – 14:12  Safety and tolerability of myeloablative busulfan-based conditioning
                 regimens in young children below the age of 18 months affected by
                 LSD: combined experience of two transplant centers
                 Valeria Calbi (IT)

  14:12 – 14:24  Allo-HSCT for LSDs in the NBS era: combined experience in
                 US and Europe
                 Jaap Jan Boelens (US)

  14:24 – 14:34  Update on the phase 1/2 Clinical Trial of Intravenous FBX-101
                 (AAVrh10.hGALC) Administered after UCBT for the treatment of Krabbe
                 Disease
                 Maria Escolar (US)

  14:34 – 14:44  Libmeldy gene therapy in the real world
                 Valeria Calbi (IT)

  14:44 – 14:54  Update on HSPC-GT for MPSIIIA
                 Rob Wynn (UK)

  14:54 – 15:04  Update on HSPC-GT for MPSI IH
                 Francesca Tucci

  15:04 – 15:20  Update on HCTGT in CD3 and MPSII
                 Rob Wynn (UK)

  15:20 – 15:45  Discussion, Q&A

15:45 – 16:15  Coffee Break

16:15 – 17:30  Session II - Pathophysiology of IEI and its role in cellular therapy
   Chairs: Romain Levy (FR), Fabian Hauck (DE)

  16:35 – 16:55  A novel Inborn Error of Pattern-triggered Immunity
                 Fabian Hauck (DE)

  16:55 – 17:15  Dupilumab for primary atopic diseases
                 Romain Levy (FR)

  17:15 – 17:30  Discussion, Q&A

17:30 – 18:00  IEWP study committee meeting (invitation only)

19:30  Networking Event
Saturday, 23 September 2023

09:00 – 10:00 Session III - Hematopoietic defects (Telomeropathies)
Chairs: Carsten Speckmann (DE), Carmem Bonfim (BR)

- 9:00 – 9:30 Radiation- and Alkylator-Free BMT for Dyskeratosis Congenita
  Suneet Agarwal (US)
- 9:30 – 9:45 HSCT for telomeropathies in Latin America
  Carmem Bonfim (BR)
- 9:55 – 10:00 Discussion, Q&A

10:00 – 10:30 Coffee Break

10:30 – 12:00 Session IV - Conditioning/PK
Chairs: Arjan Lankester (NL), Despina Moshous (FR)

- 10:30 – 10:50 Busulfan exposure in PID patients: what is the sweet spot?
  Jaap Jan Boelens (US)
- 10:50 – 11:00 Fludarabine PK/PD: Model-Based Dosing for Everyone?
  Christopher Dvorak (US)
- 11:00 – 11:10 ARTIC study final results or combined Trep PK data
  Arjan Lankester (NL)
- 11:10 – 11:20 Treo/Bu conditioning and fertility
  Arjan Lankester (NL)
- 11:20 – 11:40 Toxicities of PTCY in IEIs
  Despina Moshous (FR)
- 11:40 – 12:00 Discussion, Q&A

12:00 – 12:45 Keynote Lecture
The Journey of HSCT in SCID
  Elie Haddad (CA)

12:45 – 14:00 Lunch Break

14:00 – 15:15 Session V - Challenging indications and novel approaches
Chairs: Mary Slatter (UK), Tomas Fox (UK)

- 14:00 – 14:25 HSCT for JIA/Still
  Juliana Silva (UK)
- 14:25 – 14:35 HSCT for complex CVID: don`t do it!
  Claudia Wehr (DE)
- 14:35 – 14:45 HSCT for complex CVID: do!
  Emma Morris (UK)
- 14:45 – 15:00 Solid organ tx and HSCT
  Paul Szabolcs (US)
- 15:00 – 15:15 Discussion, Q&A
15:15 – 16:15 Session VI - HLH and related diseases
Chairs: Claire Booth (UK)

- 15:15 – 15:30 EBV control
- 15:30 – 15:40 DLI after SCT for pHLH
- 15:40 – 15:50 Polymorphism or risk allele? PRF1 A91V in trans with a “severe” PRF1 mutation
- 15:50 – 16:00 Gene editing for XIAP
- 16:00 – 16:15 Discussion, Q&A

16:15 – 16:45 Coffee Break

16:45 - 17:10 Session VII – Panel Discussion
Conceptual approach to gene therapy for HLH – a panel discussion
Stephan Ehl (DE), Claire Booth (UK), Despina Moshous (FR)

17:15 – 18.30 Session VIII - IEWP Studies and proposals
Chairs: Bénédicte Neven (FR), Michael Albert (DE)

- 17:15 – 17:25 HSCT for CTLA-4 Haploinsufficiency
- 17:25 – 17:35 Update on HSCT for STAT1GOF
- 17:35 – 17:45 Outcome of second allogenic transplant in non-SCID IEI
- 17:45 – 17:55 FHLH2/3
- 17:55 – 18:05 HSCT for C1Q deficiency
- 18:05 – 18:15 Haplo HSCT for CGD
- 18:15 – 18:25 AGORA update
- 18:25 – 18:30 Discussion, Q&A

20.00 Networking Dinner
Sunday, 24th of September 2023

09:15 – 10:00  **Session IX - How can we use registries in a more effective manner?**  
*Chairs: Markus Seidel (AT), Christopher Dvorak (US)*

- 9:15 – 9:25  Landscape of ‘Registry’ Options in Europe and the US/Canada  
Christopher Dvorak (US)  
Markus Seidel (AT)
- 9:25 – 9:40  “Lost in registration”/ The path of a PID patient into the registry maze  
Stephan Ehl (DE)
- 9:40 – 9:55  “Speaking different languages” / How to get registries to talk to each other and share common data elements to enable transatlantic studies of rare diseases  
Larisa Broglie (US)
- 9:55 – 10:00  Discussion, Q&A

10:00 – 10:45  **Session X - Long-term follow-up and late complications**  
*Chairs: Reem Elfeky (UK), Emma Morris (UK)*

- 10:00 – 10:10  US SCID long-term outcome post-HSCT  
Christopher Dvorak (US)
- 10:10 – 10:20  Non-osteopenic bone pathology as a late effect post-HSCT in patients with IEI  
Reem Elfeky (UK)
- 10:20 – 10:30  Long-term follow-up post-gene therapy for patients with IEI  
Emma Morris (UK)
- 10:30 – 10:45  Discussion, Q&A

10:45 – 11:15  **Coffee Break**

11:15 – 12:30  **Session XI - “Classic” indications (SCID, non-SCID...)**  
*Chairs: Manfred Höning (DE), Andrew Gennery (UK)*

- 11:15 – 11:35  Conditioning SCID Infants Diagnosed Early randomized busulfan study  
Sung-Yun Pai (US)
- 11:35 – 11:50  GPOH SCID registry: infection, therapy, outcome  
Manfred Höning (DE)
- 11:50 – 12:05  RAG1/2 gene therapy (RECOMB) results  
Arjan Lankester (NL)
- 12:05 – 12:15  LV-GT for X-SCID  
Sung-Yun Pai (US)
- 12:15 – 12:30  Discussion, Q&A

12:30 – 12:45  **Final Remarks**
*Benedicte Neven (FR), Michael Albert (DE), Markus Seidel (AT)*
Thanks to our sponsors!

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For further information, please contact:
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