



Inborn Errors Working Party

Bénédicte Neven (chair)

Michael Albert (secretary/ESID chair)

Paris, France

Munich, Germany

STUDIES

1. Second and third allo HSCT in patients with (Non SCID) PID
2. TCR $\alpha\beta$ or PTCY mismatched HSCT in PID
3. Graft failures and immune cytopenia in cord blood transplantation (CBT) for inherited metabolic diseases (IMD)
4. HSCT in patients with IFNGR1/2 deficiency
5. Evaluation of characteristics and outcomes of ICF syndrome after allo HSCT
6. Outcome of haplo identical donor in CGD patients*
7. Setting the stage for newborn screening and gene therapy of FHL2 and FHL3*

* If you wish to participate in data collection for these studies, please contact us at iewp@ebmt.org

RECENT PUBLICATION ON BEHALF OF IEWP

[Hypomorphic RAG deficiency: impact of disease burden on survival and thymic recovery argues for early diagnosis and HSCT.](#) Blood 2022

C Schuetz, J Gerke, M Ege, J Walter, M Kusters, A Worth, J A Kanakry, D Dimitrova, B Wolska-Kuśnierz, K Chen, E Unal, M Karakukcu, O Pashchenko, J Leiding, T Kawai, P J Amrolia, D Berghuis, J Buechner, D Buchbinder, M J Cowan, A R Gennery, T Güngör, J Heimall, M Miano, I Meyts, E C Morris, J Rivière, S O Sharapova, P J Shaw, M Slatter, M Honig, P Veys, A Fischer, M Cavazzana, D Moshous, A Schulz, M H Albert, J M Puck, A C Lankester, L D Notarangelo, B Neven

[The efficacy and safety of allogeneic stem cell transplantation in Mevalonate Kinase Deficiency.](#) *Pediatr Rheumatol* 2022

Jerold Jeyaratnam, Maura Faraci, Andrew R Gennery, Katarzyna Drabko, Mattia Algeri, Akira Morimoto, Tiarlan Sirait, Arjan C Lankester, Michael Albert, Benedicte Neven, Joost Frenkel

[Hematopoietic stem cell transplantation for adolescents and adults with inborn errors of immunity: an EBMT IEWP study.](#) Blood 2022

Michael H Albert, Tiarlan Sirait, Dirk-Jan Eikema, Katerina Bakunina, Claudia Wehr, Felipe Suarez, Maria Laura Fox, Nizar Mahlaoui, Andrew R Gennery, Arjan C Lankester, Rita Beier, Maria Ester Bernardo, Venetia Bigley, Caroline A Lindemans, Siobhan O Burns, Ben Carpenter, Jaroslaw Dybko, Tayfun Güngör, Fabian Hauck, Su Han Lum, Dmitry Balashov, Roland Meisel, Despina Moshous, Ansgar Schulz, Carsten Speckmann, Mary A Slatter, Brigitte Strahm, Duygu Uckan-Cetinkaya, Isabelle Meyts, Tanja C Vallée, Robert Wynn, Bénédicte Neven, Emma C Morris

[Hematopoietic stem cell transplantation for Wiskott-Aldrich syndrome: an EBMT Inborn Errors Working Party analysis.](#) Blood 2022

Michael H Albert, Mary A Slatter, Andrew R Gennery, Tayfun Güngör, Katerina Bakunina, Benyamin Markovitch, Sheree Hazelaar, Tiarlan Sirait, Virginie Courteille, Alessandro Aiuti, Olga V Aleinikova, Dmitry Balashov, Maria Ester Bernardo, Ivana Bodova, Benedicte Bruno, Marina Cavazzana, Robert Chiesa, Alain Fischer, Fabian Hauck, Marianne Ifversen, Krzysztof Kałwak, Christoph Klein, Alexander Kulagin, Alphan Kupesz, Baris Kuskonmaz, Caroline A Lindemans, Franco Locatelli, Su Han Lum, Alexey Maschan, Roland Meisel, Despina Moshous, Fulvio Porta, Martin G Sauer, Petr Sedlacek, Ansgar Schulz, Felipe Suarez, Tanja C Vallée, Jacek H Winiarski, Marco Zecca, Bénédicte Neven, Paul Veys, Arjan C Lankester

[Outcome of haematopoietic cell transplantation in children with lysosomal acid lipase deficiency: a study on behalf of the EBMT Inborn Errors Working Party.](#) BMT 2023

Su Han Lum, Milen Minkov, Simon A Jones, Sheree Hazelaar, Tiarlan Sirait, Jane E Potter, Polina Stepensky, Frederic Garban, Herbert Pichler, Jerry Stein, Zuhre Kaya, Ansgar Schulz, Karin Mellgren, Cristina Diaz de Heredia, Cecile Pochon, Susana Riesco, Miguel Angel Diaz, Gérard Michel, Caroline Lindemans, Bernd Gruhn, Michael H Albert, Arjan C Lankester, Bénédicte Neven, Robert Wynn

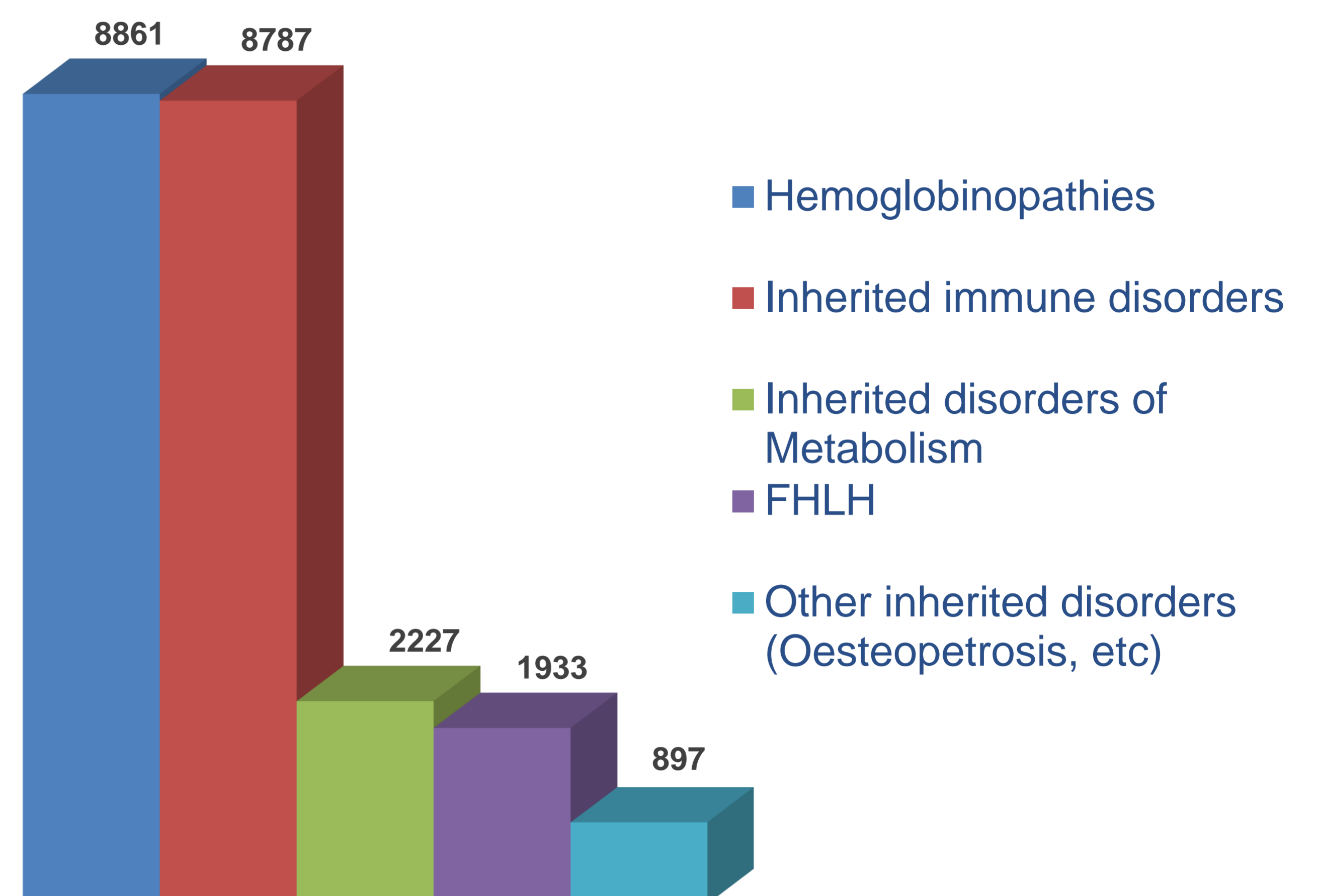
[Decision making on HSCT in patients with hemoglobinopathies; an EBMT Pediatric Diseases Working Party and Inborn Errors Working Party scenario-based survey on physicians' perspectives.](#) BMT 2023

Hilda Mekelenkamp, Martine de Vries, Benny Markovitch, Tiarlan Sirait, Arwen H Pieterse, Joëll Bense, Katharina Kleinschmidt, Michael H Albert, Bénédicte Neven, Selim Corbacioglu, Arjan Lankester, Frans Smiers

[Busulfan-fludarabine- or treosulfan-fludarabine-based myeloablative conditioning for children with thalassemia major.](#) Ann Hematol 2022

Roswitha Luftinger, Natalia Zubarovskaya, Jacques-Emmanuel Galimard, Annamaria Cseh, Elisabeth Salzer, Franco Locatelli, Mattia Algeri, Akif Yesilipek, Josu de la Fuente, Antonella Isqro, Amal Alseraihy, Emanuele Angelucci, Frans J Smiers, Giorgia La La Nasa, Marco Zecca, Tunc Fisgin, Emel Unal, Katharina Kleinschmidt, Christina Peters, Arja Lankester, Selim Corbacioglu.

NUMBER OF PATIENTS REGISTERED AT EBMT REGISTRY



The data was retrieved in March 1st, 2023

IEWP SESSIONS AT EBMT ANNUAL MEETING 2023

Tuesday, 25 April 2023

07:30 - 08:45 Inborn Errors Working Party Business Meeting

Bénédicte Neven (France)

Meeting room 153

11:00 - 12:15 Inborn Errors Working Party Session

Bénédicte Neven (France)

Maillot

Paed2-1 Biology of thymus transplantation, challenges and outcome: the European experience

Graham Davies (UK)

Paed2-2 Thymus development and related disease

Georg Hollander (UK)

Paed2-3 Thymus Reconstitution and HSCT: Lessons from Mouse Models

Graham Anderson (UK)

Paed2-4 Discussion

16:30 - 17:45 Inborn Errors Oral Session

Michael Albert (Germany) & Maria Ester Bernado (Italy)

Maillot

IEWP FUTURE EVENTS 2023

▪ Mid-term meeting: 22-24 June 2023 in Brescia, Italy

Three EBMT working parties (Autoimmune, Inborn Errors and Paediatric) will all meet for the Midterm Meeting in Immune Dysregulatory, Autoinflammatory and Autoimmune Diseases. The meeting will focus on therapeutic approach to classical as well as emerging immunological diseases from an interdisciplinary viewpoint, bringing together both transplant physicians and disease specialists to evaluate the transplant and cellular therapy options.

▪ Annual conference of IEWP: 22-24 September 2023 in Graz, Austria

Contact IEWP

Bénédicte Neven	Chair	benedicte.neven@aphp.fr
Michael Albert	Secretary	michael.albert@med.uni-muenchen.de
Tiar Sirait	Study coordinator	tiarlan.sirait@ebmt.org
Jeroen Knippenberg	Data manager	jeroen.knippenberg@ebmt.org