



European Society for Blood and Marrow Transplantation

Inborn Errors Working Party

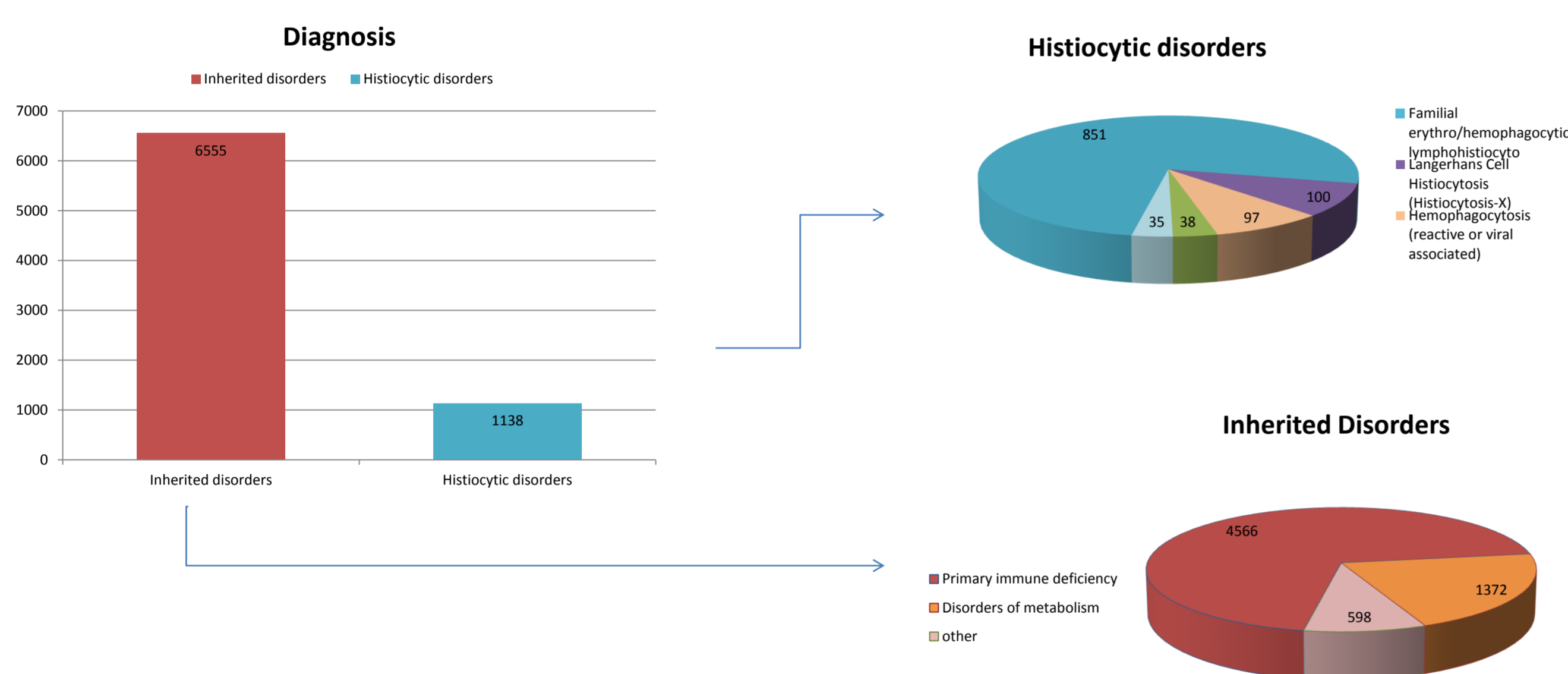
Chair: Andy Gennery – Newcastle, UK
Secretary: Arjan Lankester – Leiden, Netherlands

Studies

New Studies:

- **Outcome of HSCT for DOCK8 deficiency**
Contact: Michael.Albert@med.uni-muenchen.de
- **SCETIDE analysis of SCT in CID to 2013**
Contact: benedicte.neven@aphp.fr
- **SCETIDE analysis of SCT in SCID to 2013**
Contact: A.Lankester@lumc.nl
- **IPEX syndrome – Outcome with or without HSCT (joint with PIDTC)**
Contact: a.r.gennery@ncl.ac.uk, bacchetta.rosa@hsr.it
- **Longterm outcome of HSCT for SCID (joint with PIDTC)**
Contact: a.r.gennery@ncl.ac.uk

Numbers in the registry



Recent Publications

- Wolska-Kuśniercz B, Gregorek H, Chrzanowska K, Piątoś B, Pietrucha B, Heropolitańska-Pliszka E, Pac M, Klaudel-Dreszler M, Kostyuchenko L, Pasic S, Marodi L, Belohradsky BH, Čiznár P, Shcherbina A, Kilic SS, Baumann U, Seidel MG, Gennery AR, Syczewska M, Mikołuc B, Kałwak K, Styczyński J, Pieczonka A, Drabko K, Wakulińska A, Gathmann B, Albert MH, Skarżyńska U, Bernatowska E; Inborn Errors Working Party of the Society for European Blood and Marrow Transplantation and the European Society for Immune Deficiencies. (2015) **Nijmegen Breakage Syndrome: Clinical and Immunological Features, Long-Term Outcome and Treatment Options - a Retrospective Analysis.** J Clin Immunol 35 538-549
- Fioredda F, Iacobelli S, van Biezen A, Gaspar B, Ancliff P, Donadieu J, Aljurf M, Peters C, Calvillo M, Matthes-Martin S, Morreale G, van 't Veer-Tazelaar N, de Wreede L, Al Seraihy A, Yesilipek A, Fisher A, Bierings M, Ozturk G, Smith O, Veys P, Ljungman P, Peffault Delatour R, de Toledo Codina JS, Or R, Ganser A, Afanasyev B, Wynn R, Kalwak K, Marsh J, Dufour C. (2015) **Stem cell transplantation in severe congenital neutropenia: an analysis from the European Society for Blood and Marrow Transplantation.** Blood 126:1885-1892
- Slatter MA, Boztug H, Pötschger U, Sykora K-W, Lankester A, Yaniv I, Sedlacek P, Glogova E, Veys P, Gennery AR, Peters C on behalf of the EBMT Inborn Errors and Paediatric Diseases Working parties. (2015) **Treosulfan-based conditioning regimens for allogeneic Haematopoietic stem cell transplantation in children with non-malignant diseases.** Bone Marrow Transplant 50 1536-1541
- Bode SFN, Ammann S, Al-Herz W, Bataneant M, Bryceson YT, Dvorak CC, Gehring S, Gennery A, Gilmour KC, Gonzalez-Granado LI, Groß-Wieltsch U, Ifversen M, Lingman-Framme J, Jordan MB, Matthes-Martin S, Meesters R, Meyts I, van Montfrans JM, Moshous D, Pachlopnik-Schmid J, Pai S-Y, Palacin PS, Schuermann U, Schuster V, Seidel M, Speckmann C, Stary J, Stepensky P, Karl-Walter Sykora K-W, Tesi B, Vraetz T, Waruiri C, Lehmsberg K, Ehl S, for the Inborn Errors Working Party of the EBMT. (2015) **Infections in primary immunodeficiencies fulfilling criteria for hemophagocytic lymphohistiocytosis: implications for differential diagnosis.** Haematologica 100 978-988

Contact

Andrew Gennery
Chair IEWP
a.r.gennery@ncl.ac.uk

Arjan Lankester
Secretary IEWP
a.lankester@lumc.nl

Roz Gale
Ac. Secr. to A.R. Gennery
Roz.Gale@ncl.ac.uk

EBMT 2016

Monday 4th April

IEWP business meeting
07:00 – 09:00 hrs / room B2 (Level 1 / Pav 5)

Presidential symposium

O004 The Inborn Errors Working Party study of long-term outcomes of haematopoietic stem cell transplantation in patients with severe combined immunodeficiencies.
A. Laberko*, M. Slatter, A. Lankester, R. Bredius, A. Schulz, M. Hoenig, B. Neven, N. Mahlaoui, B. Gaspar, A. Worth, S. Burns, F. Porta, V. Courteille, A. Fischer, A. Gennery

Posters

P291 Early and Late Outcomes After Allogeneic Cord Blood Transplantation For Leukodystrophies: A Joint Study of Eurocord, Inborn Errors WP-EBMT and Duke University J. J. Boelens*, K. Page, A. Paviglianiti, H. Allewelt, F. Volt, J. Hol, G. Michel, M. A. Diaz, V. Bordon, T. O'Brien, P. J. Shaw, C. Kenzey, M. Ayas, E. Gluckman, V. Rocha, A. Ruggeri, J. Kurtzberg and on behalf of Eurocord, WP Inborn Errors EBMT and Duke University Medical Center

Tuesday 5th April

IEWP session
11:00 – 12:30 hrs / Room B5

Presentations:	
11:00 - 11:20	Gene therapy for monogenic bone marrow disorders: current progress and future prospects Speaker: Silvia.Giassa (United Kingdom)
11:40 - 12:00	The future of cell therapies in metabolic diseases Speaker: Robert.Stone (United Kingdom)
11:20 - 11:40	SCT/ Gene Therapy in Hemoglobinopathies Speaker: Simone.Sisti (Italy)
12:00 - 12:20	P-CID and HSCT Speaker: Stefan.Ehl (Germany)
12:20 - 12:30	Conclusion Speaker: Andrew.Gennery (United Kingdom)

Posters

P564 Use of Defibrotide to treat Transplant-Associated Thrombotic Microangiopathy – a Retrospective Study of the Paediatric Diseases and Inborn Errors Working Parties of EBMT L. Yeates*, M. Slatter, S. Bonanomi, W. Lim, S. Ong, A. Dalissier, W. Barberi, A. Schulz, M. Duval, C. Heilmann, A. Willekens, H. Hwang, C. Uderzo, P. Bader, A. Gennery and Paediatric Diseases and Inborn Errors Working Parties of EBMT

Future Meetings

- Joint 10th PDWP Meeting/IEWP Educational Day
Rhodes, May 2016



- ESID Biennial Meeting
Barcelona, September 2016



- IEWP Autumn Meeting with Educational Pre-meeting
Ghent, Nov 2016



- IEWP 2nd Thymic Disorders Workshop
Leiden, November 2016



Further details from: Roz.Gale@ncl.ac.uk

Significant Collaborations

