



European Society for Blood and Marrow Transplantation

Inborn Errors Working Party

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

Studies

- Outcome of HSCT for DOCK8 deficiency
- SCETIDE analysis to 2014
- IPEX syndrome – Outcome with or without HSCT (joint with PIDTC)
- Longterm outcome of HSCT for SCID (joint with PIDTC)
- HSCT for STAT3-GOF (with PIDTC)
- HSCT for LRBA deficiency (with PIDTC)
- Cord Blood SCT for HLH (with Eurocord)
- HSCT for Porphyria (with CIBMTR)
- Outcome of CGD and WAS with Treo/flu or Bu/flu conditioning
- Outcome of LAD HSCT 2008 – 2015
- CGD HSCT outcomes
- Domino HSCT for PID (with PIDTC)

Participate in data collection
Contact: IEWPebmt@lumc.nl

Recent Publications

Nademi Z, Slatter MA, Dvorak CC, Neven B, Suarez F, Fischer A, Booth C, Rao K, Laberko A, Bertand Y, Koltan S, Dębski R, Rodina J, Flood T, Abinun M, Gennery AR, Hambleton S, Ehl S, Cant AJ on behalf of the Inborn Error Working Party of EBMT and ESID. **Haematopoietic Stem Cell Transplant in Patients with Activated PI3K Delta Syndrome.** *J Allergy Clin Immunol* 2016

Speckmann C, Doerken S, Aiuti A, Albert M, Al-Herz W, Allende L, Ariganello P, Avcin T, Perez-Becker R, Cancrini C, Ghosh S, Gennery A, Gilmour K, Gonzalez-Granado L, Hambleton S, Hauck F, Hoenig M, Moshous D, Niehues T, Picard C, Rieber N, Schulz A, Schwarz K, Seidel M, Soler P, Stepensky P, Strahm B, Vraetz T, Worth A, Fuchs S, Uhlmann A, Ehl S, on behalf of the PCID-study of the Inborn Errors Working Party of the EBMT. (2016) **A prospective study on the natural history of patients with profound combined immunodeficiency (P-CID): a "first fifty" analysis.** *J Allergy Clin Immunol*

Yeates L, Slatter MA, Bonanomi S, Lim WFL, Ong SY, Dalissier A, Barberi W, Shulz A, Duval M, Heilmann C, Willekens A, Hwang HYW, Uderzo C, Bader P, Gennery AR. (2016) **Use of Defibrotide to treat Transplant-Associated Thrombotic Microangiopathy – a Retrospective Study of the Paediatric Diseases and Inborn Errors Working Parties of the European Society of Blood and Marrow Transplantation.** *Bone Marrow Transplant* 2016

Chiesa R, Ruggeri A, Paviglianiti A, Zecca M, Gonzales-Vincent M, Bordon V, Yaniv I, Lawson S, Poire X, Dini G, Abecasis M, Al-Seraihy A, Kenzey C, Bierings M, Locatelli F, Gluckman E, Schulz A, Gennery AR, Page K, Kurtzberg J, Rocha V, on behalf of Eurocord, IEWP and CTIWP of EBMT. (2016) **Outcomes after unrelated umbilical cord blood transplantation for children with osteopetrosis.** *Biol Blood Marrow Transplant* 2016;22:1997-2002

Morillo-Gutierrez B, Beier R, Rao K, Burroughs L, Schulz A, Ewins A-M, Gibson B, Sedláček P, Krol L, Strahm B, Zaidman I, Kalwak K, Talano J-A, Woolfrey A, Fraser C, Meyts I, Müller I, Wachowiak J, Bernardo ME, Veys P, Sykora K-W, Gennery A, Slatter M. **Treosulfan based conditioning for allogeneic haematopoietic stem cell transplantation in children with Chronic Granulomatous Disease. Multicentre experience on behalf of the EBMT Inborn Errors Working Party.** *Blood* 2016;128:440-8

Slack J, Albert MH, Balashov D, Belohradsky B, Bertaina A, Bleesing J, Booth C, Büchner J, Buckley RH, Chardin M, Deripapa E, Drabko K, Eapen M, Feuchtinger T, Finocchi A, Gaspar HB, Ghosh S, Gillio A, Gonzalez-Granado LI, Grunebaum E, Guengoer T, Heilmann C, Helminin M, Higuchi K, Imai K, Kalwak K, Kanazawa N, Karasu G, Kucuk ZY, Laberko A, Lange A, Mahlaoui N, Meisel R, Moshous D, Muramatsu H, Parikh S, Pasic S, Schmid I, Schuetz C, Schultz KR, Shaw PJ, Slatter MA, Sykora K-W, Tamura S, Taskinen M, Wawer A, Wolska-Kuśnierz B, Cowan MJ, Fischer A, Gennery AR on behalf of the Inborn Errors Working Party of the European Society for Blood and Marrow Transplantation and the European Society for Immunodeficiencies, Stem Cell Transplant for Immunodeficiencies in Europe (SCETIDE), the Center for International Blood and Marrow Transplant Research, and the Primary Immunodeficiency Treatment Consortium. **Outcome of Haematopoietic Stem Cell Transplantation for DNA-Double Strand Breakage Repair Disorders.** *J Allergy Clin Immunol*

Hoenig M, Lagresle-Peyrou C, Pannicke U, Notarangelo LD, Gennery AR, Slatter M, Cowan MJ, Stepensky P, Al-Mousa HA, Al-Zahrani D, Pai S-Y, Gaspar HB, Oshima K, Imai K, Yabe H, Noroski L, Wulfraat N, Soler Palacin P, Muramatsu H, Debatin K-M, Schuetz C, Moshous D, Schulz AS, Schwarz K, Fischer A, Friedrich W, M. Cavazzana M, on behalf of the EBMT Inborn Errors Working Party **Reticular Dysgenesis: International Survey on Clinical Presentation, Transplantation and Outcome.** *Blood* (re-submitted)

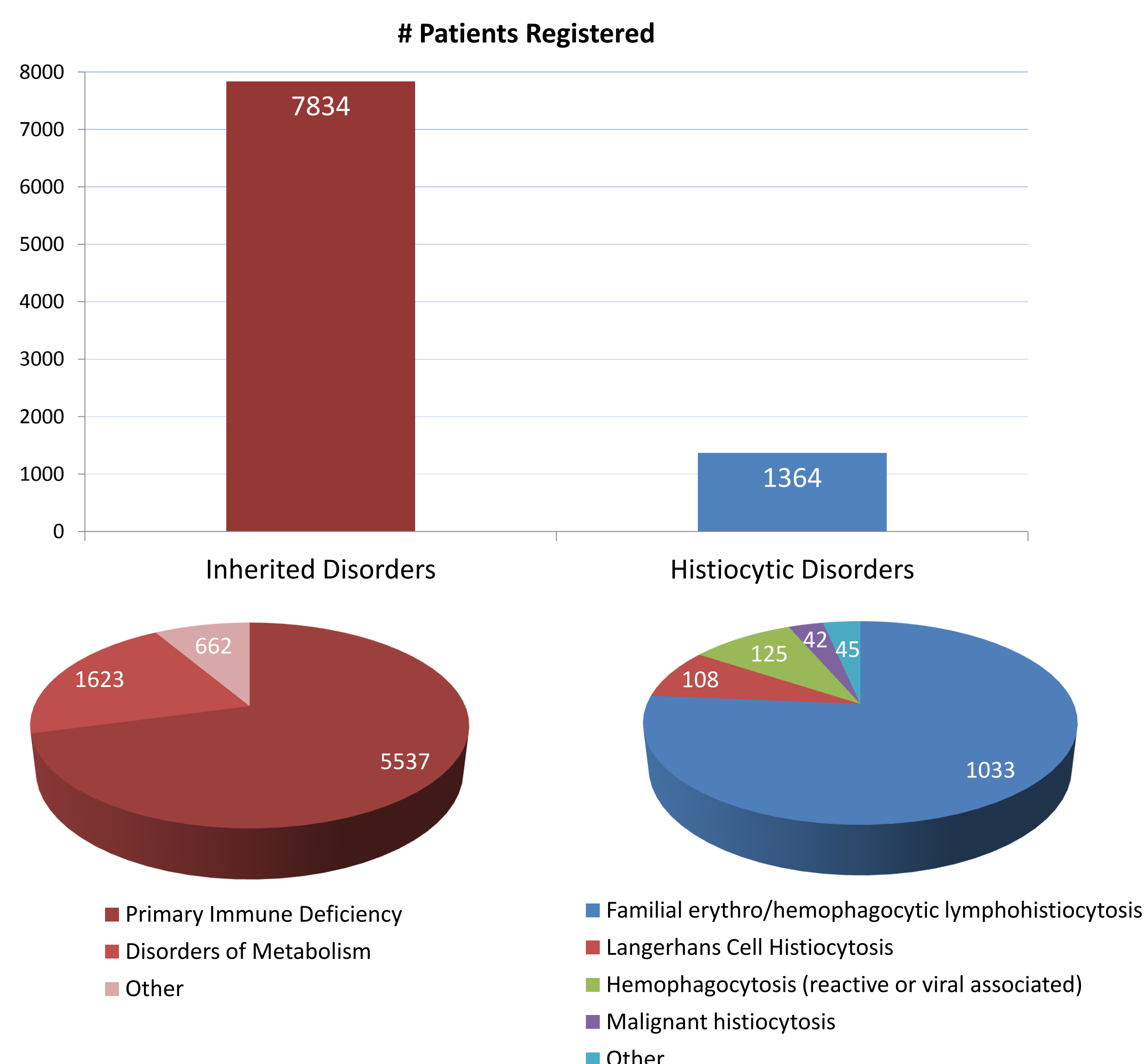
Leiding JW, Okada S, Abinun M, Hagin D, Shcherbina A, Balashov DN, Kim VY, Guthery S, Pulsipher M, Lilic D, Devlin L, Christie S, Depner M, Fuchs S, van Royen-Kerkhof A, Linemans C, Petrovic A, Sullivan KE, Bunin N, Kilic SS, Arpachi F, de la Calle-Martin O, Martinez-Martinez L, Roifman CM, Aldave JC, Kobayashi M, Ohkawa T, Imai K, Iguchi A, Gennery AR, Slatter M, Ochs HD, Morio T, Torgerson TR on behalf of IEWP of EBMT and PIDTC. **Hematopoietic stem cell transplantation in patients with Gain of Function STAT1 Mutation.** *J Allergy Clin Immunol* (re-submitted)

Shekhovtsova Z, Bonfim C, Ruggeri A, Nichele S, Page K, AlSeraihy A, Barriga FC, de Toledo Codina JS, Veys P, Boelens JJ, Mellgren K, Bittencourt H, O'Brien T, Shaw PJ, Chybicka A, Volt F, Giannotti F, Gluckman E, Kurtzberg J, Gennery AR, Rocha V, on behalf of Eurocord, Cord blood committee of CTIWP and IEWP of EBMT. **A Risk Factor Analysis of Outcomes After Unrelated Cord Blood Transplantation for Children with Wiskott-Aldrich Syndrome.** *Haematologica* (submitted)

Significant Collaborations



EBMT Registry



Marseille IEWP-events

Monday 27 March

IEWP business meeting

07:00-09:00

Room: Endoume 3

Tuesday 28 March

IEWP session

11:00-12:30

Room: Amphithéâtre Callegoue

Program IEWP session:

- 11:00-11:20 Despina Moshous
From pathophysiology to treatment: New approaches for HLH
- 11:20-11:40 Mary Slatter
HSCT for monogenic autoimmune disorders
- 11:40-12:10 Arjan Lankester
Update from SCETIDE – HSCT for SCID in the 21st Century
- 12:10-12:30 Jörn-Sven Köhl
HSCT for Adult ALD

Future Events 2017



Contact IEWP

Andrew Gennery
Arjan Lankester
Roz Gale
Henric-Jan Blok

Chair IEWP
Secretary IEWP
Secretary A. Gennery
EBMT data manager

Andrew.Gennery@newcastle.ac.uk
A.Lankester@lumc.nl
Roz.Gale@newcastle.ac.uk
H.J.P.Blok@lumc.nl