



European Society for Blood and Marrow Transplantation

Inborn Errors Working Party

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

Studies

New Studies:

- IEWP/PIDTC joint study – Long-term (>20 yr) follow up of SCID HSCT.

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Patients with Severe Combined Immune Deficiency have been treated by transplantation since the 1960's.

There is a growing cohort of long-term survivors who continue to be followed, but there is no published evidence on the long-term outcome of transplantation for this patient group.

This study will correlate genetic data, transplant donor, conditioning regimen, immune reconstitution and long-term health outcomes in patients with greater than 20 years follow up transplanted in Europe and North America

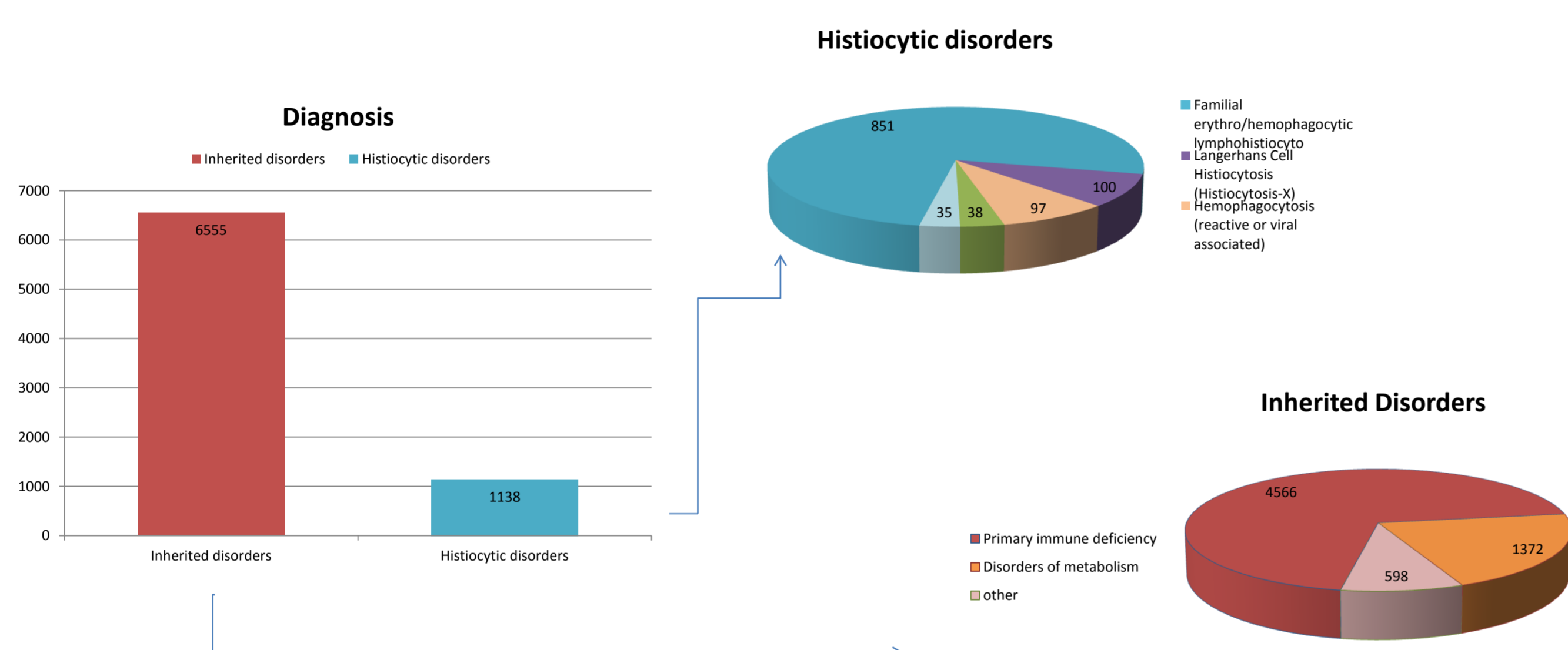
- The PCID Study: outcomes and predictors.

Contact: stephan.ehl@uniklinik-freiburg.de

- Cord Blood Transplantation in Leukodystrophies. (IEWP/Eurocord joint study)

Contact: J.J.Boelens@umcutrecht.nl

Numbers in the registry



Recent Publications

- Wehr C, et al. [Multicenter experience in hematopoietic stem cell transplantation in 25 patients with common variable immunodeficiency.](#) *J Allergy Clin Immunol.* 2015 Jan 14. pii: S0091-6749(14)01732-1. doi: 10.1016/j.jaci.2014.11.029. [Epub ahead of print] PMID: 25595268
- Aydin SE, et al. [DOCK8 deficiency: clinical and immunological phenotype and treatment options.](#) *J Clin Immunol* 2015 Jan 28. [Epub ahead of print] PMID: 25627830
- Gungor T, et al. [Reduced-intensity conditioning and HLA-matched haemopoietic stem-cell transplantation in patients with chronic granulomatous disease: a prospective multicentre study.](#) *Lancet.* 2014;383:436-48
- Bontant T, et al. [Survey of CMV management in pediatric allogeneic HSCT programs, on behalf of the inborn errors, infectious diseases and pediatric diseases working parties of EBMT.](#) *Bone Marrow Transplant.* 2014;49:276-9
- Angelucci E, et al. [Hematopoietic stem cell transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel.](#) *Haematologica.* 2014;99:811-20
- Fuchs S, et al. [Patients with T⁺/low NK⁺ IL-2 receptor \$\gamma\$ chain deficiency have differentially-impaired cytokine signaling resulting in severe combined immunodeficiency.](#) *Eur J Immunol.* 2014;44:3129-40

EBMT 2015

IEWP business meeting

Monday 23 March 08:00 – 08:50 hrs room 3B/10 (B3 level)

IEWP session

Tuesday 24 March 11:00 – 12:30 hrs Çamlica Hall

11:00 - 11:15	★ Abstract WP020: Treosulfan-based conditioning for Chronic Granulomatous Disease -The European Experience on behalf of the IEWP Speaker: Beatriz MORILLO-GUTIERREZ (United Kingdom)
11:15 - 11:30	★ Abstract WP013: SCT in paediatric AML: Does the type of conditioning regimen matter?- on behalf of the PDWIP Speaker: Giovanna LUCCHINI (United Kingdom)
11:30 - 11:45	★ Abstract WP012: Myeloablative Chemoconditioning for Allogeneic SCT in children with ALL. An EBMT PDWIP Study Speaker: Christina PETERS (Austria)
11:45 - 12:00	★ Abstract WP021: Outcome after stem cell transplantation for severe combined immunodeficiency in Europe: Current results and future perspectives on behalf of the IEWP of the EBMT Speaker: Arjan LANKESTER (The Netherlands)
12:00 - 12:15	★ Abstract WP022: Duke-Eurocord Study on the outcomes of Cord Blood Transplantation for Leukodystrophies Speaker: Jaap Jan BOELEN (The Netherlands)
12:15 - 12:30	★ Abstract WP023: Update SCETIDE data to 2012 Speaker: Nizar MAHLAOU (France)

Future Meetings

How to transplant a patient with PID workshop

Leuven, Belgium April 24th 2015



IEWP Autumn meeting

Seville 16-18th October 2015



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

Significant Collaborations



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