

**Chair: Arjan Lankester**  
**Secretary: Michael Albert**

## Studies

Topic*	Disease Type	Joint
HSCT in LAD	PID	IEWP – PDWP
Outcome of Domino HSCT	PID	IEWP
HSCT in CGD	PID	IEWP
BuFlu vs. TreoFlu in WAS	PID	IEWP – Scetide
HSCT in IFN $\gamma$ R1/R2-D	PID	IEWP – ESID – LASID – CIS ASID – PIDTC – Scetide
HSCT in porphyria	IMD	IEWP – CIBMTR
Outcomes after CBT in IMD	IMD	IEWP – Eurocord
HSCT in LAL-D (Wolman)	IMD	IEWP
Eculizumab for HSCT-TMA		PDWP – IEWP
HSCT in HLH adults	HLH	CMWP – IEWP
UCBT in children with FHLH	HLH	Eurocord – IEWP
Late effects after HSCT for HLH	HLH	PDWP – IEWP

\* If you wish to participate in the data collection for these studies, please contact:  
IEWPebmt@lumc.nl

## Recent Publications on behalf of IEWP

### Hematopoietic stem cell transplantation in patients with gain-of-function signal transducer and activator of transcription 1 mutations. *J Allergy Clin Immunol* 2017

J.W. Leiding\*, S. Okada\*, D. Hagin, M. Abinun, A. Shcherbina, D.N. Balashov, V.H.D. Kim, A. Ovidia, S.L. Guthery, M. Pulsipher, D. Lilic, L.A. Devlin, S. Christie, M. Depner, S. Fuchs, A. van Royen-Kerkhof, C. Lindemans, A. Petrovic, K.E. Sullivan, N. Bunin, S. Sebnem Kilic, F. Arpac, O. de la Calle-Martin, L. Martinez-Martinez, J. C. Aldave, M. Kobayashi, T. Ohkawa, K. Imai, A. Iguchi, C. M. Roifman, A. R. Gennery, M. Slatter, H. D. Ochs, T. Morio\*\*, and T. R. Torgerson\*\*, on behalf of the Inborn Errors Working Party of the European Society for Blood and Marrow Transplantation and the Primary Immune Deficiency Treatment Consortium

### Reticular dysgenesis: international survey on clinical presentation, transplantation, and outcome. *Blood* 2017

M. Hoenig, C. Lagresle-Peyrou, U. Pannicke, L. D. Notarangelo, F. Porta, A. R. Gennery, M. Slatter, M. J. Cowan, P. Stepensky, H. Al-Mousa, D. Al-Zahrani, SY. Pai, W. Al Herz, H. B. Gaspar, P. Veys, K. Oshima, K. Imai, H. Yabe, L. M. Noroski, N. M. Wulffraat, KW Sykora, P. Soler-Palacin, H. Muramatsu, M. Al Hilali, D. Moshous, KM. Debatin, C. Schuetz, EM Jacobsen, A. S. Schulz, K. Schwarz, A. Fischer, W. Friedrich and M. Cavazzana, on behalf of the European Society for Blood and Marrow Transplantation (EBMT) Inborn Errors Working Party

### Treatment of Severe Forms of LPS-Responsive Beige-like Anchor Protein (LRBA) Deficiency by Allogeneic Hematopoietic Stem Cell Transplantation. *J Allergy Clin Immunol* 2017

M. G. Seidel, K. Böhm, F. Dogu, A. Worth, A. Thrasher, B. Florin, A. İkinçioğulları, A. Peters, S. Bakhtiar, M. Meeths, P. Stepensky, I. Meyts, S.O. Sharapova, L. Gámez-Díaz, L. Hammarström, S. Ehl, B. Grimbacher, and A. R. Gennery, on behalf of the Inborn Errors Working Party of the European Group for Blood and Marrow Transplantation

### A risk factor analysis of outcomes after unrelated cord blood transplantation for children with Wiskott-Aldrich syndrome. *Haematologica* 2017

Z. Shekhovtsova, C. Bonfim, A. Ruggeri, S. Nichele, K. Page, A. AlSeraihy, F. Barriga, J.Sánchez de Toledo Codina, P. Veys, JJ Boelens, K. Mellgren, H. Bittencourt, T. O'Brien, P.J. Shaw, A. Chybicka, F. Volt, F. Giannotti, E. Gluckman, J. Kurtzberg, A.R. Gennery, V. Rocha on behalf of Eurocord, Cord Blood Committee of Cellular Therapy and Immunobiology Working Party of the EBMT, Federal University of Parana, Duke University Medical Center and Inborn Errors Working Party of the EBMT

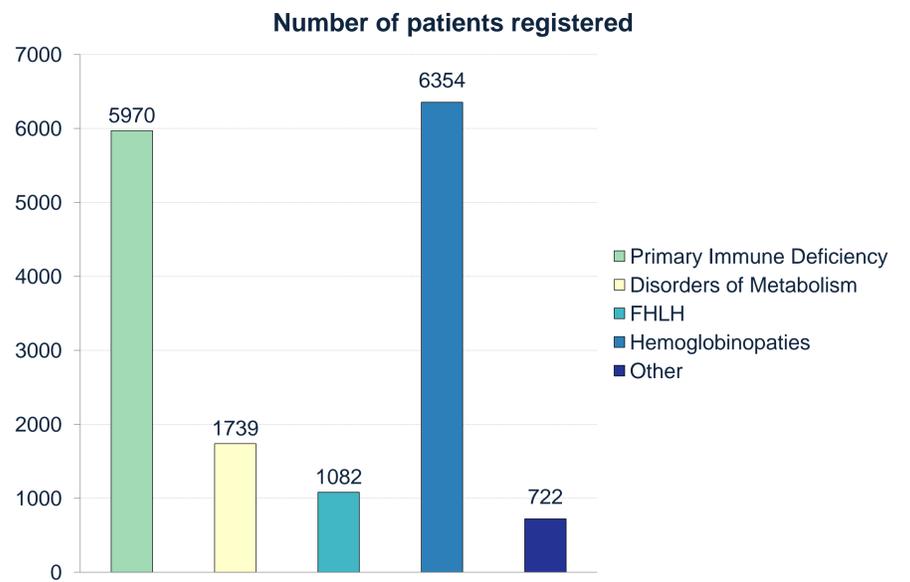
### Long-term follow up of IPEX syndrome patients after different therapeutic strategies: an international multicenter retrospective study. *J Allergy Clin Immunol* 2017

F. Barzagli, L.C. Amaya Hernandez, B. Neven, S. Ricci, Z.Y. Kucuk, J.J. Bleesing, Z. Nademi, M.A. Slatter, E.R. Ulloa, A. Shcherbina, A. Roppelt, A. Worth, J. Silva, A. Aiuti, L. Murguía-Favela, C. Speckmann, M. Carneiro-Sampaio, J. Folloni Fernandes, S. Baris, A. Ozen, E. Karakoc-Aydiner, A. Kiykim, A. Schulz, S. Steinmann, L.D. Notarangelo, E. Gambineri, P. Lionetti, W.T. Shearer, L. R. Forbes, C. Martinez, D. Moshous, S. Blanche, A. Fisher, F.M. Ruummele, C. Tissandier, M. Ouachee-Chardin, F. Rieux-Laucat, M. Cavazzana, W. Qasim, B. Lucarelli, M.H. Albert, I. Kobayashi, L. Alonso, C. Diaz De Heredia, H. Kanegane, A. Lawitschka, JJ. Seo, M. Gonzalez-Vicent, M.A. Diaz, R.K. Goyal, M.G. Sauer, A. Yesilipek, M. Kim, Y. Yilmaz-Demirdag, M. Bhatia, J. Khlevner, E.J. Richmond Padilla, S. Martino, D. Montin, O. Neth, A. Molinos-Quintana, J. Valverde-Fernandez, A. Broides, V. Pinsk, A. Ballauf, F. Haerynck, V. Bordon, C. Dhooge, M.L. Garcia-Lloret, R. G. Bredius, K. Katwak, E.Haddad, M.G. Seidel, G. Duckers, SY. Pai, C.C. Dvorak, S. Ehl, F. Locatelli, F. Goldman, A.R. Gennery, M. J. Cowan, MG Roncarolo, and R. Bacchetta on behalf of the Primary Immune Deficiency Treatment Consortium (PIDTC) and the Inborn Errors Working Party (IEWP) of the European Society for Blood and Marrow Transplantation (EBMT)

## Significant Collaborations



## EBMT Registry



## Lisbon IEWP-events

### Monday March 19<sup>th</sup>

IEWP Business meeting  
7.30 – 9.00  
Room 5 A

### Tuesday March 20<sup>th</sup>

IEWP session  
11.00 – 12.30  
Auditorium V

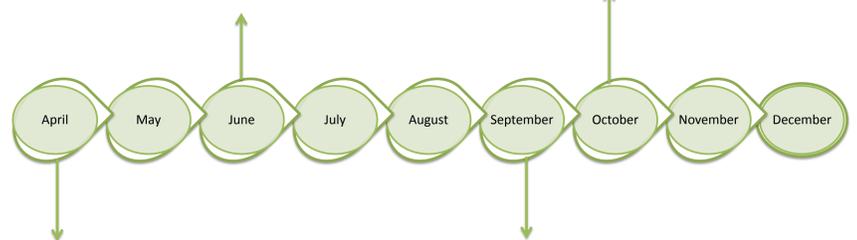
### Program IEWP session:

- 11:00-11:18 Gene Therapy vs SCT for ADA-SCID  
*Bobby Gaspar (UK)*
- 11:18-11:36 SCT in leukocyte adhesion deficiency (IEWP/PDWP study)  
*Shahzad Bakhtiar (Germany)*
- 11:36-11:54 HaploSCT in Hemoglobinopathies  
*Josu de la Fuente (UK)*
- 11:54-12:12 Gene therapy in Hemoglobinopathies  
*Alessandro Aiuti (Italy)*
- 12:12-12:30 Outcomes after CBT in IMD (IEWP/EUROCORD study)  
*Su Han Lum (UK)*

## Future Events 2018

PDWP, IDWP, IEW & Nurses  
group meeting  
Verona June 7-9, 2018

ESID meeting  
Lisbon 24-27 October, 2018



Workshop on HSCT for PID in adults  
and young adolescents (AYA)  
Munich April 13<sup>th</sup>, 2018

IEWP autumn meeting  
Leiden 28-30 September, 2018

## Contact IEWP

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