



Inborn Error Working Party

Arjan Lankester (chair)
Michael Albert (secretary/co-chair)

Leiden, the Netherlands
Munich, Germany

Studies

Topics	Disease
AYA PID	PID
Haplo PID	PID
Second allo*	PID
Mevalonate Kinase Deficiency *	PID

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

Recent Publication on behalf of IEWP

Hematopoietic Stem Cell Transplantation for DNA Double Strand Breakage Repair Disorders Front Pediatr 2020
Wolska-Kuśnierz Beata, Gennery Andrew R.

Targeted busulfan-based reduced-intensity conditioning and HLA-matched HSCT cure hemophagocytic lymphohistiocytosis Blood Adv2020
Felber M, Steward CG, Kentouche K, Fasth A, Wynn RF, Zeilhofer U, Haunerding V, Volkmer B, Prader S, Gruhn B, Ehl S, Lehmsberg K, Müller D, Gennery AR, Albert MH, Hauck F, Rao K, Veys P, Hassan M, Lankester AC, Schmid JP, Hauri-Hohl MM, Güngör T.

Extended clinical and immunological phenotype and transplant outcome in CD27 and CD70 deficiency Blood 2020
Ghosh S, Köstel Bal S, Edwards ESJ, Pillay B, Jiménez Heredia R, Erol Cipe F, Rao G, Salzer E, Zoghi S, Abolhassani H, Momen T, Gostick E, Price DA, Zhang Y, Oler AJ, Gonzaga-Jauregui C, Erman B, Metin A, Ilhan I, Haskologlu S, Islamoglu C, Baskin K, Ceylaner S, Yilmaz E, Unal E, Karakucuk M, Berghuis D, Cole T, Gupta AK, Hauck F, Kogler H, Hoepelman AIM, Baris S, Karakoc-Aydiner E, Ozen A, Kager L, Holzinger D, Paulussen M, Krüger R, Meisel R, Oommen PT, Morris E, Neven B, Worth A, van Montfrans J, Fraaij PLA, Choo S, Dogu F, Davies EG, Burns S, Dücker G, Becker RP, von Bernuth H, Latour S, Faraci M, Gattorno M, Su HC, Pan-Hammarström Q, Hammarström L, Lenardo MJ, Ma CS, Niehues T, Aghamohammadi A, Rezaei N, İkinciogullari A, Tangye SG, Lankester AC, Boztug K.

Hematopoietic Stem Cell Transplantation for Combined Immunodeficiencies, on Behalf of IEWP-EBMT Front Pediatr 2020
Neven B, Ferrua F.

Hematopoietic cell transplantation in chronic granulomatous disease: a study of 712 children and adults Blood 2020
Chiesa R, Wang J, Blok HJ, Hazelaar S, Neven B, Moshous D, Schulz A, Hoenig M, Hauck F, Al Seraihy A, Gozdzik J, Ljungman P, Lindemans CA, Fernandes JF, Kalwak K, Strahm B, Schanz U, Sedlacek P, Sykora KW, Aksoylar S, Locatelli F, Stepensky P, Wynn R, Lum SH, Zecca M, Porta F, Taskinen M, Gibson B, Matthes S, Karakucuk M, Hauri-Hohl M, Veys P, Gennery AR, Lucchini G, Felber M, Albert MH, Balashov D, Lankester A, Güngör T, Slatter MA.

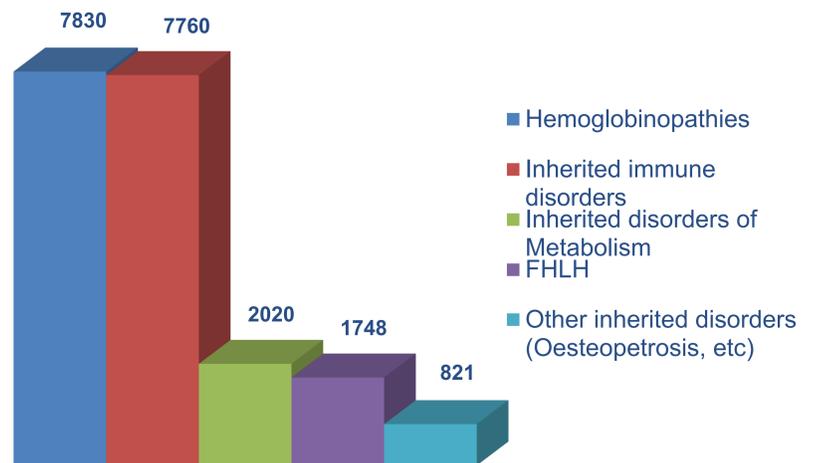
Long-term outcome of LRBA deficiency in 76 patients after various treatment modalities as evaluated by the immune deficiency and dysregulation activity (IDDA) score J Allergy Clin Immunol 2020
Tesch VK, Abolhassani H, Shadur B, Zobel J, Mareika Y, Sharapova S, Karakoc-Aydiner E, Rivière JG, Garcia-Prat M, Moes N, Haerynck F, Gonzales-Granado LI, Santos Pérez JL, Mukhina A, Shcherbina A, Aghamohammadi A, Hammarström L, Dogu F, Haskologlu S, İkinciogullari AI, Köstel Bal S, Baris S, Kilic SS, Karaca NE, Kutukculer N, Girschick H, Kolios A, Keles S, Uygun V, Stepensky P, Worth A, van Montfrans JM, Peters AMJ, Meyts I, Adeli M, Marzollo A, Padem N, Khojah AM, Chavoshzadeh Z, Avbelj Stefanija M, Bakhtiar S, Florin B, Meeths M, Gamez L, Grimbacher B, Seppänen MRJ, Lankester A, Gennery AR, Seidel MG;

Significant Collaborations



EBMT Registry

Number of patients registered



IEWP sessions at EBMT anual meeting

Tuesday, March 16

- 11:00 - 11:15 Antibody-based conditioning in SCID and beyond *Agnieszka Csechowiz*
- 11:15 - 11:30 Clinical and immunological outcome after HSCT in SCID, a SCETIDE cohort study *Arjan Lankester*
- 11:30 - 11:45 A prospective outcome study in profound combined immune deficiency *Stephan Ehl*
- 11:45 - 12:00 Advances in stem cell therapy in metachromatic leukodystrophy *Valeria Calbi*
- 12:00 - 12:15 Q&A
- 16:15 - 17:30 Inborn errors oral session

Wednesday, March 17

- 09:15 - 10:30 The expanding field of cellular therapies in inborn errors of immunity (IEI)

IEWP Future Events 2021



Contact IEWP

- Arjan Lankester Chair A.Lankester@lumc.nl
- Michael Albert Secretary/co-chair Michael.Albert@med.uni-muenchen.de
- Tiar Sirait Study Coordinator T.Sirait@lumc.nl