

Studies

Topic*	Disease type	Joint
AYA PID	PID	
Haplo in PID	PID	
ICF Syndrome	PID	
IFNg R1 R2	PID	
Hypomorphic RAG	PID	IEWP – Scetide
CD27/CD70 deficiency	PID	IEWP – ESID

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

Recent Publications on behalf of IEWP

Hematopoietic stem cell transplantation for CD40 ligand deficiency: results from an EBMT/ESID-IEWP-SCETIDE-PIDTC Study. *J Allergy Clin Immunol* 2019

Ferrua, F., S. Galimberti, V. Courteille, M. A. Slatter, C. Booth, D. Moshous, B. Neven, S. Blanche, A. Laberko, A. Shcherbina, D. Balashov, E. Soncini, F. Porta, H. Al-Mousa, B. Al-Saud, H. Al-Dhekri, R. Arnaout, R. Formankova, Y. Bertrand, A. Lange, J. Smart, B. Wolska-Kusnierz, V. M. Aquino, C. C. Dvorak, A. Fasth, F. Fouyssac, C. Heilmann, M. Hoenig, C. Schuetz, J. Kelečić, R. G. M. Bredius, A. C. Lankester, C. A. Lindemans, F. Suarez, K. E. Sullivan, M. H. Albert, K. Kalwak, V. Barlogis, M. Bhatia, V. Bordon, W. Czogala, L. Alonso, F. Dogu, J. Gozdziak, A. Ikinciogullari, G. Kriván, P. Ljungman, I. Meyts, P. Mustillo, A. R. Smith, C. Speckmann, M. Sundin, S. J. Keogh, P. J. Shaw, J. J. Boelens, A. S. Schulz, P. Sedlacek, P. Veys, N. Mahlaoui, A. Janda, E. G. Davies, A. Fischer, M. J. Cowan and A. R. Gennery

Risk Factors Affecting Outcome of Unrelated Cord Blood Transplantation for Children with Familial Haemophagocytic Lymphohistiocytosis *Br J Haematol* 2018

Furtado-Silva, J. M., A. Paviglianiti, A. Ruggeri, J. J. Boelens, P. Veys, A. A. Ahmari, M. Zecca, F. Locatelli, G. Michel, F. Volt, C. Kenzay, P. Sedlacek, K. Rao, A. Lankester, E. Gluckman, and V. Rocha. On behalf of Eurocord/ Monacord, Cord Blood Committee of Cellular Therapy, Immunobiology and Inborn Errors Working Parties of the European Blood and Marrow Transplant Group.

Outcome of Domino Hematopoietic Stem Cell Transplantation in Human Subjects: An International Case Series. *J Allergy Clin Immunol* 2018

Belderbos, M. E., A. R. Gennery, C. C. Dvorak, H. J. Blok, D. J. Eikema, J. M. F. Silva, P. Veys, B. Neven, R. Buckley, T. Cole, M. J. Cowan, W. S. Goebel, M. Hoenig, C. Y. Kuo, E. R. Stiehm, R. Wynn, and M. Bierings. On behalf of Inborn Errors Working Party of the European Group for Blood and Marrow Transplantation and the Primary Immune Deficiency Treatment Consortium

Hematopoietic Stem Cell Transplantation as Treatment for Patients with Dock8 Deficiency. *J Allergy Clin Immunol Pract* 2018

Aydin, S. E., A. F. Freeman, W. Al-Herz, H. A. Al-Mousa, R. K. Arnaout, R. C. Aydin, V. Barlogis, B. H. Belohradsky, C. Bonfim, R. G. Bredius, J. I. Chu, O. C. Ciocarlie, F. Dogu, H. B. Gaspar, R. S. Geha, A. R. Gennery, F. Hauck, A. Hawwari, D. D. Hickstein, M. Hoenig, A. Ikinciogullari, C. Klein, A. Kumar, M. R. S. Iversen, S. Matthes, A. Metten, B. Neven, S. Y. Pai, S. H. Parikh, C. Picard, E. D. Renner, O. Sanal, A. S. Schulz, F. Schuster, N. N. Shah, E. B. Shereck, M. A. Slatter, H. C. Su, J. van Montfrans, W. Woessmann, J. B. Ziegler, and M. H. Albert. On behalf of Inborn Errors Working Party of the European Group for Blood and Marrow Transplantation and the Primary Immune Deficiency Treatment Consortium

Long-term follow-up of IPEX syndrome patients after different therapeutic strategies: An international multicenter retrospective study *J Allergy Clin Immunol* 2018

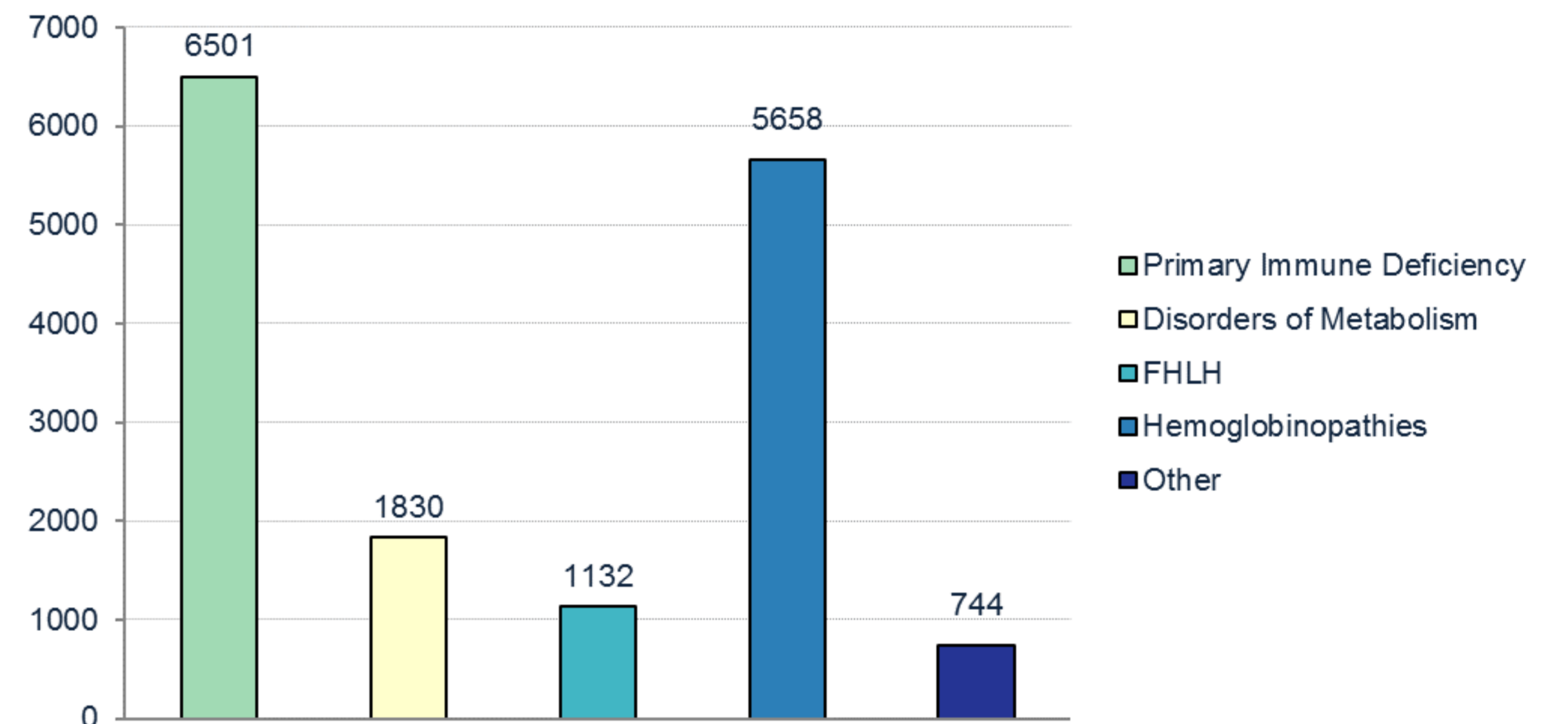
Barzaghi, F., L. C. Amaya Hernandez, B. Neven, S. Ricci, Z. Y. Kucuk, J. J. Bleesing, Z. Nademi, M. A. Slatter, E. R. Ulloa, A. Shcherbina, A. Roppelt, A. Worth, J. Silva, A. Aiuti, L. Murguía-Favela, C. Speckmann, M. Carneiro-Sampaio, J. F. Fernandes, S. Baris, A. Ozen, E. Karakoc-Aydiner, A. Kiykim, 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. Ruemmele, 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, J. J. 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. Dhooze, M. L. Garcia-Lloret, R. G. Bredius, K. Kalwak, E. Haddad, M. G. Seidel, G. Duckers, S. Y. Pai, C. C. Dvorak, S. Ehl, F. Locatelli, F. Goldman, A. R. Gennery, M. J. Cowan, M. G. Roncarolo and R. Bacchetta. On behalf of 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

Number of patients registered



Frankfurt IEWP events

Monday March 25th

IEWP Business meeting
7.30 – 9.00
Analog 1 + 2

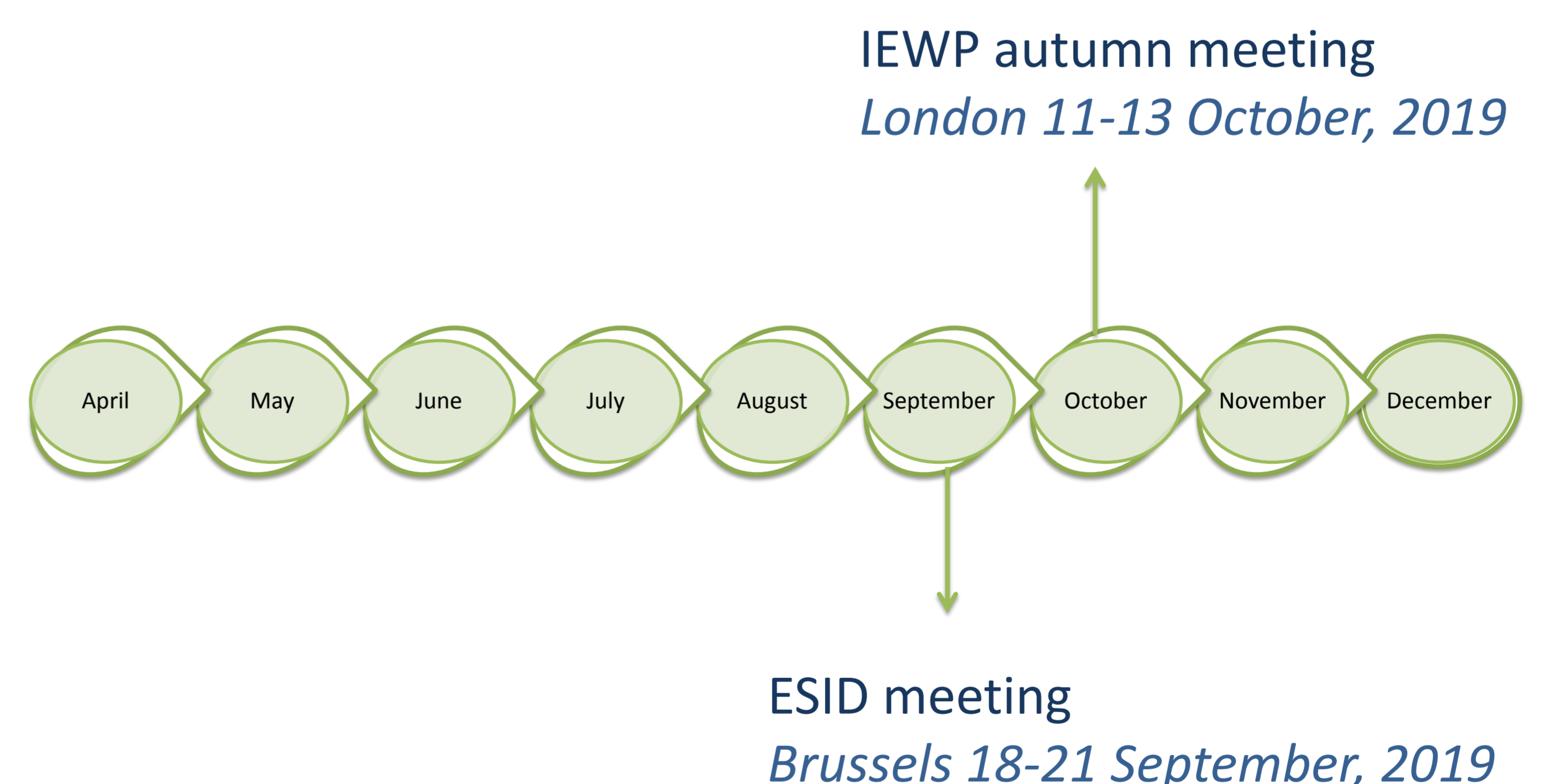
Tuesday March 26th

IEWP session
11.00 – 12.30
Panorama 3

Program IEWP session:

- 11.00 – 11.15 Gene therapy in MPS-1
Maria Ester Bernardo
- 11.15 – 11.30 Busulfan and treosulfan based conditioning in WAS
Michael Albert
- 11.30 – 11.45 Outcome of HSCT in children and adults with CGD
Robert Chiesa
- 11.45 – 12.00 Clinical and immunological outcome of HSCT in SCID
Arjan Lankester
- 12.00 – 12.15 Treatment of athymia with thymus allografting
Graham Davies
- 12.15 – 12.30 T-cell gene editing in CD40 ligand deficiency
Pietro Genovese

Future Events 2019



Contact IEWP

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