

Paediatric Working Party

Membership

Dr Mary Mathias	Chair, London (GOSH)
Dr Jeanette Payne	Secretary, Sheffield
Jayanthi Alamelu	London (Evelina)
Neha Bhatnagar	Oxford
Tina Biss	Newcastle
Jayashree Motwani	Birmingham
John Grainger	Manchester
Simone Greene	Hull
Simone Stockley	Nottingham
Anne Kelly	Addenbrookes

Meetings

Since the last AGM the PWP has held 3 teleconference meetings.

Summary of activities

Immune Tolerance Induction (ITI) consensus update post emicizumab commissioning.

This has been published in Haemophilia and will be available on the UKHCDO website.

Emicizumab PUP and MTP data.

Given the delay in starting the NHD PUP registry data collection caused by the enquiry, the Chair asked the NHD to request a 'snap-shot' of data on use of emicizumab in children under 12 years with and without inhibitors. This has been collated by Liz Chalmers and was presented by Mary Mathias as a poster at EAHAD 2021. No children under 4 months had received emicizumab. There was considerable centre variation in the use of emicizumab for non-inhibitor children.

A national advisory group/MDT has been running smoothly with 3 monthly teleconferences with terms of reference for discussion and recording of outcome. Calls for cases to discuss are sent out to paediatric treating centres prior to the dates with a data pro forma. The feedback has been positive in terms of support for centres. We are also using this forum to collect data about inhibitors arising in children on emicizumab.

PUP registry on the NHD. This has now been established and will hopefully be a valuable prospective data collection tool. Centres recording data about early exposures will be prompted for follow-up information to 50 EDs

PWP membership at other UKHCDO meetings: Inhibitor WP, DMWP, DAG and Adverse events panel

Other projects: data collection has inevitably been delayed by the COVID epidemic and prior to that by uncertainty around the NHD research database in terms of requirement for consent. We will now continue to pursue:

- a. Inhibitors in Haemophilia B- national data on under 18s with inhibitors in haemophilia B have been collated by Dr Bhatnagar with a plan for submission as a Letter to the Editor of Haemophilia.

- b. Bleeding phenotype and treatment in children with severe FVII deficiency (Dr Biss kindly completing this work started by Dr Bhatnagar).

Given the increasing concern of the PWP members that recombinant VWF concentrate is very unlikely to receive a paediatric license before 2026, we are pursuing the possibility of off-license use, which has already been agreed for named patients in one of the devolved nations.

Dr Mary Mathias
Chair, Paediatric Working Party
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