

Paediatric Working Party

Membership

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| Dr Elizabeth Chalmers | Chair, Glasgow |
| Dr Jeanette Payne | Secretary, Sheffield |
| Dr Jayanthi Alamelu | London (Evelina) |
| Dr Neha Bhatnagar | Oxford |
| Dr Tina Biss | Newcastle |
| Dr Mary Mathias | London (GOSH) |
| Dr Jayashree Motwani | Birmingham |
| Dr Mike Richards | Leeds |
| Dr Oliver Tunstall | Bristol |

The working party membership was renewed in October 2017.

Meetings

Two per annum with additional telecoms as required.

Last meeting: September 2018.

Summary of activities.

1. ITI Outcome data

- ***Retrospective follow up of Immune tolerance therapy in the UK (2003-2015)***

Retrospective outcome data on the results of immune tolerance therapy in the UK (2003-2015 inclusive) has been collected from 8 UK CCCs treating children with inhibitors. Results of this analysis have been presented at the AGM previously & a manuscript is currently in preparation.

- ***Management & outcome of Immune tolerance using a standardised regimen (with inhibitor WP) - ongoing data collection in collaboration with IWP***

A standard ITI protocol based on the current UKHCDO Guideline was agreed and commissioned previously. New inhibitors are reported to the NHD and prospective data collection on ITI outcomes has now been ongoing for almost 2 years. An update of the data collected so far will be presented at the AGM.

2. Intracranial haemorrhage in inherited bleeding disorders

Retrospective data collection (2003-2015) on cases of ICH occurring in children <16yrs of age in the UK has been completed and has been published in the journal Haemophilia. The data highlight the continuing risk of this complication in very young children with

severe bleeding disorders. Prospectively data collection on ICH in both children and adults has been added to the NHD adverse events reporting scheme.

The WP is also in the process of setting up a PUP registry via the NHD which will collect data on early exposure to FVIII and FIX in children with severe haemophilia. Data is also being collated in relation to the following areas:

- Inhibitors in Haemophilia B
- Outcomes in moderate Haemophilia
- Bleeding and treatment in children with rare coagulation disorders

The PWP also intends to pilot a national advisory group which will aim to act as a forum for advice on the management of children with inherited bleeding disorders. Further information on this will be circulated shortly.

Dr Elizabeth Chalmers
Chair, Paediatric Working Party
October 2018