

# Inhibitor Working Party

## *Membership*

Dan Hart	Chair
Kate Talks	Secretary
Liz Chalmers	
Peter Collins	
Georgina Hall	
Charlie Hay	
Ri Liesner	
Mike Makris	
Ben Palmer	
Charles Percy	
Anne Riddell	

## *Meetings*

The group met once face to face with multiple teleconferences to progress different projects. The group's membership was reconstituted in April/May 2017. We are very grateful for the time and work of those leaving the group, notably Savita Rangarajan, Mike Williams and Trevor Baglin.

## *Activities*

### **Revised ITI guideline publication**

Since last year, our revised UKHCDO ITI guideline has been published in the Haemophilia journal (Collins et al, Haemophilia 2017; 23:654-659) and acknowledged at a number of open meetings. There have been only 2 requests for a virtual MDT to discuss challenging inhibitor cases. Colleagues are reminded that this remains on offer, accessible through Lynne Dewhurst at NHD and convened as a teleconference by Inhibitor or Paeds working party chair with additional representation by either adult or paediatric treaters depending on the case.

### **Laboratory survey**

We are grateful to the NEQAS team who were able to disseminate our laboratory survey to 119 laboratories, with responses returned from 62% of the centres. This questionnaire was designed to understand the national heterogeneity of inhibitor assay performance and reporting as well as build additional links with laboratory lead scientists. Anne Riddell represents our laboratory colleagues on the Inhibitor WP and facilitated the questionnaire design and delivery with NEQAS. The preliminary data analysis was presented in poster format at ISTH 2017 by Ian Jennings (NEQAS), and the Inhibitor WP authorship team are working on a final draft for publication.

### European Medicines Agency Meta-analysis publication

Dr Brigitte Keller-Stanislawski (Paul Ehrlich Institute) was the academic lead for the tri-partite meta-analysis of Pednet, FranceCoag and UKHCDO raw data pertaining to brand specific inhibitor risk. Dr Stanislawski has submitted the completed manuscript for publication on 2 occasions on behalf of all collaborators but as yet without success. We hope to overcome some recent bureaucratic hurdles to enable this large data set to be placed in the public domain in the coming months.

### New agent Policy Working Groups

Both Obizur and Emicizumab have established Policy working groups led by Dan Hart and Peter Collins respectively. Each has additional clinical contributors and patient representation. The commissioning process is currently proceeding through the NICE Commissioning Support Program before returning for Policy confirmation and commissioning consideration.

### THUNDER project (Treatment of Haemophilia, Unmet Need and Disease Epidemiology in the Real world)

Prof Hay presented the THUNDER data as an oral presentation at ISTH 2017. This has been a considerable amount of work for the NHD team and IWP membership. It is a collaboration with Roche on an area of mutual interest, to define the treatment burden in both inhibitor and non-inhibitor setting. It scrutinises our real-world data via NHD with an ever increasing richness of patient reported outcome measures, notably Haemtrack records including treatment details, bleed rates, sites of bleeds, pain scores, Haemophilia Joint health score reporting and interruption of planned daily activity. These are currently being analysed with a view to publication in early 2018.

### Prospective data collection - Acquired haemophilia A and PUPs

These prospective data collections continue. Longitudinal follow up in both clinical scenarios (Acquired Haemophilia A and Previously Untreated Patients with Severe Haemophilia) will generate important data sets to clarify outcomes and support future commissioning as well as ensure equity of treatment access and guideline adherence. Revision of the adverse event reporting portal for inhibitors has delayed its launch until this AGM

Dr D Hart  
Chair, Inhibitor Working Party  
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