# Inhibitor Working Party

### *Membership*

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

The Inhibitor Working party have formally met face-to-face twice in year with multiple informal interactions on subjects outlined below.

Subsequent to recombinant porcine FVIII, Susoctocog alpha (Obizur®) NHS England Clinical Commissioning approval for Acquired Haemophilia A (AHA), Obizur® is now available for clinical use. Prospective AHA clinical data collection continues and the UKHCDO Data Analysis Group has agreed a collaboration with a Public Health Trainee (Louise Gray), to analyse the first 20 UK cases of Obizur® use. This is a prelude to analysis of the continued prospective data collection of all Acquired Haemophilia A over the past 3 years. NHD management is actively addressing how to support this analysis to be overseen by Charles Percy and Dan Hart.

Successful clinical commissioning of Emicizumab (Hemlibra®) for both inhibitor and non-inhibitor indications has occupied much of the committee's time in year. Re-drafting of consensus Immune Tolerance Induction has been under consideration by both Inhibitor and Paediatric Working Parties, informed by latest international perspectives from the ISTH 2019 meeting. Finalisation of this proposal will be ready for and discussed at the Annual General Meeting. Prospective data collection continues for both ITI and chronic inhibitor indication Emicizumab use under the leadership of Liz Chalmers and Georgina Hall respectively. There is likely to be a European, investigator-initiated study of Emicizumab in ITI led by Elisa Mancuso (Milan), albeit with some subtle differences in protocol compared to the UK consensus ITI guidance. Centres may wish to consider this study.

Mike Makris has continued to update the working party about the detail and limitations of the Roche quarterly Emicizumab safety announcements. Various members have continued to actively lobby Roche on multiple fronts for a study of Emicizumab use in first year of life, with recent announcement of an agreed observational study in first year of life, with Peter Collins being a co-lead investigator with Karin Fijnvandraat (Amsterdam). Study protocol detail and timelines are awaited. A UK specific registry of Emicizumab use is in final approvals, led by Charles Hay on behalf of the NHD.

Anne Riddell continues to communicate with comprehensive care centre service-laboratory groups to facilitate set up and availability of specific assays necessary for monitoring patients receiving either Obizur or Emicizumab.

A European & Canadian large cohort genome sequence study to assess high titre inhibitor risk in severe Haemophilia A has been supported in principle by the working party, pending securing

funding. Dan Hart currently leads attempts to construct a funding consortium for this GENESIS study.

The working party are grateful to National Haemophilia Database staff for their continued support to deliver the multiple work streams detailed above.

Dr D Hart
Chair, Inhibitor Working Party
October 2019