

## **Chairperson's Report 2018**

Welcome for the third time to the QEII Centre in London for the 2018 Annual General Meeting of the UKHCDO. The two previous AGMs have been very well attended compared to the preceding meetings held outside London and we thought a larger suite of rooms in this setting would be an ideal venue in which to celebrate the 50<sup>th</sup> anniversary of the foundation of the National Haemophilia Database. We have invited number of past and retired UKHCDO members to join us and some have accepted; we very much hope they enjoy the educational and social aspects of the meeting.

We have followed the same format as previous years with one or two small changes; we will have an educational day on the Thursday, aiming to cover many current topic issues of scientific and clinical interest. These sessions will be open to all members, our invited past members, guests, Sponsors and some trainees if numbers allow. The Friday sessions will start with an open presentation of the annual statistics and after morning coffee break we will have a business AGM, closed to Members and invited guests only.

The 3<sup>rd</sup> year of my current term as UKHCDO Chair has again been demanding and challenging but I continue to be supported by my close colleagues at Great Ormond Street Hospital Haemophilia Centre who shoulder the clinical burden and I am extremely grateful and lucky to have them as my team-mates.

When I wrote my report last year the announcement by the Prime Minister that there would be a Public Inquiry to investigate treatment-related infection of haemophiliacs and others in the 70's-90's was relatively recent news. We were still waiting to hear which Government department would lead the Inquiry and what the full remit would be. Twelve months on the process is underway; the terms of reference have been developed and published, the preliminary hearings have been heard and the Inquiry is onto the gathering evidence phase. The Inquiry team are making arrangements to visit the National Haemophilia Database where a large volume of archived material is held and they also wish to access relevant parts of the electronic database. The NHD is working with current haemophilia treaters to make sure that the data held is as comprehensive as possible and meanwhile all Trusts with Haemophilia Comprehensive Care and Treatment Centres have been asked by the Inquiry office to search for any relevant documentation at their hospitals that should be disclosed as evidence to the Inquiry Investigators. We are well aware that these current exercises are putting a real burden on our stretched Services but we know that as an Organisation we all recognise the devastating effect that these infections have had on patients and their families and we are all totally committed to helping the Inquiry reach a fully-informed conclusion.

At the same time as at looking back to the misery caused by the transfusion transmitted infections in our patients we have had an enormously exciting year in our treatment journey with a new product available to treat our most difficult patients – those with resistant inhibitors. Emicizumab or Hemlibra has been licensed and received NHS approval to be used and many patients and clinicians have already seen immense benefit with very significant reductions in bleed rate and considerable improvements in quality of life. The pathway to successful adoption of NHSE Policy is a convoluted one but we received unerring

support from the Lead Commissioner for Haemophilia - Will Horsley – for both the Emicizumab and the other adopted Policy – the use of recombinant porcine factor VIII in the treatment of bleeding in acquired haemophilia. In the coming months we are going to be working on securing the use of Emicizumab for non-inhibitor patients with an additional Policy and we are hopeful that it is not too long before we will be doing the same for other novel treatments including additional new molecules and gene therapy for both haemophilia A and B.

Meanwhile our standard haemophilia A products went through a re-tender process in late 2017 which commenced in February 2018. It was a collaborative project between the UKHCDO and the NHS England Clinical Reference Group (CRG) for Haemophilia and, as with previous tenders, we benefitted from considerable expertise and support from Alison Greenwood and Wendy Roach from the NHSE Commercial Medicines Unit. The tender was highly successful in terms of the projected savings to be realised for the NHS; a number of patients had to switch products and we recognise the inconvenience to patients and the time invested by Centre staff in the administration required to switch patients to realise the savings. In 2018 we also tendered for all the other products that are not recombinant factor VIII and IX; modest savings were achieved here.

Over the last year we have seen progress in a number of UKHCDO initiatives and you will hear about a number of them during the AGM. The Data Analysis Group (DAG) is a sub-group of the Data Management Working Party and it meets by teleconference monthly. The main role of this Group is the assessment and prioritisation of requests for data from a number of sources, many of which generate income which supports the running of the NHD. We have completed reports requested by a number of commercial companies and abstracts and publications have resulted from several of these.

The Clinical Studies Group has been in existence for 2-3 years and has partnered with the James Lind Alliance to establish a Priority Setting Partnership on Bleeding Disorders with the aim of identifying the most important research areas going forwards. The James Lind Alliance runs the Partnership with well established and validated methodology and brought together patients, carers and health and social care professionals to identify the important unknowns in diagnosis and treatment in the field of bleeding disorders. It has now completed its work and the three highest ranked research priorities in inherited bleeding disorders were: 1. How can we balance the risk and benefit of antithrombotic (blood thinning) treatment for cardiovascular disease (including heart attacks and strokes) in patients with bleeding disorders?; 2. How should heavy periods be managed in women with bleeding disorders?; 3. What are the most effective treatments for acute and chronic pain in people with haemophilia? The full list of the top 10 priorities can be found at <http://www.jla.nihr.ac.uk/top-10-priorities/>.

Last year during the AGM we introduced the format for the 2018/9 Peer Review process and I am delighted to say that our partnership with the West Midlands Quality Review Service has developed to the 'almost-there' stage and the 1<sup>st</sup> review using these high-quality robust standards will happen in the same month as the AGM. All reviews should be completed by AGM 2019 when we should aim to have a detailed discussion on how the results of the Peer Review can help to shape our Services going forward. I would like to thank John Hanley for

his sterling efforts in Chairing the Peer Review Working Party and to the other members on the Group. We also had considerable support from nursing, physio and other colleagues and patient representatives and I would like to thank them for their help with this initiative and other UKHCDO work streams.

Finally I would like to express my gratitude for the continuing support of the rest of the Executive Committee; Professor Peter Collins as Vice Chair; Dr Kate Talks as Secretary and Dr Pratima Chowdary as Treasurer. Both Pratima and I are up for re-election for a 2<sup>nd</sup> 3 year term; the outcome of this is not finalised at the time I am writing this report. On behalf of the Executive and all members I would also like to thank Professor Charlie Hay for his sustained efforts in running and managing the National Haemophilia Database and all the other members of NHD staff including the statisticians for their hard work in supporting the UKHCDO. Special thanks go to Lynne Dewhurst for her ongoing hard work which has underpinned the continued achievements of the NHD. Lastly – and not at all least - is considerable personal gratitude to Sarah Rooney without whose support the role as UKHCDO Chair would not be remotely manageable.

Dr R Liesner  
UKHCDO Chair 17/10/18