

Chairperson's Report

It is the time of year again for me to summarize and give my perspectives on UKHCDO and National Haemophilia database (NHD) activities over the past 12 months and for the near future. We are having the Annual General Meeting in London again because a straw poll of members showed that to be the most convenient of three UK sites suggested and we are very keen to ensure that the increased attendance figures that we have seen over the last few years continues. We are at a new venue this year and we are hopeful that it will be as good as or better than the QEII Centre in Westminster.

I was re-elected as UKHCDO Chair for a 2nd term at last year's AGM and have now done 4 years of a possible maximum of 6 years. I have been supported by the same Executive Committee for the past 2 years and the stability has been extremely helpful in dealing with challenges that we have faced over the last year and I am very grateful for the continued support from them all. The Executive Committee has started to regularly remind members at the Advisory Committee meetings that there will have to be changeover of Executive staff over the next 2-3 years and we continue to urge our colleagues to consider applying for these UKHCDO roles as they come up. I believe the rest of the Executive will support me in agreeing that these national roles are stimulating and rewarding and enable those in post to have real influence over haemophilia/bleeding disorder care in the UK. Ultimately our aim is to ensure we deliver world-class haemophilia care to our patients whilst optimising the outputs from the database and our working parties and task forces so that we and the rest of the treating community can learn from the UK experience.

The Public Inquiry into the treatment-related infection of haemophiliacs and other individuals in the 1970's-90's moved into its phase of gathering evidence from those infected and affected in Spring 2019. The Inquiry is expecting to receive well over a thousand written statements and many of these individuals have been giving oral evidence around the UK over the last months. The statements, transcripts and videos of oral evidence have graphically demonstrated the overpowering misery and devastating long-term impact that the infections have had on the patients and their families and as an Organisation we remain totally committed to ensuring we do as much as we can to help the Inquiry establish what happened and reach a fully-informed conclusion. The Inquiry team have had access to all the archived and electronic material held at the National Haemophilia Database and in addition we have had a huge increase in requests from patients and/or their next-of-kin for the information we hold; in some cases we have treatment related data that appears to be no longer available from the hospitals that care/cared for the patients. However we are also aware that we do not have a complete dataset as we only hold what was submitted from the treating hospitals. All our archived data is now available in electronic format and we are responding promptly to all requests that come to us. We also continue to work with current haemophilia treaters to try and trace a small number of individuals who have been lost to follow-up in case they have not been offered testing for transfusion transmitted infection. Since my last report the UKHCDO has been granted Core Participant status by the Chair of the Inquiry which means that we are represented and can engage optimally with the process as it develops over the coming months.

In May 2019 we were informed that the All Party Parliamentary Group (APPG) into Haemophilia and Contaminated Blood had initiated a separate Inquiry into access to treatment looking at the current systems of licensing, procurement, commissioning and prescription of treatments in the UK and they aimed to talk to patients, clinicians and commissioners. We formed a sub-group of members to write and submit a response which we did and one of our colleagues, Pratima Chowdary, gave oral evidence to a group of MPs. We are yet to hear more about the next phase of this Inquiry but we will be communicating further when we hear more. There is no doubt that the information we

submitted to this Inquiry on current treatment practices would have been considerably enhanced if the timing had been delayed by 9-12 months by which time we will have a wealth of detailed data from the 2018/9 Peer Review programme.

Following a period of time preparing for the Peer Review -which involved setting up a Working Party and the development of a close collaboration with the West Midlands Quality Review Service (WMQRS) - the reviews started in late 2018 soon after the last AGM. By the time of this year's AGM, 29 of a planned 37 visits will have been completed. I would like to wholeheartedly thank not only WMQRS for their input and John Hanley for his energetic ongoing efforts in to getting us to this point but also all our UKHCDO members and all the many nurses, physios, social workers, lab scientists, psychologists, managers, patients and carers who agreed to be trained and have participated in what have been comprehensive multidisciplinary reviews of our haemophilia services. Once the reviews are finished in the next few months an important piece of work will commence to take forward the results of the Peer Review and ensure all the hard work helps to shape our Services for the future. For more detail on some of the common themes of concern from the reviews please see John Hanley's report. I firmly believe that this current piece of work will form an excellent foundation for the next review of services in another 4-5 years when further developments could include collection of outcome data for example.

Over the last year we have seen progress in other UKHCDO initiatives and you will hear about some of them during the AGM. There are a number of guidelines which have been finalised and are due for publication soon such as the UKHCDO guidelines for the laboratory measurement of factor replacement therapies in the treatment of congenital haemophilia as well as guidance for the laboratory measurement of patients during treatment with Emicizumab. There is also a rewritten and updated UKHCDO guideline on prophylaxis in haemophilia and updated ITI guidance incorporating Emicizumab for bleed prevention in ITI which is in its final draft stage. The Data Analysis Group (DAG) is a sub-group of the Data Management Working Party and it continues to meet by teleconference monthly. It has continued to receive many requests for data from industry, from working parties and others doing clinical research into patients with bleeding disorders. We have completed a number of reports during the year and abstracts and publications have resulted from some of these.

Last year I discussed the exciting breakthrough in the very difficult treatment journey faced by patients with haemophilia A and resistant inhibitors as Emicizumab / Hemlibra was licensed and received NHS approval to be used in any patient with an inhibitor. During 2019 we have again seen the implementation of new NHSE Policy, this time the use of this drug for severe haemophilia A patients without a current inhibitor. Once again we have received substantial ongoing support from the Lead Commissioner for Haemophilia - Will Horsley - who has helped to bring this Policy through all the required NHSE hurdles with the result that it was available for use for this group of patients from summer 2019. I am aware that we are ahead of many of our European colleagues who are still negotiating with their funding streams and are not yet able to use it for such patients. However, I am sure that all my colleagues in the treating community will agree that we are still finding our way with trying to establish which patients will benefit most from this new class of drugs and when to use it. On the horizon are other novel agents and gene therapies now in phase 2/3 trials and one of our future challenges will be to negotiate the introduction of these into the UK market. More immediately will be a new tender for all products to treat haemophilia A in early 2020. This again will be a collaborative project between the UKHCDO and the NHS England Clinical Reference Group (CRG) for Haemophilia and we again will benefit from considerable expertise and support from Wendy Roach. Alison Greenwood has moved on from her role in blood disorders procurement and after a partnership of many years we gratefully thank her and wish her well in her new NHSE role.

Lastly but definitely not least I wish to discuss the National Haemophilia Database and its staff. The work of the Inquiry and other initiatives has placed an unprecedented burden on the staff and I and the rest of the Executive are hugely grateful to Lynne Dewhurst and all the NHD team and to Charlie Hay for their determined efforts during the year. Rachel Lockwood worked all hours to upload our archive onto the electronic database and the NHD is enhanced as a result. In addition to the Inquiry work it became evident during 2018 that we should to apply to be a 'Research Registry' and between them Charlie Hay, Pratima Chowdary, Peter Collins and Lynne Dewhurst have completed this large piece of work and we have just recently started to electronically consent patients. During the last year the NHD Manager post has been vacant which has added to the burden on the NHS staff, but I am delighted to report that we have now recruited to this post and our new Manager will start in December. There is no doubt that the NHD still requires more staff and we will be looking into that in early 2020. Meanwhile we have recognised that the increased activities and work of the NHD requires an increase in clinical oversight and the UKHCDO Ltd Board agreed to fund an extra 2 PAs for an NHD Co-Chair to support Charlie Hay and introduce some succession planning. We are currently recruiting to that post and should have news by or soon after the AGM. In addition, one UKHCDO Executive position - the Vice Chair held by Professor Peter Collins - is due for replacement or renewal and expressions of interest have been circulated.

Finally, I would like to express my gratitude for the continuing support of my team at Great Ormond Street Hospital Haemophilia Centre who allow me to fulfil this role. Also Sarah Rooney at the NHD - once again she has provided crucial support and assistance to me in this position and for that I remain continually and extremely grateful.

Dr Ri Liesner,
UKHCDO Chair
October 2019