

Cystinosis Foundation UK Conference - The Chairman's Summary (Roy Forsyth)

Thank You All For Attending!

Thank you to all who came along to the Manchester Conference Centre on the 24th September - we had an excellent attendance. As an organisation, it is one of our basic aims to provide education about the condition and its treatments, so this was the main motivation for the event. And it is your participation that gives us the encouragement to keep doing what we do. There hadn't been a UK conference since Nottingham in 2004, so we were well overdue in organising another, although some interim attempts hadn't progressed due to uncertainty of interest.

The venue was chosen based on it being a convenient location for the majority of people, with good transport connections and accommodation that was not too expensive. The Manchester Conference Centre is a dedicated facility so the conference rooms were properly set up which added to the feel of the day. The main lecture theatre we were in was banked so hopefully everyone could see the presenters. The venue also had several smaller rooms, one of which we used for the crèche, and another as a 'breakout room' for the morning adult session. There was also the benefit of a conference facilitator who ran around in the background helping us set things up - this was a great help on the day as there were a number of small chores that needed to be finalised quickly.

Perhaps one of the most important things for a day like this is the food. We enjoyed a good selection for lunch and the children were well catered for too. Hopefully you all had something that met your needs. The chocolate cake looked particularly nice although I never managed to get a slice! We will certainly consider using the Manchester Conference Centre again in the future.

The Presentations

In the morning, after the initial opening of the conference, we split into two groups – one for adults, one for children.

The group for adults was given a presentation by consultant nephrologist and clinical director of the University Hospital Birmingham, Graham Lipkin. The main focus of this talk was on the issues around the transition of children into adult care. The need to look at the development of the care adults with Cystinosis receive was also raised and this will be addressed under the umbrella of the UK Renal Registry.

The children's group had a presentation from William van't Hoff, consultant paediatric nephrologist at Great Ormond Street Hospital, London. This presentation focused on the basics of what the condition is and the current treatments, all very important information for new families coming along to these events. The presentation utilised material from a new book that has been jointly produced by the Cystinosis Foundation (USA) and Orphan Europe, with advice from William van't Hoff himself.

In the afternoon we heard from Raptor Pharmaceuticals and their chief medical officer, Patrice Rioux. This focused on the development of their enteric coated drug called RP103, which could be used as an improved replacement for CystagonTM. Raptor's work has had some trials, the results of which were presented. The Raptor team travelled from America to attend our conference, so we owe them particular thanks for the huge effort in making that journey. Accompanying the Raptor team were researchers who conducted interviews with individual families and made an online electronic survey available for people to complete. The information they have gathered will hopefully help with the development of improved drug treatments in the future. Thank you to all who made the extra effort to participate in these interviews and surveys.

Following the initial sessions, Professor Roz Anderson, of the University of Sunderland, talked about her team's research into "pro drug" therapy. Lisa Frost, whose PhD in this area we are currently funding, was also in attendance. This work at Sunderland has progressed extremely well and shows real promise of producing significant improvements in treatments for Cystinosis. However, these are some time off in the future.

The presentation from Professor Anderson also covered her work studying proteomics, which is funded by the Cystinosis Research Network (USA). In addition, she spoke a little about gene therapy work that she had seen presented at another event, which she felt should be highlighted to us all. This work had previously been presented at the 2010 international conference in Italy and appears promising. Genetic research has been on-going for other conditions for some time. However, this is not a quick or easy path to follow.

Prof Anderson was keen to stress that the various research programmes should be able to work in partnership together, and not in competition, and our event allowed these researchers to meet, hear about each other's work and develop professional relationships.

We then heard from Dr Patrick Harrison of University College Cork, who is involved in genetic therapy research. Dr Harrison explained just how small the defective gene parts he is researching are, and just how difficult this type of research is. As a comparison, 20 years has been spent on researching cystic fibrosis gene therapy and it still has not been cracked. His work is funded by the Cystinosis Foundation Ireland and the Cystinosis Research Foundation (USA). Representatives from Irish Foundation attended our conference and we enjoyed meeting our friends again.

A short Q&A panel session closed the very busy day and our brains were all a little tired after absorbing so much information in such a small space of time. The time constraints of a one day event do mean that attendees are bombarded with information and have little "free" time during the day. However, we really feel this was a worthwhile event.

We must make a final note of thanks to the two organisations that helped fund the conference. First, the Martin Currie Charitable Foundation, who we were introduced to us through our new member, Neil Hutchison. We hope they will be on-going supporters of the Foundation. Secondly, Orphan Europe, who turned up with a strong team of representatives and had a small stand at the conference. They handed out copies of the new booklet on Cystinosis aimed at children and the new board game developed by Cystinosis Foundation USA and Orphan Europe.

We had a creche facility on the day in one of the break out rooms and a local company came in and provided that service for us. All the children seemed to enjoy their time there and the feed back on this was very positive.

Will we do this again? The answer is "yes, definitely"! However, it may not be for a couple of years as there is an international conference expected next year in France.

During registration everyone received a conference brochure and some leaflets containing information about the Cure Cystinosis International Registry (CCIR). For those of you who haven't registered for this yet, we hope you will do so soon. The information you provide about living with Cystinosis will greatly help direct future research in a common direction.

Thank you again to everyone that came along. It was good to meet you and we look forward to seeing you again at the next conference.

Roy Forysth, Chairman, Cystinosis Foundation UK