

UK Medicines for Children Research Network (MCRN)

MCRN/BAPN Nephrology CSG Meeting

Thursday 16th October 2008

Cancer Research Institute, 44 Lincoln's Inn Fields, London, WC2A 3PX

Minutes

In Person:

Moin Saleem (Chair)
Jennifer Blakeburn
Wendy Cook
Sally Feather
Rodney Gilbert
Sally Johnson
Caroline Jones
Ania Koziell
Jenny Newman
Manish Sinha
Mark Taylor
Kjell Tullus
William van't Hoff
Simon Waller
Alan Watson
Nicholas Webb

Laura Pilkington

Apologies:

Jan Dudley
Rodney Gilbert

Introduction

Moin Saleem (MS) opened the meeting and welcomed all in attendance. MS introduced Wendy Cook as the new MCRN consumer representative and Laura Pilkington as the new CSG Administrator. Apologies were noted from Jan Dudley and Rodney Gilbert.

Review of minutes from the previous meeting

The minutes were reviewed by the group and were accepted as being an accurate record of the discussions that took place at the last CSG meeting on Thursday 6th March 2008.

Updates from the MCRN Coordinating Centre

The following updates from the Coordinating Centre were reported:

a) Annual progress report of CSGs

LP informed the group that the Department of Health would be undertaking a three-year progress review of the MCRN in January 2009. The documentation supporting this review has to be submitted to the UKCRN in November and the MCRN are currently working on its content. It was noted that the work of the MCRN CSGs and their role in portfolio development and prioritisation of research topics is one of the key elements of the MCRN.

LP informed the group that each CSG was been asked to produce a report outlining their progress made during the past 18 months (January 2007 to June 2008). LP referred to the report that has been produced for the MCRN/BAPN Nephrology CSG and thanked everyone for their contributions. LP informed the group that elements of each report

would be incorporated into the main body of the review document. It was noted that the Department of Health interview will take place on Tuesday 20th January 2009. ***LP to provide feedback from the review at the next meeting***

b) Feedback from the consumer induction day

JN provided feedback from the consumer induction day to the CSG. The induction day was held on Wednesday 13th June 2008 in London and gave all of the recently appointed parents/carers the opportunity to learn more about the work of the MCRN and their roles within an MCRN CSG. 30 parents out of 36 attended the induction. As part of the day, Jennifer Blakeburn (UK Programme Manager for ERA-NET) and Dr Monica Lakhnpaul (ERA-NET Methodology Consultant) conducted a workshop in order to get a strong consumer input into the ERA-NET PRIOMEDCHILD project. Parents were given the opportunity to suggest topics, which they felt were important from their own experiences. A short training exercise on participating in a Delphi consensus exercise followed. Wendy Cook informed the group that the induction day was very helpful and that she was very keen to get involved with the work of the Nephrology CSG.

Update on studies

a) Nephrotic syndrome – double blind pilot study (MS/NW)

It was noted that data for the original pilot study is still being collected. Kidney Research UK has turned down an application for funding (i.e. £150,000). Funding has therefore been sought from the HTA. The HTA have asked to know why the study failed funding with Kidney Research UK. The HTA have agreed to defer their decision until the end of November to allow enough time for the investigators to provide this information. In the mean time, it was noted that the BAPN have offered some funds to keep the study coordinator in her role. However, the study coordinator has since left and this money has not been used. ***This study is a priority for the CSG***

b) Rare disease registry (MS)

This study has received £350,000 from the Medical Research Council (MRC) UK and £35,000 from Kidney Research UK. This registry will help to inform how children with rare diseases are treated in the future. Nick Black, Chair of the National Clinic Audit Advisory Group will be involved with this project. Other collaborators include Charlie Tomson, Chairman of the renal registry.

c) Does an addition of an ARB maintain GFR in children with CKD on ACEi? (KT)

The group referred to paper 3 of the meeting papers, which detailed a summary of the recent developments that have taken place. The group agreed that there were two main issues: 1) Feasibility and 2) The sourcing of the drugs. Regarding the feasibility issues, members agreed that it would be helpful to know how many centres would be able to sign up to this study. ***The decision was made for the local PI's from each site (i.e. CSG members) to record the number of children between the ages of five and 15 years who would be eligible for inclusion into this study based on the criteria outlined in the paper*** Regarding the sourcing issues, KT is meeting with a pharma company tomorrow. It was noted that the amount of funding required to run this study would be in the region of £800,000 and £1.5 million.

d) Does taurolock compared with heparin reduce the incidence of catheter related infections in paediatric haemodialysis catheters: a double blind randomised control trial (CJ)

It was noted that the meeting with the MCRN went well. ***Feasibility data will need to be provided (i.e. information on the number of new lines each unit inserts each year and the haemodialysis infection rate).* It was agreed that the research leads in each centre should aim to get this data^{**} It was noted that the person interested in running the study was off work but is due to return in November. The placebo will be made at cost price rather than for free. Members agreed that it would be good if Taurlock could be made from the same company as the placebo.

e) Research into the impact of the NICE guideline CG54 on the diagnosis of renal parenchymal defects in childhood (AW)

It was noted that a funding application has been submitted to the HTA and the team are currently awaiting an outcome. The group were informed that a study team from Bristol have put in a similar application for under two years. A research fellow is in post and waiting to start the trial. Updates on the study will continue to be provided

until there is a need for members of the CSG to get more actively involved. The work on this study was commended (MS).

f) CIQ antibodies (MT)

Good progress has been made; several centres have now been approved after many delays. GOSH are currently collecting data.

g) Use of trimethoprim in the prophylaxis of urinary tract infections in babies born after antenatal renal pelvis dilatation (AS)

****This protocol needs to be circulated to the CSG for review****

h) Vitamin D (ergocalciferol) supplementation in children with early chronic kidney disease – a multicentre, randomised, double-blinded, placebo-controlled study

This study was reviewed by the CSG in March 2008. The aim of the study is to determine the prevalence of vitamin D insufficiency and deficiency in Scottish children with Chronic Kidney Disease and to investigate the differences in bone mineral and hormonal metabolism, bone health, bone turnover and markers of extra-skeletal calcification between those with and without hypovitaminosis D. It was noted that an application to secure preliminary funding for this study was not successfully obtained. An application to the EME NIHR programme grants has since been made. ****This study should be logged as a study that will come to the CSG for further review in 2009****

Proposed Studies

a) Rituximab in nephritic syndrome

The group referred to a copy of the protocol and listened to a presentation given by Dr Ania Koziell (Chief Investigator). The aim of this study is to evaluate the efficacy and safety profile of Rituximab in heavily steroid dependent and steroid resistant nephrotic syndrome in children. The trial design will be a prospective, multi-centre, treatment, randomised, open, three parallel arm assignments, safety and efficacy trial. The CSG were very supportive of this study and a decision was made to form a working group to further develop the protocol, definitions, numbers and feasibility. Working group members will need to be decided (CSG members/BAPN study group members) and the trial will need to be registered. Timelines will also need to be considered. ****AK and MS to liaise****

b) Quality of life Questionnaire (AW)

The group referred to paper 4 of the meeting papers. The function of the questionnaire is to audit the Quality of Life (QOL) of children with chronic renal disease in order to explore the impact of treatment modality. It is child friendly and is quick to complete taking approximately 15 minutes. QOL questionnaires are used widely in the USA for liver and heart transplants. CSG members were very supportive of this study and were keen to get involved. ****AW to look at ethnicity questions**** ****AW to circulate a document that members can take to psychology members in their centre to roll out****

Update on ERA-NET PRIOMEDCHILD (JB)

The UK work package of ERA-NET PRIOMEDCHILD was established to conduct a priority setting exercise to inform a common paediatric research agenda and to identify key research topics within medicines for children.

A literature search and a stakeholder survey were conducted (March 2008) that collected information about gaps in the current research/knowledge relating to medicines for children. The research topics from this process were fed into 14 subject specific web-based Delphi surveys, which were disseminated widely across Professional Societies and other European stakeholder groups. After completion of these surveys the study data was analysed. Following the analysis of the first Delphi survey, participants were asked to complete one further survey containing the research topics which did not reach consensus in round one. Each of the research topics in this second survey were modified or reworded where possible using the comments provided from round one.

The total number of research topics that reached consensus and therefore were agreed as priority areas to be taken forward to the common research agenda was 216, 32% of the initial list of topics (14 priorities reached consensus from the Nephrology survey).

It was noted that the MRC (UK) is hosting a 2-day European Forum for ERA-NET PRIOMEDCHILD on 6 -7 November 2008 at the Wellcome Collection Conference Centre in London. The aim of the Forum is to openly discuss the research needs and opportunities in medicines for children by taking account of the outcomes of the priority setting exercise. The discussions at the conference will be vital to inform the remit of the PRIOMEDCHILD European call for research proposals, jointly supported by the national funding bodies. Delegates will represent all stakeholder groups relevant to medicines for children including, paediatricians, scientists from the pharmaceutical industry and academia, funding bodies and charities, consumers, parents/carers) and pharmacists etc. Delegates from the European Medicines Agency (EMA) and World Health Organisation (WHO) will also be in attendance. All CSG Chairs have been invited to attend. ****JB to provide feedback from the ERA-NET Conference at the next meeting****

Development of a Work Plan for 2008-2009

Due to time commitments the group agreed that this item should be carried forward to the next meeting.

Current calls for funding

LP informed the group that all CSGs are being encouraged to identify research priorities and to develop study proposals that are of relevance to medicines for children. LP informed the group that this process should include CSG members approaching people externally about clinical studies and making sure that they are aware of the services that the MCRN can provide. A list of current funding opportunities will be circulated to CSG members before each meeting, as information.

Any other business

AW informed the group that he had been approached by the MCRN Industry Team to review a commercial protocol. No further any other business was reported.

Date, time and location for next meeting

The next face-to-face meeting will take place on Thursday 30th April 2009 between 11.00am and 3.00pm at the Medical Research Council Head Office (MRC), 20 Park Crescent, London, W1B 1AL.

<http://www.mrc.ac.uk/About/Findus/index.htm>

****Refreshments and lunch will be included****