

MCRN/BAPN Paediatric Nephrology CSG Meeting; Monday 15th March 2010 (meeting held at the MRC Head Office, London; 11:00 – 15:00)

Attendees: Moin Saleem (MS), Jan Dudley (JD), Alan Watson (AW), Caroline Jones (CJ), Kjell Tullus (KJ), Leak Krischock (LK), Mark Taylor (MT), Mine Orlu Gul (MOG), Nicholas Webb (NW), Simon Waller (SW), Wendy Cook (WC), Laura Pilkington (LP), Ania Koziell (AK), Amy Farmer (AF), Larissa Kerecuk (LK), Sally Feather (SF), Sally Johnson (SJ), William van't Hoff (WvH).

Apologies: Rodney Gilbert (RG) and Mark Woodward (MW). Manish Sinha (MS) was also not in attendance.

AGENDA ITEM	SUB-TOPIC	KEY DISCUSSION POINTS	ACTION POINT(S)	INITIAL(S)
1. Welcome, introductions and apologies	N/A	The chair opened the meeting and welcomed everyone in attendance. Apologies were noted, as above. It was noted that a Paediatric Urology representative has been appointed to the CSG, Dr Mark Woodward. Unfortunately Dr Woodward was unable to attend the meeting but he plans on attending the meeting in July.	No action required.	N/A
2. Review of minutes from previous meeting and matters arising	N/A	The minutes from the last meeting on 06/11/09 were reviewed by the group and accepted as an accurate recording of the discussions that took place.	No action required.	N/A
3. Updates from the MCRN CC	MCRN 3 rd Annual Conference	This took place on 19/11/09 at the Echo Arena in Liverpool and was a great success attracting over 260 delegates including representatives from the pharmaceutical industry, universities, charities, patient organisations and NHS staff. Plans for the 2010 conference are underway.	No action required.	N/A
	MCRN CSGs and the Paediatric (Non-Medicines) Specialty Group	LP presented an up date about the Paediatric (Non-Medicines) Specialty Group to the CSG. She explained that a document has been drafted looking at how the MCRN CSGs can assist in the development of the Paediatric (Non-Medicines) portfolio. There are three proposals that have been agreed by the Specialty Group and MCRN CSG Chairs' Forum. Proposal 1: Appointment of Specialty Group Members across each of the CSGs. This will ensure bi-directional communication regarding non-medicines activity. Proposal 2 and Proposal 3 relate to those CSGs that have got external funding in place such as the Nephrology CSG. Proposal 2 focuses on the appointment of additional non-medicines	Keep the group up to date with the integration of non-medicines into CSGs.	LP

		positions. Proposal 3 allows the establishment of topic specific groups whose remit will cross the barriers of medicines and non-medicines research. These proposals will become operational over the coming months subject to ratification from the MCRN Board.		
4. Update on consumer activities	N/A	<p>WC has been asked to deliver the opening address at the next International Podocyte Congress, which is going to be held in Bristol in June. This will be the first time this meeting has been held in the UK, and will feature all the best researchers from around the world in this field.</p> <p>WC has also given her support recently to Marieke Van Meel, founder of the European Nephrotic Syndrome Foundation in Holland (www.nephcEurope.eu; website still under construction). ESPN WINS doctors are involved in this project; Professor Moin Saleem and Dr Rachel Lennon are the UK advisors. The aims of the NephcEurope Foundation are as follows: Advisory role from the patients perspective to parents, patients, medical professionals, the pharmaceutical industry and all other stakeholders and those involved in the treatment of patients with Nephrotic Syndrome: ONE STOP SHOP; Fundraising, encouraging, promoting and performing research for all varieties of Idiopathic Nephrotic Syndrome; Organizing, coordinating and chairing of information-gatherings for various stakeholders; Providing and creating sound information on Nephrotic Syndrome e.g. by issuing website & leaflets about Nephrotic Syndrome; Increasing the awareness on a European level of the challenges that patients with Nephrotic Syndrome are faced with during their life; Improving the rights to equal health care standards on a European level for all patients.</p> <p>WC informed Members that there is still an unmet need for parent information e.g. coping with relapses, talking to siblings etc. WC is keen to work with the CSG to develop a study around this. The group suggested that it would be a good idea if WC had a think about what the questions are that need answering and bring them back to the next CSG meeting. Members also thought it would be useful if WC could find out whether or not parents would be supportive of the idea of setting up rare disease support groups attached to each renal unit. WC agreed to take both of these points forward.</p>	<p>Find out if there is enthusiasm from the various parent groups about setting up rare disease support groups attached to each renal unit.</p> <p>Think about what the questions are that need answering re parent information.</p>	<p>WC</p> <p>WC</p>

5. Update on studies in development	ACHIEVE	This is a single centre study running at GOSH. 50 patients have been recruited to date (target = 70 patients). Recruitment will stop towards the end of April/beginning of May and there will be a one year follow-up.	Ask Dr Rukshana Shroff for a summary of the results for the next meeting. Add this item to the agenda for the next meeting.	MS LP
	APPROVE	The first application to the HTA was unsuccessful. The study team are now considering working up a related study on infants with antenatal hydronephrosis and statistical advice re patient numbers etc is being sought. More information should be available for the CSG by the next meeting.	Start thinking about the next steps. Add this item to the agenda for the next meeting.	JD/SJ/KT LP
	ARB Study	KT presented an update to the group ' <i>where are we now?</i> ' The initial application to the HTA was turned down. The HTA gave the following reasons for it being unsuccessful: It was unclear why the study was so expensive; Inadequate involvement of a CTU (statistics were calculated by the ICH statistician, rather than a CTU); Insufficient patient and public involvement; Sample size was too low; Insufficient consideration/research into other similar studies taking place. The group discussed the project again and it was suggested that it might be worth including obesity patients in the study design. It was agreed that KT should continue to work on a new application and to take the issues, raised by the HTA/CSG, in consideration when doing so.	Address the issues raised by the HTA in a new application. Bring a revised version of the protocol to the next CSG meeting. Add this item to the agenda for the next meeting.	KT KT LP
	C1q Antibody Study	This study has been overtaken by the work of the MPGN group in relation to the second RaDaR pilot study.	No action required.	N/A
	Taurolock Study	An updated version of the protocol was circulated prior to the meeting. It was noted that there were some outstanding points regarding sample size that need to be recalculated, as the primary end point is number of catheter days to first infection. CJ has been working on this with Carol Gamble, one of the statisticians from the MCRN. CSG Members were asked to consider the following points and feedback any comments/suggestions to CJ after the meeting: Who could act as PI at the centres who are willing to participate; What resources would each centre require to run the study in terms of administration/ nursing etc;	Read the proposal and send any comments to CJ. Suggestions re PIs at each of the participating centres to be sent to CJ. Each centre to give CJ an idea of costings re nurse time.	CSG Members CSG Members CSG Members

		Recording in CRF basic data e.g. FBC; Exit site infection; CRP etc (could be collected retrospectively); Monthly bloods to be collected for bacterial DNA from central line; If the patient has a CRBSI then at that point data will need to be collected prospectively. The CRF will include clinical, laboratory and treatment data. CJ was advised to speak to Ruth Gilbert, CI of CATCH, and seek her input as well.	Speak to Ruth Gilbert, CI of the CATCH study.	CJ
	Rituximab Study	AK presented an update to the group. There has been one working group meeting to date. Members in the working group include: MS, NW, KT RG and Sally Hulton. A protocol has been drafted and has gone through the BAPN, a CTU and an R&D Office. Roche are happy to fund the drug but not the study and this funding is subject to the study being linked with adults. The group discussed the study and it was agreed that the next steps should involve 1) convening another working group meeting as soon as possible (via teleconference) 2) Speaking to Roche again to see if they would be prepared to sponsor the study as opposed to just funding the drug, as this would help get things moving more quickly 3) Circulate the protocol to the CSG.	Set up another working group meeting; speak to Roche and circulate the protocol to the CSG for formal review.	AK
6. Update on funded/portfolio studies	Nephrotic Syndrome	This is a HTA funded study. Site Specific Information forms are in progress and some have been approved; each unit will be shown how to enter the study data and have the process about how it will all work explained to them.		
	RaDaR	A <i>strategy</i> has been proposed to improve the care of patients with rare kidney diseases in the UK. It addresses issues of equality of access to services, care pathway design, improving productivity and quality of care, empowerment of patients and their carers, audit and research. It offers a system to provide expert advice to commissioners. The strategy applies to all children and adults with rare kidney diseases, and is consistent with the recent recommendations for transition and transfer from paediatric to adult care from the Renal Association and the British Association for Paediatric Nephrology, endorsed by the Royal College of Physicians of London. Although designed for kidney patients it has the potential for generic utility beyond adult and paediatric nephrology. <i>Disease-specific working groups</i> will be established for each disease or group of diseases. These groups will develop proposals	Circulate strategy document with minutes.	LP

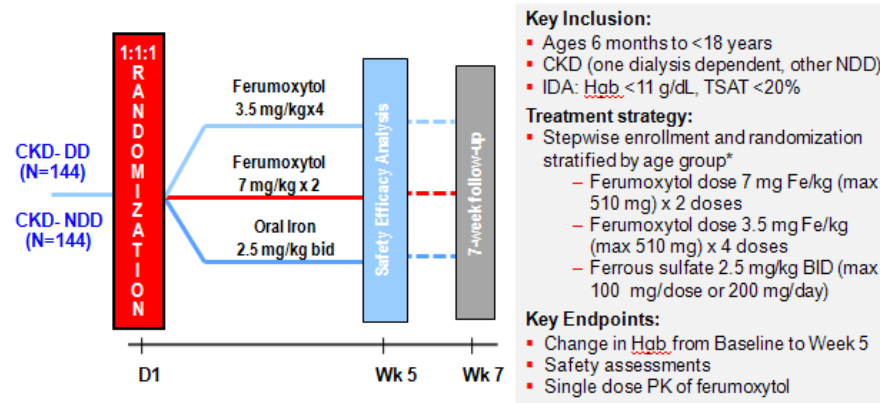
		for the seamless integration of diagnostic and treatment services, audit and research, patient information and empowerment. The CSG were keen for these working groups/leads to be ratified by the CSG before set-up. <i>Care pathways</i> will be developed, which recognise: the tension between the convenience of treatment near home, and the need to access highly specialised services, which might mean travelling long distances. For many rare diseases, a combination of periodic specialist review and advice needs to be combined with local care; the multiorgan involvement of some rare diseases requiring interaction with other specialists; the growing identification of genetic causes for many rare diseases requiring closer interaction with clinical geneticists, and diagnostic laboratory services in genetics. The pathways will take into consideration: the age of presentation; the nature and range of clinical manifestations; the diagnostic process; the logistics and constituents of treatment; co-morbidities; psychosocial issues. The <i>Renal RaDaR [rare diseases registry]</i> is integral to the success of this strategy. It will facilitate clinical care, audit and research: It will be organised within the structures of the UK Renal Registry; Disease-specific working groups will develop specific elements in RaDaR building cost effectively on a generic platform; Initial establishment of the generic RaDaR platform with short life research funding will be followed by a sustainable funding model, for example, based on capitation as presently in place for the UK Renal Registry and Renal Patient View; Governance of RaDaR will be the responsibility of the Renal Association.		
	Kids Kidney Research	This study is a qualitative research study adopted by the MCRN looking at styles and methods of teaching dialysis methods and techniques to parents. Veronica Swallow is the Chief Investigator. Ethical approval is in place and phase I is due to start soon.	No action required.	LP
7. Possible future studies	Formulation issues with Cystagon	Following the last CSG meeting, MOG and LK have discussed Cystagon with their colleagues in terms of current studies up and running and formulation issues. In brief, it was concluded that research in this area is very much under control.		

<p>8. Industry Matters</p>		<p>In setup: MCRN047 (20050256) - Amgen - Darbepoetin alfa/Anaemia CKD Receiving/Not Receiving Dialysis (SD109) *LRNs: LSE (GOSH), WM (BCH) *CLRN: NT&W (1), WY (1) *Start of recruitment target: 03/2010 *End of recruitment: 11/2012?</p> <p>MCRN063 – Takeda/ICON – TAK-491_109 PK (SD075) *LRNs: GMLC (RMCH), LSE (GOSH), T (QMC), SW (BRCH) *Start of recruitment target: 03/2010 *Targets: UK=12 (4 sites) *Time point targets: enrol the first subject in April 2010 (no specific date) *Recruitment of Cohorts 1 and 2 is planned to end on 8 November 2010 *Planned start for Cohort 3 is 20 December 2010</p> <p>Pre-adoption: Takeda – hematide/ESA/CKD programme *Micera</p> <p>Open: MCRN026 (0954_337-01) Merck/Parexel – hypertension *LRNs: GMLC (RMCH), LSE (GOSH) *CLRNs: HIOW (1) *Start of recruitment: 17/07/2009 *End of recruitment: 3/2010 *Targets: GMLC = 2-3 (3), LSE = 2-3 (1), HIOW=2-3 (0)</p> <p>MCRN041 (C08-003B) – Alexion – Eculizumab/PT-sensitive aHUS adolescents (SD089) *CLRNs: NTW (1) *Start of recruitment: 27/08/2009 *End of recruitment: 05/2010+ *Targets: NTW (1) = 0-1 (0)</p> <p>Closed: MCRN003 (MK0954-326) - MSD – Cozaar/Proteinuria</p>	<p>Notify Andrew Rose, MCRN Industry Liaison Manager, as to whether or not there have been any changes in clinical practice as a result of the closed portfolio studies.</p> <p>Each unit to reply to MS re their expression of interest.</p>	<p>CSG Members</p> <p>CSG Members</p>
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* >100% recruitment, n=9, t=8 (2 MCRN sites)
 * Follow-up ends: 31/10/2011
 * Positive outcomes, changes to SmPC
<http://cjasn.asnjournals.org/cgi/content/abstract/CJN.06620909v1>

MS has been approached by a company looking to set up and do a Phase I trial to extend their license for an IV Iron preparation. The following summary was shown to the group.

CKD 251 and CKD 252:
Two Identical RCTs in Subjects with CKD



*Stepwise enrolment of older then younger age groups, and randomization into treatment groups stratified by age group: 12 to <18 yr, then 6 to <12 yrs, 2 to <6 yrs, and 6 months to <2 yrs



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MS has been asked for expressions of interest from each individual unit for participation in this phase I/II trial on an iron preparation. Each CSG Member agreed to notify MS should their unit be interested in taking part in this study.

9. Future strategies

A reflective discussion re what the CSG has achieved in the first 2 years of its establishment was undertaken. The focus of these discussions was on the progress of studies, successful or otherwise, and an honest assessment of whether the CSG has succeeded enough. In terms of

Each unit lead to discuss with their unit priorities for research and feed these ideas back at the

CSG Members

		taking things forward the following points were recorded: 1) There is a definite need to work together on designing a portfolio of studies and for this not to be done in isolation. It was suggested that each centre could be asked about their research ideas and each unit lead could feed these back at the next CSG meeting for discussion; 2) The CSG needs to support the delivery of its existing studies; 3) The CSG should think about appointing an MCRN/BAPN fellow to work on projects. This person would be responsible for working across the centres on grant applications; coordinating additional study group meetings outside of the CSG etc 4) The CSG should consider more cross-CSG working activities (e.g. engaging the pharmacy and pharmacology/methodology CSGs where possible).	next CSG meeting. Discuss writing a fellowship proposal.	MS/NW
10. Latest funding opportunities		An update of the latest calls for proposals from the NIHR and selected calls from major Research Councils was circulated prior to the meeting for information. Calls for Fellowship awards and training opportunities were also included. Members were directed to www.rdfunding.org.uk for a comprehensive list of funding opportunities from all of the large funders.	No action required.	N/A
11. Any other business	N/A	NW is trying to establish interest for a Cinacalcet study in the under 6's. It needs a CRF and detailed PK sampling. Expressions of interest should be sent to NW. 12 patients are needed in total.	Send expressions of interest to NW.	CSG Members
12. Date, time and location of next meeting	N/A	The next meeting will be held on Tuesday 6 th July 2010 at the Medical Research Council Head Office, London (11:00 – 15:00).	No action required.	N/A