

## **The UK Medicines for Children Research Network** **Clinical Studies Groups**

The UK Clinical Research Network (UKCRN) was established to provide support for clinical research and to facilitate the conduct of randomised trials and other well-designed studies. The Medicines for Children Research Network (MCRN) is a topic specific research network (TCRN) managed by the UKCRN

The MCRN was developed by a consortium from The University of Liverpool, Royal Liverpool Children's Hospital, Imperial College London, The National Perinatal Epidemiology Unit (University of Oxford), Liverpool Women's Hospital and The National Children's Bureau. The Coordinating Centre for the MCRN is based in the University of Liverpool's Institute of Child Health at the Royal Liverpool Children's Hospital and led by its Director, Professor Rosalind Smyth.

The MCRN has been established to provide a world-class health service infrastructure to support clinical paediatric research and remove barriers to its conduct. The aims of the Network are:

**“to facilitate the conduct of randomised trials and other well-designed studies of medicines for children, including those for prevention, diagnosis and treatment”**

And by this, it is the intention of the MCRN to:

- Improve the care of children and their families
- Improve the coordination of research
- Improve the speed of research
- Maintain and enhance the quality of research
- Improve the integration of research
- Widen participation in research

This document outlines the key principles underpinning the membership and composition of the MCRN CSGs and details the main duties and qualities required of members.

### **Background**

Due to the relative paucity of clinical trials involving children, the task of developing a portfolio of trials of medicines for children presents challenges, but also exciting opportunities to develop a new research agenda where previously little has existed. The MCRN Clinical Studies Groups have been established to facilitate this process and were set up along the lines of the successful model developed by the National Cancer Research Network (NCRN) (information on the NCRN CSGs can be located on the following website: <http://www.ncrn.org.uk/csg/index.asp>).

Due to the urgency to establish a portfolio, individuals who already held a leadership role for research within an associated clinical group were invited to Chair some of the CSGs. In other instances Chairs were nominated by relevant RCPCH specialty groups. These individuals were subsequently invited to chair the groups for an initial period of 2-3 years, but the appointment of Chairs and members will be done by an open and transparent process to ensure that the entire paediatric research community have the opportunity to contribute to the portfolio. Additionally, as a result of the pressure of establishing CSGs without delay and the desire to build on the

enthusiasm and commitment of individuals who had expressed an interest in forming CSGs, a limited number of core members were appointed directly by the CSG Chairs, with approval by the MCRN Executive. These core groups consist of around 6-8 key experts and are in post for an initial period of 2-3 years, after which renewal of membership will be subject to an open and transparent process. Additional members are also required to ensure multidisciplinary involvement, and it is recommended that such members should be selected competitively involving an advert and request for CV on an appropriate website. The MCRN Coordinating Centre staff will advise Chairs on this process.

### **Remit and Terms of Reference for MCRN Clinical Studies Groups**

The MCRN currently has 11 Clinical Studies Groups, covering most paediatric specialty areas;

- Anaesthesia, Intensive Care and Pain Control and Cardiology
- Diabetes, Endocrinology and Metabolic Medicine
- Gastroenterology, Hepatology and Nutrition
- General Paediatrics
- Methodology
- Neonatal
- Neurosciences
- Pharmacy and Pharmacology
- Rheumatology, Allergy, Nephrology, Infection and Immunity (RANII)
- Respiratory and Cystic Fibrosis
- The United Kingdom Children's Cancer Study Group (UKCCSG) - this is affiliated to the MCRN as a CSG, while recognising that it is also a Specialist Research Network within the NCRN

Within each group, sub-groups may be established to ensure that each specialty is adequately addressed.

### **Responsibilities**

The CSGs should aim to bring together the expertise and enthusiasm necessary to establish them as an authoritative voice in the paediatric research community. The main role of the MCRN CSGs is the consideration of clinical studies to run through the MCRN. This is the primary, but not sole, route by which clinical studies are considered in the development of the MCRN research portfolio. Clear processes for the leadership and membership of these CSGs have been established to promote transparency, and full understanding of this new role.

All MCRN CSGs have a generic remit to:

- be responsible for developing and overseeing its portfolio of studies
- propose and develop new trials and other well-designed studies
- consider trials proposed by others, and advise the MCRN Trial Adoption Committee
- provide specific advice to investigators
- ensure consumer involvement in all activities

## **Operation**

The CSGs are supported by the MCRN Coordinating Centre (MCRNCC) and the CSG Administrator has the role to oversee the organisation and administration of the CSGs. The CSG Administrator is based at the MCRNCC and attends and is responsible for arranging all CSG meetings, circulating agendas and papers and taking minutes of the meetings (see Appendix 1iii)

Clinical Studies Groups hold two key CSG meetings per year and all CSG meetings and teleconferences are attended and supported by the CSG Administrator. CSGs must ensure a regular dialogue between meetings and email discussion forums are being set up to facilitate this.

## **Funding of CSG Meetings**

The MCRNCC funds travel costs (second class rail or equivalent), but not accommodation costs, for these CSG meetings. Accommodation is available free of charge at the MCRN Coordinating Centre in Liverpool, and the MRC Clinical Trials Unit, London. One teleconference per year is funded by MCRN. The cost of any further meetings or teleconferences must be met by sources other than the MCRNCC.

The National Cancer Research Network CSGs are funded entirely by contributions from the leading cancer charities and the Medical Research Council. The MCRNCC has made links with funding bodies within the Association of Medical Research Charities (AMRC) and has aligned each CSG to the appropriate funding bodies. It is hoped that ultimately these funding bodies may be able to contribute towards the cost of running the MCRN CSGs.

## **Membership**

The membership must be composed to reflect, as reasonably as possible, the various agencies involved in the development and implementation of high quality protocols in the area of Medicines for Children. As a minimum, CSGs should include multi-professional representation and should be drawn from as wide a geographical area as possible. Group membership should seek to strike a balance between experienced members with a strong track record in clinical trials and less experienced, but equally committed, members showing the potential to become leaders in the research community. Membership of CSGs must include at least one representative from outside the UK (Scotland, Wales or Northern Ireland).

**Consumer Representation:** Each CSG should seek to involve consumers (children, young people or parents) in all aspects of its work. The nature of this involvement may vary:

- some groups may wish to invite a topic-specific consumer or a consumer representative onto their group.
- a shadow consumer panel of young people or parents may need to report their views to the CSG
- others may wish to work through known charitable bodies, perhaps bringing several representatives together at times.
- the CSG agenda may require that specially designed exercises are designed for focus groups of children, young people or parents in order to capture their views.

Consumer representatives are appointed in consultation with the MCRN Consumer Liaison Officer (CLO) based at the MCRNCC. The CLO offers advice and support to CSGs and consumers to enable meaningful engagement. The CLO will sit on the CSG Chairs Forum to ensure continued dialogue on consumer representation between CSG meetings.

The MCRNCC will fund travel costs, child care costs and other out-of-pocket expenses for consumers who attend CSG meetings.

**Ex-officio members:** Ex-officio members are made up from funders and members of the MCRN Executive.

**Funding body representatives:** Relevant charitable bodies are in receipt of all meeting papers and are invited to attend all meetings as non voting members.

**International representation:** Groups are encouraged to consider appropriate international representation. Any international appointments should be supported by a formal application, reviewed by the group and ratified by the MCRN Operational Group.

**External expertise:** Additional input might be achieved by inviting experts in a particular field to address specific issues/research ideas rather than to become full members.

Formal links with MCRN Local Research Networks are encouraged.

The MCRN will work closely with the pharmaceutical industry and will undertake appropriate clinical trials in which the pharmaceutical industry is a sponsor or partner. CSG members with specific or general expertise may be asked to be members of adoption panels which evaluate proposed trials from the pharmaceutical industry via the commercial trials adoption process under development with UKCRN. Evaluation will include relevance to the NHS and the important unmet needs of child health, statistical validity (at the level of an experienced clinical trialist) and feasibility. These evaluations will need to be undertaken as a matter of priority.

**Appointment of Membership:** Future membership of CSGs will be appointed through an open process (see Appendix IV). Vacancies will be routinely advertised through established MCRN communication routes (e.g. websites). Other channels, such as professional bodies, will be used as appropriate. The CSGs will review applications (CVs) and recommend the appointment of new members. These will be ratified by the MCRN Executive. Notification of appointment will be by letter from the CSG Chair. New members will be provided with an induction pack and invited to attend an induction day.

Membership will be for three years with the possibility of an extension for a further two years duration. Decisions on extensions will be made by the MCRN Operational Group, following an application in which the overriding consideration will be the needs of the group and its work. At the end of a term of membership a one-year period must elapse before members can reapply for a further term. Exceptions to this, should they arise, will be reviewed on a case-by-case basis.

Members whose term of office expires before the study for which they are the Chief Investigator has been completed will maintain links with the group through the submission of written reports. Invitations/requests to attend should be made only

when pressing issues demand and should be by prior agreement with the Chairman of the group.

Membership may be terminated if a scientific member fails to attend three consecutive meetings.

Membership is in an individual capacity and attendance of deputies for specific meetings should reflect exceptional circumstances and be by prior arrangement with the Group Chair or CSG Administrator. The effectiveness and composition of the groups will be considered as part of the three yearly review of its portfolio.

## Accountability

**MCRN Operational Group:** The responsibility for the MCRN CSGs is held by the MCRN Operational Group, chaired by the Director of the MCRN, Professor Rosalind Smyth. The Operational Group will manage the performance of the CSGs, the appointment of Chairs, oversee the appointment of new members and the development of new groups. The group is also responsible for operations within the MCRN and the performance management of the network overall. Two nominated CSG Chairs represent the interests of the CSGs in this group and feedback issues to the CSG Chairs Forum. The chairs will serve on the Operational Group on a 2-yearly basis.

**MCRN CSG Chairs' Forum:** The CSG Chairs' Forum enables dynamic interaction between all MCRN CSG Chairs. The main responsibilities of this forum are:

- to guarantee that the research portfolio of studies is balanced, relevant and appropriate;
- to ensure that all CSGs operate rigorous standards in prioritisation, development and assessment of MCRN studies
- to provide a forum where CSG views can be discussed and represented at the MCRN Operational Group and Trial Adoption Committee.

Click here for full remit and terms of reference for CSG Chairs Forum:

[Shortcut to Remit and Terms of Reference for CSG Chairs Forum.doc.Ink](#)

**MCRN Trial Adoption Committee:** The Trial Adoption Committee considers the suitability of studies to run through the Medicines for Children Research Network. Studies are reviewed by the Trial Adoption Committee for their quality and appropriateness to be adopted by the network. Two nominated CSG Chairs, other than those serving on the Operational Group represent the CSGs on this committee, serving for 2 years on a rotational basis.

Click here for full remit and terms of reference for MCRN Trial Adoption Committee:

[Shortcut to Remit and Terms of Reference for MCRN Trial Adoption Committee.doc.Ink](#)

**APPENDICES:**

APPENDIX I	Job description for CSG Scientific Members
APPENDIX II	Job description for CSG Chairs
APPENDIX III	Job description for CSG Administrator
APPENDIX IV	Recruitment of MCRN Chairs and Members
APPENDIX V	Clinical Studies Groups Scope Documents

## **APPENDIX I - MCRN CSG Job Description - Scientific Membership**

### **1. DUTIES**

Members are expected to attend all meetings of the group and will contribute to the maintenance and further development of its portfolio of research by:

- actively engaging with trials within the group's portfolio, for instance through entering patients, offering information and advice to collaborators, presenting findings as appropriate;
- identify existing high quality studies that should be adopted by the group;
- generating ideas for new trials, which may involve working with the Cochrane Collaboration and James Lind Alliance;
- contributing to the development of high quality applications through the review of trial ideas and protocols submitted to the group;
- contributing to consultation exercises (eg NICE) undertaken by the group as requested;
- contributing to the group's annual report and peer-review of the portfolio
- providing expert advice to the Chair, MCRN funders, and the wider clinical paediatric community as required.
- providing expert advice to the commercial trials adoption process under development with UKCRN CC

### **2. QUALITIES**

Members should have a track record of participation in research in the relevant paediatric fields. Members should contribute actively to the work of their group, and support studies within the group portfolio. The intention is to develop a portfolio that is well balanced and draws on local MCRN strengths and therefore stimulates accrual. The composition of each CSG should reflect this and members should, therefore, be able to demonstrate some of the following:

- experience of collaborative clinical trials activity in a leadership capacity;
- success in trial accrual into national studies;
- evidence of publications and/or presentations nationally/internationally;
- links with paediatric research networks;
- an enthusiasm and commitment to developing research into medicines for children.
- Members should be prepared to maintain commercial confidentiality when working with the pharmaceutical industry and to declare conflicts of interest.

### **3. RELATIONSHIPS**

Members will be responsible to the MCRN Operational Group through the CSG Chair. Administrative support will be provided by the MCRN CSG Administrator.

### **4. REMUNERATION**

Work relating to the groups is regarded as national work for the purposes of the consultant contract and job planning. Membership of a CSG is unpaid. Reasonable travel expenses (second class fares) will be reimbursed in accordance with University of Liverpool finance policy.

## **APPENDIX II - MCRN CSG Job Description – Chair**

### **1. DUTIES**

The Chair will directly or by delegation:

- Oversee the portfolio of trials developed or adopted by the group.
- Contribute to studies developed or adopted by the group.
- Monitor the progress of the group.
- Receive input from the MCRN CC.
- Liaise with other National and International trials organisations.
- Propose membership of the group in consultation with the MCRN Operational Group.
- Report to the MCRN Operational Group annually and through the peer review mechanism.
- Provide advice to the MCRN Operational Group or MCRN, by attending all regular meetings.
- Promote good clinical research practice.
- Provide expert advice to the commercial trials adoption process under development with UKCRN CC

### **2. QUALITIES**

The Chair must have highly developed leadership skills, be an excellent communicator and skilful team player. Specifically the post holder needs to be able to demonstrate the following experience and competencies:

- Clinician with academic excellence in paediatric medicine.
- Previous experience of chairing research meetings effectively.
- Evidence of an ability to provide leadership to a research group.
- Be prepared to take action and implement decisions.
- Previous track record of collaborative research in paediatric medicine.
- The Chair should be prepared to maintain commercial confidentiality when working with the pharmaceutical industry and to declare conflicts of interest.

### **3. RELATIONSHIPS**

Responsible to the MCRN Operational Group, and through this to the MCRN CC.

Administrative support will be provided by the MCRN CSG Administrator.

The term of appointment will be 3 years in the first instance.

### **4. REMUNERATION**

This post of Chair of an MCRN CSG is unpaid. Reasonable travel expenses will be reimbursed in accordance with University of Liverpool finance policy.

### **APPENDIX III - Job description for CSG Administrator**

The post provides an ideal opportunity for an organised and motivated individual with experience of coordinating and administering the meetings and activities of clinical and scientific staff from a range of backgrounds. You will work closely with the Chairs of the MCRN CSGs to arrange and support the groups meetings, and provide feedback from these meetings to the MCRN Coordinating Centre. The post is based at the MCRN Co-ordinating Centre, at the Institute of Child Health in Liverpool, but the postholder will be required to attend around 10 meetings of CSGs per year, and occasionally other relevant meetings, outside Liverpool. It is not anticipated that these journeys will require overnight stays. You will be required to be involved in a broad range of responsibilities associated with the effective management of these meetings and activities, including:

- Arranging and attending two meetings a year for each of around 10 CSGs. At least half of these meetings will be at the MCRN Coordinating Centre in Liverpool, the remainder at other locations in the UK. This will involve arranging dates, ensuring that adequate facilities are available and circulating the agenda and relevant papers for each meeting.
- Organising and attending teleconference meetings for the CSGs, as needed and ensuring that all attendees are aware of the teleconference arrangements and requirements.
- Working closely with the CSG's Chairs to draft the agenda for the meetings, circulating these, with associated documents, to the groups' membership prior to the meetings, and ensuring that the agenda items are adequately addressed at the meetings.
- Taking minutes of CSG meetings, circulating minutes and action points following the meetings, and ensuring that action points are carried out within the designated timeframe.
- Presenting a summary of MCRN progress and activities to the CSG members at the meetings, and updating Chairs and members on MCRN developments.
- Providing feedback to MCRN Coordinating Centre staff on the activities of the CSGs and reporting on their progress and any developments.
- Working with the MCRN Coordinating Centre staff and CSG Chairs to support the appointment of new Chairs or members as appropriate, and facilitating the induction of new Chairs and members to the groups.
- Responding for requests for information on MCRN activities and CSG issues from both within and beyond the MCRN CSG membership.
- Keeping attendance records for the CSG meetings, and ensuring that these records are made available on the MCRN website.
- Overseeing the payment of travel expenses for CSG Chairs and members, in accordance with University of Liverpool financial procedures.
- Keeping up to date with research developments and findings within the MCRN and the wider academic and clinical community.
- Maintaining links with the UKCRN and other clinical research networks, and collaborating on, or contributing to, joint projects as and when appropriate.

## **APPENDIX IV – Recruitment of MCRN Chairs and Members**

The process for recruitment of new members and future Chairs to Clinical Studies Groups must be open, transparent and competitive. Such positions should be made available to all members of the paediatric clinical research and consumer community and adverts must be placed on such outlets to reach a wide coverage of the relevant audience.

### **Proposed Process for Recruitment of CSG Chairpersons and Members**

#### **Process for Recruitment of CSG Chair:**

- Vacancy advertised via approved communication routes
- Applicant(s) requested to send CV and covering letter outlining suitability for role to CSG Administrator
- MCRN Executive Committee acts as Selection Committee, receiving CVs in advance and making appointment via teleconference.
- Successful applicant informed via letter from MCRN Director

Term of office for MCRN CSG Chair: **3 years**

#### **Process for Recruitment of CSG Membership:**

- Vacancy advertised via approved communication routes
- Applicants requested to send CV and covering letter outlining suitability for role to CSG Administrator
- CSG elects a 'Selection Group' of at least 3 members who are independent of all applicants (to avoid conflict of interests) to select the most suitable candidate.
- Following closing date for applications, all CVs posted on relevant CSG Email Discussion Forum for review by selection group (within a firm timescale)
- Selection group recommend an appointment.
- Chair produces report to justify the group's choice of member, to be sent to MCRN Executive.
- Appointment ratified by MCRN Executive following review of report and CV of successful applicant.
- Successful applicant informed via letter from CSG Chair.

Term of office for MCRN CSG Members: **3 years**

#### **Appeals Process:**

Should there be an appeal raised during the selection process the MCRN Executive reserves the right to review all applications.

### **Advertisement of Vacancies for CSG Chairs and Members**

#### **Recruitment of Chairs and Scientific Members:**

Vacancies for Chairs should be advertised on:

- MCRN website \*
- UKCRN website
- Through the relevant RCPCH Specialty Group – mailing list/newsletter/email discussion forum
- Identified affiliated professional body websites/email discussion lists/newsletters (see attached chart)

\* All advertisements will include details of the MCRN website, from which the CSG Document can be accessed for further information.

In addition to the above standard advertising outlets, there are specific contacts that can be used when advertising for specific allied professionals to join the CSGs:

**Research Nurse Representatives:**

The following possible routes for advertising vacancies have been identified:

- Royal College of Nursing (RCN) Research and Development Coordinating Centre
  - Career Development Opportunities page on website
  - Weekly Electronic Bulletin – Career Development section
- RCN Research Society
  - Newsletter (bi-annual)
  - Website
- Research in Child Health (RiCH) group
  - Notice board page on website
- Clinical Research Nurses Association (CRNA)
  - Newsletter
  - Website

**Specialty Nurse Representatives:**

Such vacancies could be advertised via professional bodies identified as being aligned with the CSG (see following chart)

**Pharmacists:**

- Neonatal and Paediatric Pharmacists Group (NPPG)
  - Website
  - Mailing list
- The Pharmaceutical Journal
  - Brief summary in 'Announcements' section to avoid large advertising fee.

**Topic-Specific Consumer Representatives**

- INVOLVE website
  - Mailing list
- Relevant charitable bodies

**Current list of Professional Organisations affiliated to Clinical Studies Groups**

<b>CSG</b>	<b>Affiliated Professional Body</b>	<b>Possible routes for advertising vacancies</b>
<b>Anaesthesia, Intensive Care and Pain Control</b>	Paediatric Intensive Care Society (PICS)	<ul style="list-style-type: none"> <li>▪ Contact made with Chair of PICS Study Group for mailing list</li> </ul>
	Association of Paediatric Anaesthetists	<ul style="list-style-type: none"> <li>▪ Discussion forum and job vacancies on members section of website.</li> </ul>
<b>Diabetes, Endocrinology and Metabolic Medicine</b>	British Society for Paediatric Endocrinology and Diabetes (BSPED)	<ul style="list-style-type: none"> <li>▪ Website (possible advert)</li> <li>▪ Contact known for mailing list</li> </ul>
<b>Gastroenterology, Hepatology and Nutrition</b>	British Society of Paediatric Gastroenterology, Hepatology and Nutrition (BSPGHAN)	<ul style="list-style-type: none"> <li>▪ Vacancies section on website, for consultants, nurses and dieticians</li> </ul>
	British Dietetic Association	<ul style="list-style-type: none"> <li>▪ Careers section of website (charges apply for adverts)</li> <li>▪ Vanessa Poustie member of BDA</li> </ul>
	British Association for Parenteral and Enteral Nutrition (BAPEN),	<ul style="list-style-type: none"> <li>▪ Poss utilise newsletter</li> <li>▪ Poss utilise website</li> </ul>
	RCPCH Nutrition Committee	<ul style="list-style-type: none"> <li>▪ Contact known for Chair, mailing list</li> </ul>
<b>General Paediatrics</b>	British Association of General Paediatricians	<ul style="list-style-type: none"> <li>▪ No website yet</li> <li>▪ Contact for secretary of group – poss mailing list</li> </ul>
<b>Neonatal</b>	British Association of Perinatal Medicine (BAPM)	<ul style="list-style-type: none"> <li>▪ Website</li> </ul>
	British Maternal and Foetal Medicine Society (BMFMS)	<ul style="list-style-type: none"> <li>▪ Website – possible contact made</li> </ul>
<b>Neurosciences</b>		
<b>Methodology</b>		
<b>Pharmacy and Pharmacology</b>	Neonatal and Paediatric Pharmacists Group (NPPG)	<ul style="list-style-type: none"> <li>▪ Contact known for advert on website and email mailing list</li> </ul>
	The Pharmaceutical Journal	<ul style="list-style-type: none"> <li>▪ 'Announcements Section'</li> </ul>
<b>RANII (Rheumatology, Allergy, Nephrology, Infection and Immunity)</b>		
<b>Respiratory and Cystic Fibrosis</b>	<ul style="list-style-type: none"> <li>▪ British Paediatric Respiratory Society (BPRS)</li> </ul>	<ul style="list-style-type: none"> <li>▪ Advertised posts section of website</li> <li>▪ Newsletter</li> <li>▪ Contact made – possible mailing list</li> </ul>

## APPENDIX V – Clinical Studies Groups Scope Documents

### METHODOLOGY

**INTENTION:** The intention of the group is to foster research to improve the conduct of research which aims to improve the health and well being of children.

#### WORK OF GROUP

- Liaison with the MCRN research community to identify research priorities and any organisational difficulties that are impacting negatively on the research agenda;
- Liaison with the wider research community to identify research priorities and any organisational difficulties that are impacting negatively on the research agenda;
- Co-operation with researchers on projects in development;
- Active participation in research;

**POTENTIAL OVERLAP:** There is potential for the work of the group to overlap with all the other CSGs. The CSG Chairs Forum will be the opportunity for this group to inform each of the other CSGs of its remit and the studies being considered or developed.

#### AREAS COVERED BY CSG:

Trials methodology, including:

- New ways to recruit and retain participants
- Improving what we currently do
- Improving the efficiency and effectiveness of the trial office

Views of participants, including:

- Developing consumer involvement

Statistical approaches, including:

- Longitudinal measures

Consent processes

Outcome assessment, including:

- Development of utility measures in children
- Quality of life measures in children
- Clinical outcomes of relevance to medicines for children
- Surrogate outcomes

Communication with participants, including:

- Dealing with death in trials of medicines for children

DNA sampling

Identification of eligible participants, including:

- GP databases
- Prescription databases

IT systems to facilitate conduct of trials of medicine for children

Pharmacovigilance

## RESPIRATORY AND CYSTIC FIBROSIS

**INTENTION:** The intention of the group is to identify the important clinical questions which need answering in relation to the care of children with respiratory diseases and cystic fibrosis. Once identified the intention is to progress them into relevant clinical trial protocols and seek funding opportunities to allow the trials to take place.

The group also intends to support and advise individuals or research groups who are developing studies and clinical trials in children with respiratory diseases (+CF) by endorsing their studies or advising how they might be improved.

### **WORK OF GROUP:**

- The group has been working closely with all members of the British Paediatric Respiratory Society initially in identifying key unanswered clinical questions and prioritising topics for research.
- It has interrogated the Cochrane Database to help identify those important research questions
- It has used documentation from Asthma UK to identify relevant research topics in asthma.
- It has responded to the first HTA call for Medicines for Children by submitting 7 topics for research funding. Four of these 7 studies have been progressed to the full proposal stage and we await the outcomes in June 2006. Following these we can plan and re-group our priorities for future funding opportunities.
- Prioritisation, in a timely fashion, of research priorities presented to the network.

**POTENTIAL OVERLAP:** There may be overlap with the infectious diseases CSG especially in relation to viral respiratory infections in infancy, viruses as triggers for wheezing and asthma exacerbations and in diseases such as tuberculosis. There may be overlap with other CSGs in relation to non-respiratory areas in CF.

### **AREAS COVERED BY CSG:**

- Asthma (acute and chronic management)
- RSV / acute viral bronchiolitis
- Viral-induced wheeze
- All aspects of cystic fibrosis (pulmonary and non-pulmonary)
- Bronchiectasis (CF and non-CF)
- Tuberculosis
- Management of chronic cough
- The pneumonias (bacterial / viral / fungal) including pleural effusions / empyema
- Nasal problems (infective / inflammatory)
- Respiratory failure including PICU management

We need to know the specific areas covered by the various national charities such as Asthma UK, British Lung Foundation and the Cystic Fibrosis Trust to enable as close a working relationship as possible. Transition between childhood and adulthood is an area where funding may be possible and we need to work closely with our adult respiratory colleagues to maximise study opportunities. These statements equally apply to colleagues in the pharmaceutical industry who continue to need studies undertaken for the development of licensing and clinical usage of medications.

## NEONATAL

**INTENTION:** The intention of the group is to foster research that aims to improve the health and well being of new born babies both in the period immediately after birth and that also results in improvements in health status later in life. Where studies are being considered for recruitment by the Medicines for Children Research Network priority will be given to therapeutic studies.

### **WORK OF GROUP**

- Liaison with the research community to identify research priorities and any organisational difficulties that are impacting negatively on the research agenda;
- Liaison with relevant consumers regarding research priorities;
- Liaison with the wider perinatal community regarding research priorities
- Co operation with researchers on projects in development;
- Active participation in research;
- Liaison with industry regarding development and licensing needs;
- Development of an effective “extended” collaborative neonatal network;
- Prioritisation, in a timely fashion, of research priorities presented to the network.

**POTENTIAL OVERLAP:** There is potential for the work of the group to overlap with a number of other CSGs but on the whole the number of occasions that this happens with any one group is likely to be low. Those where overlap is most likely to occur from time to time include: neurology, respiratory and gastroenterology, endocrine, pharmacy, PIC and methodology.

### **AREAS COVERED BY CSG:**

- Trials of therapeutic agents and interventions that affect or potentially affect the health and well being of newborn babies.
- Observational studies that focus on neonates or have the health and or well being of neonates as a significant outcome.

## GENERAL PAEDIATRIC

**INTENTION:** The intention of the group is to foster research to improve the conduct of research which aims to improve the health and well being of children.

### WORK OF GROUP

- Liaison with the research community to identify research priorities and any organisational difficulties that are impacting negatively on the research agenda;
- Liaison with relevant consumers regarding research priorities;
- Co-operation with researchers on projects in development;
- Active participation in research;
- Prioritisation, in a timely fashion, of research priorities presented to the network.

**POTENTIAL OVERLAP:** There is potential for the work of the group to overlap with all other CSGs.

### AREAS COVERED BY CSG:

Previous well children presenting acutely with:

- fever
- upper respiratory tract infections (inc OM, tonsillitis, pharyngitis), asthma, croup, bronchiolitis, pneumonia
- meningitis, (viral, meningococcal / pneumococcal )
- herpes encephalitis/primary herpes infections
- Kawasaki's disease
- UTI, HSP
- ITP
- febrile convulsions
- afebrile seizures
- gastroenteritis
- eczema
- other - Irritable hip, colic, osteomyelitis, septic arthritis
- pain and sedation issues

Children with chronic ongoing illness:

- asthma
- allergies
- recurrent abdominal pain
- constipation
- gastro colic reflux
- migraine/headache
- absence seizures
- primary generalized epilepsy
- breath holding, reflex anoxic seizures
- chronic fatigue/ME
- vulvovaginitis
- enuresis
- follow up of conditions in other list

## PHARMACY AND PHARMACOLOGY

**INTENTION:** The intention of the group is to foster research to improve the conduct of research which aims to improve the health and well being of children.

### **WORK OF GROUP**

- To establish the research priority of the below topics.
- To develop high quality research proposals and apply for funding where it is appropriate.
- To liaise with other CSGs to assist them in the development of research proposals and funding applications where appropriate.

**POTENTIAL OVERLAP:** All of the above topics are likely to be applicable to other CSGs; therefore there will be close liaison with chairs of other CSGs to avoid duplication.

### **AREAS COVERED BY CSG:**

- Formulation subgroup
- Pharmacokinetics and Pharmacogenomics subgroup
- Pharmacovigilance to be covered by Methodology CSG

## GASTROENTEROLOGY, HEPATOLOGY AND NUTRITION

**INTENTION :** To work in collaboration with colleagues in our specialties to design and undertake important clinical trials relevant to our patients' needs.

**WORK OF THE GROUP:**

- To seek active collaboration with expert colleagues
- To work with professional bodies such as the British Society of Paediatric Gastroenterology Hepatology & Nutrition (BSPGHAN) and the British Society of Gastroenterology (BSG) and their various Working Groups in clinical trial planning and development
- To identify priority areas of clinical trial research in our specialties
- To provide external review of research proposals
- To work with colleagues in trial development
- To liaise with industry in trial development
- To establish a dialogue with consumers regarding research priorities
- To work with consumers in trial design and planning
- To establish a balanced portfolio of high quality clinical trials

**POTENTIAL OVERLAP:**

**Diabetes, Endocrinology and Metabolic Medicine:** coeliac disease, cystic fibrosis, growth, metabolic syndrome and non-alcoholic steatohepatitis, metabolic liver disease

**Pharmacy & Pharmacology:** multiple areas of overlap

**Rheumatology, Nephrology, Immunology & Infection:** Management of rheumatological complications of inflammatory bowel disease, immunomodulation, gastrointestinal manifestations of immunodeficiency disorders, gastrointestinal manifestations of allergy, HSP, protracted diarrhoea of infancy, HIV, gastroenteritis, hepatitis, solid organ transplantation

**General Paediatrics:** constipation, gastro-oesophageal reflux disease, gastroenteritis, infant colic, irritable bowel syndrome, functional gastrointestinal disorders

**Respiratory:** cystic fibrosis, gastro-oesophageal reflux disease

**Neurosciences:** hepatic encephalopathy, cyclical vomiting syndrome

**Neonatology:** necrotizing enterocolitis, intestinal failure, short bowel syndrome, nutrition

**AREAS COVERED BY CSG:**

- Clinical studies and trials on medicines in paediatric gastrointestinal and liver disease.
- Clinical studies and trials of nutritional interventions in childhood

## NEUROSCIENCES

**The intention of the group:** To foster clinical drug trials and related research to improve the health and well being of children and young people, cared for within the specialties of paediatric neurology, paediatric neurodisability, and developmental neuropsychiatry / child and adolescent mental health and by hospital based and community paediatricians and other health care services with neurological, neuro-developmental and psychiatric disorders.

**The work of this group** will include:

- Liaison with the MCRN research community to identify research priorities where neuroscience trials expertise may prove helpful;
- Liaison with the wider paediatric neurology, paediatric muscle disease, paediatric neurodisability, and developmental neuropsychiatry / child and adolescent mental health research communities and their consumer communities, through representatives of the British Paediatric Neurology Association, their Epilepsy Interest Group, the UK paediatric Muscle Interest Group, the British Academy of Childhood Disability, the child and adolescent psychiatry research community, to identify research priorities and help develop trials and feed priorities into the MCRN for adoption;
- Co-operation with researchers on projects in development;
- Active participation in research;
- Prioritisation, in a timely fashion, of research priorities presented to the network.

**Potential overlap with other CSGs:** The CSG Chairs Forum will be the opportunity for this group to inform each of the other CSGs of its remit and the studies being considered or developed.

### **Areas covered by neurosciences CSG**

Topics which can be included within the remit of the neurosciences CSG include:

Drug trials of:

- Antiepileptic drugs (AEDs) e.g. Buccal Midazolam pre-hospital for continuing seizures; combined Prednisolone and Vigabatrin for Infantile Spasms / West syndrome; Prednisolone for other epileptic encephalopathies and non-convulsive status; head to head comparisons of AEDs for specified patient groups, epilepsies and seizures.
- Migraine treatments e.g. Pizotifen and Propranolol for prevention, other drugs used for prevention, drugs used as rescue treatments.
- Drug treatments for Idiopathic (benign) Intracranial Hypertension e.g. Furosemide, Acetazolamide, Prednisolone.
- Drug treatments for neurally mediated syncope e.g. Slow Sodium, Fludrocortisone, Atropine, Propranolol, Midodrine.

- Sleep disorders treatments e.g. Melatonin.
- Drugs for spasticity and dystonia e.g. Baclofen, Dantrolene, Tizanidine, Diazepam.
- Drugs used in stroke e.g. in secondary prevention, and in “clot-busting”.
- Drugs for use in other acute encephalopathies and acquired brain injuries e.g. acutely (early) and late.
- Drugs for muscular dystrophies.
- Drugs for a range of symptoms in children with developmental disorders, or behavioural phenotypes, or neuropsychiatric disorders, including children with specific learning disabilities, Attention Deficit Hyperactivity Disorder, Autism Spectrum Disorders, and related neuropsychiatric disorders.
- Drugs for mental illness e.g. depression; schizophrenia.
- Drugs for specific rare or very rare neurological diseases e.g. Friedreich’s Ataxia, Alternating Hemiplegia of Childhood, Ataxia Telangectasia, particularly where new understanding of e.g. the molecular basis of the disease has led to the development of rational but un-tested treatment approaches.

Views of participants, including:

- Developing consumer involvement
- Involvement of consumers in prioritising, scoping, selection of outcomes, recruitment, consenting and retention strategies, participant information, dissemination, and implementation.