



## Paediatric Research Roundtable

December 2008

Executive Summary



## PAEDIATRIC RESEARCH ROUNDTABLE – EXECUTIVE SUMMARY

In December 2008 a one-day Paediatric Research Roundtable was hosted by the NSW Government's Office for Science and Medical Research (OSMR) and the Children's Hospital, Westmead. The purpose of the Roundtable was to explore the level of interest of NSW researchers from a range of disciplines in the potential formation of a NSW Paediatric Research Network.

The Roundtable participants consisted of clinicians and researchers from the three tertiary children's hospitals, representatives from the neonatal centres, NSW clinical trials centres, pharmaceutical industry and government agencies relevant to the health and well being of children and young people. A full list of the Roundtable attendees can be found in Appendix 1.

The Roundtable provided an opportunity to follow-up on the recommendations arising from a Reference Group of eminent paediatric researchers and representatives from relevant NSW Government agencies that delivered a report to the Office for Science and Medical Research in August 2006. The Report outlined the issues and barriers to conducting clinical trials to research the safe and effective use of medicines for paediatric populations.

One of the difficulties for paediatric clinical trials is the relatively small numbers of children affected, especially for chronic diseases. The Reference Group considered that to obtain samples sizes of sufficient power to inform health policy and practice, the formation of collaborations between paediatric clinical researchers, and the development of standardised protocols and data collection for multicentred trials will be required.

To foster research collaboration the Reference Group recommended that a NSW Paediatric Pharmaceutical Clinical Trials Research Network be formed. The aim of such a Research Network would be to develop a sustainable structure for facilitating high quality randomised clinical trials into medicines for children. A key driver for the Research Network is the need to address the gap in the research evidence for the safe and effective use of medicines for paediatric populations by increasing efforts to encourage clinical trials that include infants, children and young people in NSW and Australia.

### **Network Development and the Office for Science and Medical Research**

The OSMR is supporting the establishment of clinical research networks in order to facilitate research collaborations across the NSW. The aim of the OSMR network development program is to link researchers across the State to enhance collaborative opportunities and to promote the translation of research into clinical practice. Networks also provide the potential to share expertise and resources to reduce infrastructure costs and attract increased investment.

To date, the development of major research networks in spinal cord injury and related neurological conditions, cardiovascular disease and complementary medicines, has assist in attracting over \$18 million in funding from State, Commonwealth and private sources.

## **Paediatric Research Roundtable Program**

The major topic for discussion at the Roundtable was the potential of developing a consortium of major paediatric tertiary teaching hospitals and their allied Universities to establish a set of research priorities and support significant research programs for the conduct of a range of clinical trials. Discussions centered around the possible benefits and barriers to the formation of a Network to bring together key stakeholders interested in collaborative efforts to build efficacy data concerning treatments and interventions provided to paediatric populations.

The Paediatric Research Roundtable program consisted of the following three sessions:

### **Session 1 – Introduction and Background**

The first session provided the background to the Roundtable, including the Expert Reference Group that reported to OSMR in 2006 that was the starting point for the consideration of the formation of a paediatric research network in NSW.

Representatives from each of the three major NSW paediatric tertiary treatment centres provided a presentation on the perceived advantages and barriers to the paediatric research network from the perspective of their research programs and interests.

### **Session 2 – Models and Infrastructure**

The second session focused on the various models for research networks and some of the infrastructure to support clinical trial activity in NSW. The NSW Cardiovascular Research Network provided an example of how a joint OSMR and Heart Foundation initiative was helping to drive research collaboration and opportunities. The presentation on the Australian Kidney Trials Network demonstrated the benefits gained from establishing a governance structure to support high quality, investigator-initiated clinical trials.

The NHMRC Trials Centre outlined some of the infrastructure support available to assist a paediatric research network through the Outreach NHMRC Enabling Grant. The NSW Clinical Trials Business Development Centre highlighted the advantages a Network would offer as a single point of contact for agencies interested to conducting paediatric clinical trials in NSW.

An overview was provided of international and Australian trends and initiatives to increase paediatric clinical trial activity with the goal of improving the level of evidence for the effective treatment of children and young people. The need for NSW to consider how best to participate in this global effort and enhance interaction with international bodies was emphasised.

### **Session 3 – Formation and Function of a NSW Paediatric Research Network**

The third session consisted of a facilitated discussion with all of the Roundtable participants to canvass the scope of a paediatric research network and consider the next steps required to progress the initiative.

## **Paediatric Research Network Scope**

One of the dilemmas for determining a focus for the network is the diversity of children. Infants, children and young people are a relatively heterogeneous group with very different needs and considerations across the age span, clinical issues and ethnic backgrounds. For example, the pharmacodynamics and pharmacokinetics of medicines differ across the paediatric age ranges and require specialist expertise and understanding of child development.

There are even greater barriers to the use of safe and efficacious medications for neonatal populations including limited support for neonatal clinical trials within the current pharmaceutical licensing structure in Australia, and the lack of research infrastructure for neonatal clinical trials. The difficulty in obtaining suitable medication formulation and dosages for infants and very young children is another barrier to the conduct of neonatal clinical trials. The need to develop appropriate methodologies to study small numbers as an alternative to the randomised control trial design was acknowledged.

The network membership would need to involve the 3 Tertiary hospitals, 9 Neonatal centres, and relevant sectors in public/community health. The importance of including consumer participation in the network was also discussed. It was considered that parents are best placed to provide useful insights for the network as well as having the potential to become powerful advocates for the activities of the network.

Initially, the focus of the network would be NSW. However, it is envisaged that opportunities for research collaborations across Australia and internationally, to increase participation numbers to provide studies with sufficient power, would be sought as the network becomes established.

## **Benefits of a Paediatric Research Network**

Participants agreed that there were several benefits from the formation of a research network focused on increasing research aimed at improving child health. Some of the advantages were considered to be the:

- enhanced opportunities to pool the available expertise and the potential to share research infrastructure;
- provision of a direct point of contact for pharmaceutical industries interested in establishing clinical trials; and
- ability to combine the track record of network researchers to make NSW more marketable as a place to conduct clinical trials.

A research network had the potential to increase the number of trials and build the critical mass for high quality multisite research. If the network represented all of the major treatment centres it would add to the power of studies by increasing the number of sites able to participate and the potential to recruit larger numbers of participants.

The network could also act as a more credible and effective advocacy group. The provision of a coordinated voice of respected clinicians and researchers could raise awareness of the issues with the general public, pharmaceutical industry and government policy and funding agencies. This could assist in

increasing the willingness to allow children to participate in research and greater infrastructure support for the conduct of research.

Partnerships between researchers and government agencies should be fostered to highlight issues and influence strategic directions, as well as facilitate youth participation the research process. Such partnerships could also promote recruitment to clinical trials and disseminate the findings from research to help families to make informed decisions concerning treatment options.

### **Resources and Requirements**

By providing a platform to bring researchers and clinicians together, a paediatric research network could promote a more efficient use of infrastructure and the sharing of research resources. The provision of a central database of researchers and research programs would assist early career researchers to identify potential mentors and to access training opportunities.

The establishment of a network is also likely to require a considerable amount of time and resources. A new structure would need to ensure that it did not duplicate or detract from existing associations. Any programs provided through the formation of a paediatric research network would need to compliment rather than competed with other initiatives.

While a network could provide a greater ability to strategically set research priorities and plan for increased research capacity, a centralised coordination system, however, may have the effect of over-riding local issues and needs. In addition, care would be needed to not to exclude the contribution of general practitioners who treat the majority of children and families as many children have minimal contact with major hospitals or children's institutions.

A plan for the formation of a paediatric research network would need to take these issues into account.

### **Next Steps**

It was proposed that in order to maintain the momentum from the Roundtable and to consider and deliver the next steps, OSMR will convene a working group. The three major paediatric tertiary treatment centres and neonatal care will be represented on the Working Group.

The key issues for the consideration of the Working Group will be:

- **Equity** – how to ensure the network structure has the appropriate inclusion of that represents the 3 Tertiary hospitals, 9 Neonatal centres, and the community sector.
- **Supervision** – to identify an appropriate organisation to act as the host for the network coordinator, providing the necessary infrastructure and support for the position.

- **Networking** – how best to provide the organisational support for membership in acknowledgement that membership takes time and resources.

One of the outcomes from the Working Group will be the development of a draft strategic framework for the research network including the proposed governance structure for consultation with the potential membership. The necessary mechanisms to ensure that the network captures and is responsive to clinicians, researchers, trials centres, consumer / advocacy groups, Aboriginal health research, industry professional bodies will need to be identified and articulated.

The appointment of a network coordinator will be a crucial first step in establishing the research network. The Working Group will be tasked with scoping the proposal for the potential appointment of an OSMR-funded project officer to establish a Network, including the position description and priorities.

The Working Group is likely to meet 3-4 times over the first nine months of 2009. It is envisaged that the Working Group will involve others as relevant to specific themes or strategies, and may co-op others to join sub-groups as necessary.

### **Potential Outcomes**

Paediatric populations require a better choice of treatments and interventions appropriate to their age and founded on the best available evidence of safety and efficacy. The NSW Paediatric Research Network will have the goal of facilitating research that will lead to the translation of findings into improved practice in the use of treatments across the paediatric spectrum of age ranges.

The formation of a NSW Paediatric Research Network would have several benefits for NSW including the establishment of a mechanism for the formal exchange of paediatric expertise, especially regarding research on medicinal products used for children. The Research Network could improve communication of data regarding medicines (in development) for children and facilitate research collaborations particularly in relation to multicentred research involving treatments for rare and low prevalence paediatric disorders.

A coordinated approach to research collaboration and infrastructure would enhance access to research leadership and technical skills to assist the conduct of clinical trials. The development of standardised protocols and data collection for multicentred trials will improve and facilitate the study of drug therapy, as has occurred in pediatric oncology. The Research Network could also facilitate a coordinated response to regulations and legislation that impact on the conduct of paediatric pharmaceutical clinical trials and the participation of paediatric populations in research.

## APPENDIX 1 PAEDIATRIC RESEARCH ROUNDTABLE ATTENDANCE

| Participant  | Organisation   |
|--|--|
| <b>Chair</b>   |  |
| Professor Jonathan Craig                                       | Sub-dean, Clinical Epidemiology, the University of Sydney, Westmead Children's Hospital                              |
| <b>Presenters</b>  |  |
| Professor Glenn Marshall                                       | Director, Centre for Children's Cancers and Blood Disorders, Sydney Children's Hospital                              |
| Professor Alison Jones   | Professor of Pharmacology, Chair Quality Use of Medicines Committee - Hunter Medical Research Institute              |
| Mr Gary Disher   | Director, NSW Cardiovascular Research Network, NSW Health Foundation   |
| Dr Wendy Hague   | Clinical Trials Program Director, NHMRC Clinical Trials Centre   |
| Dr Catherine Bourgeois   | Director, NSW Clinical Trials Business Development Centre, Cancer Institute  |
| Professor Alan Cass  | Director, Renal Division, The George Institute<br>Director, Poche Centre for Indigenous Health, University of Sydney |
| Dr Madlen Gazarian   | Head, Paediatric Therapeutics Program, School of Women's & Children's Health, University of NSW                      |
| <b>Westmead Research Precinct / Children's Hospital</b>        |  |
| Professor Robert Booy  | Head of Research, National Centre for Immunisation Research & Surveillance   |
| Dr Geoff McCowage  | Oncologist   |
| A/Professor Chris Cowell                                       | Head, Institute of Endocrinology & Diabetes  |
| Professor Elizabeth Elliott                                    | Director, Australian Paediatric Surveillance Unit  |
| Dr Kimberley Lischlikis  | Project Officer, Clinical Trials Unit  |
| Professor Kathryn North  | Neurogenetics and Professor of Paediatrics, University of Sydney   |
| Ms Anne O'Neill  | Research and Development Manager   |
| <b>Randwick Research Precinct / Sydney Children's Hospital</b> |  |
| Dr Moira Clay  | Associate Director, Children's Cancer Institute Australia  |
| Professor Anne Cunningham                                      | Director of Research, Sydney Children's  |

|  |   |
|--|---|
|  | Hospital, University of NSW   |
| A/Professor Adam Jaffe   | Head, Respiratory Medicine, Sydney Children's Hospital  |
| A/Professor Katrina Williams   | Head Clinical Trials Unit, School of Women's & Children's Health  |
| <b>Hunter Medical Research Institute / John Hunter Children's Hospital</b> |   |
| Dr Ian Wright  | Neonatologist, Kaleidoscope Neonatal Intensive Care Unit, John Hunter Hospital  |
| <b>Other Key Stakeholders</b>  |   |
| A/Professor Nick Evans   | Head, Department of Neonatology, Royal Prince Alfred Hospital   |
| Ms Megan Gleeson   | Business Manager, NSW Clinical Trials Business Development Centre   |
| Dr Mitch Kirkman   | Manager - Process, Training & Quality - International Clinical Research Operations Novartis Pharmaceuticals Australia |
| Ms Moyra Lewis   | Acting Director, Pregnancy and Newborn Services Network - University of Sydney  |
| Dr David Osborn  | Staff Specialist Neonatologist, Newborn Care Royal Prince Alfred Hospital   |
| Dr Dorota Pawlak   | Research Development Manager, Juvenile Diabetes Research Foundation   |
| Dr Jacqueline Small  | Executive Member, Royal Australasian College of Physicians - Chapter of Community Child Health                        |
| Professor John Simes   | Director, NHMRC Clinical Trials Centre  |
| Ms Rowena Tucker   | CEO, Diabetes Vaccine Development Centre  |
| Ms Diana Zannino   | Biostatistician, NHMRC Clinical Trials Centre   |
| <b>Government Agencies</b>   |   |
| Dr Gerard Cudmore  | Director Medical Research, OSMR   |
| Ms Suzanne Pope  | Principal Policy Officer, OSMR  |
| Ms Maj-Britt Engelhardt  | Manager, Policy, Commission for Children and Young People   |