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The Paediatric Trials Network Australia (PTNA) submission to the Strategic Review of Health and Medical Research in Australia

The Paediatric Trials Network Australia (PTNA, formerly known as the Australian Better Treatments for Children Research Network) welcomes the opportunity to provide input into the Strategic Review of Health and Medical Research in Australia.

The PTNA is a not for profit, virtual and inclusive network of paediatric researchers from around the country committed to improving child health through the facilitation of paediatric clinical trials. A formal governance structure for this network was agreed at a recent face-to-face meeting and will be implemented in the next few months.

The PTNA is in receipt of \$850,000 funding through Education Investment Fund's (EIF) plan for the Translating Health Discovery (THD) into Clinical Applications Super Science Project managed by Therapeutic Innovation Australia (TIA) to purchase and implement a clinical trial data management system. This project is well underway, with the Request for Tender for this system through NSW Health closing for submissions on the 4th April, 2012

(<https://tenders.nsw.gov.au/health/?event=public.rft.show&RFTUUID=88835461-EA34-CEC3-2FCA881BC36C7964>).

The PTNA would like to submit the following information for consideration in response to the questions raised in the Strategic Review.

- **Why is it in Australia's interest to have a viable, internationally competitive health and medical research sector? (Terms of Reference 1 and 6)**

It is estimated that for every \$1 that is invested in clinical research in Australia results in substantially greater benefit to the health of the nation (Access Economics, *Exceptional Returns: The Value of Investing in R&D in Australia*, A Report commissioned by The Australian Medical Research Association, 2003). Supporting paediatric clinical trial research activities provides even more benefit by ensuring the health care delivered to paediatric patients is evidenced-based and providing a good foundation for wellness in adults.

There is a paucity of evidence for the treatments that are effective and safe in the paediatric population. Medical research is critical to addressing this problem. There is a growing recognition that most chronic adult diseases have their origins in childhood, especially those diseases that are currently responsible for a never-ending expansion of the health budget, including: obesity and type 2 diabetes; cardiovascular disease and stroke; poor mental health, including behavioural disturbances and depression; and chronic respiratory diseases including asthma and COPD. High quality research in children that results in effective preventative

strategies represent the only hope we have of limiting the expansion in the health budget.

Countries around the world have recognised the importance of clinical research in improving health systems and the lives of patients, and are providing significant investment in this area. The multi-million dollar investment made by the UK into their Medicines for Children Research Network (<http://www.mcrn.org.uk/>) dramatically increased the number of investigator-driven and industry sponsored clinical trials run in the UK. This greatly increased the numbers of paediatric patients benefitting from participation in these trials. A similar investment in supporting paediatric research in Australia could be expected to generate similar benefits to the Australian community and paediatric researchers, and facilitate Australia being recognised as a world leader in setting standards for paediatric research and health care. The importance of increasing the access of children to effective, safe and quality medicines has been a priority of the Australian Health Minister's Advisory Council and recognised by the Department of Health and Ageing by the establishment of the Paediatric Medicines Advisory Group in recent years.

Australia must invest in medical research, and specifically clinical research, if we are to keep pace with other countries that recognise the value of this investment, to attract and keep high quality scientists and clinicians and to provide the best possible care to people cared for by our health system. An environment conducive for medical research contributes ensuring a culture of continuous improvement in our health system.

Conducting clinical trials requires a specialised skill set that is largely unrecognised, including knowledge of ever-increasing local/national/international regulations, data and project management skills, human resource and financial management skills, and ability to community with a broad variety of scientific and non-scientific/community stakeholders. This is especially true for investigator-initiated clinical trials. Our paediatric community leads the world in many areas and is setting the agenda globally, especially in understanding the mechanisms underlying childhood diseases. Thus, we are extremely well placed to mount clinical trials addressing fundamental questions about disease causality and cure. In general trials conducted by the pharmaceutical industry do not have this priority. It is only through preventative or curative strategies that the social and economic burden of chronic disease can be reduced.

It is important for there to be a career path for clinician researchers and research coordinators, and the time and funds available to pursue research. For many clinicians, protected research time is a dream, rather than reality. Most undertake necessary protocol development and grant writing in their own time and are heavily reliant on research coordinators with a scientific or nursing background to ensure smooth clinical trial operations. However, there is no defined career pathway for such coordinators within research sites, which means it is difficult to attract this workforce, adequately compensate them for the skills required, limited opportunities for advancement.

Knowledge-led continuous improvement, innovation and research is one of the stated levers of National Health Reform. The commitment to invest in health services, public health, health policy and health system research, and establishing clinical research fellowships across hospitals and primary health care settings will be enormously important in underpinning the growth of quality and quantity of research possible in the paediatric setting.

- **How might health and medical research be best managed and funded in Australia? (Terms of Reference 2, 3 and 7)**

Only a very small percentage of the NHMRC's annual research budget supports paediatric clinical trials, making it difficult to fund these logistically challenging trials being conducted often in already resource poor environments (public health systems and community practice). Despite the economic impact of many diseases which have their origins in the paediatric years, many of them (e.g. childhood obesity and its treatment) have struggled to obtain competitive funding, despite Australia having a large group of internationally recognised clinical researchers willing to do the studies.

Australia is different to other countries (like the US) in the relative lack of philanthropic giving as a source of research funding. The provision of incentives to individuals and companies to invest in R&D should be reviewed to identify ways of addressing funding shortfalls from the public sector.

Due to the small size of the paediatric population, particularly those with chronic illnesses compared to adults, there is a necessity to engage nationally and internationally in order to recruit enough volunteers with specific medical problems to achieve scientifically valid conclusions. Clinical trial networks are therefore especially critical in paediatric research for establishing critical mass, attracting and efficient use of infrastructure, creating linkages with external partners (such as government, industry, international partners) and acting as a central contact point for efficient communication and coordination of multi-centre trials. Clinical trial networks in Australia are largely dependent on the goodwill of partnering researchers/institutions for success. Providing funding to support the administration and capacity for research within these networks will lead to greater long-term success in delivering outcomes for patients, and in the case of the PTNA, to our children.

The current approach to funding clinical trials on a project or program basis can lead to different research groups re-inventing the wheel to train/understand/implement regulatory requirements, and the potential loss of staff that have developed this knowledge at the end of grants. There would be enormous efficiencies in staffing and process achieved by establishing centralised clinical trial centres at major centres conducting clinical research with pooled staff (investigators, coordinators, statisticians, data managers, etc) that can be moved between projects at times of low or high workload on a project, that retain and improve knowledge over time, and have the critical mass to provide a research career pathway that may not be achievable within individual research groups.

In particular, the availability of centralised resources will also create increased capacity for small groups or inexperienced researchers interested in participating in clinical trials an access point to do so.

Just as such centres can improve practice within an organisation through the sharing of best practices across groups, creating a facility for such clinical trial centres to network will more effectively and rapidly increase research practices across the country, potentially increasing recruitment capacity, reduce the risk of poor research practices and increasing funder, institutional and community trust in the research being undertaken.

In respect to community and consumer participation, the pathways by which the community and researchers engage are unclear. There are also no publicly available/funded resources to help educate consumers on how to be involved in the research enterprise, whether as clinical trial volunteers, or contributors to the

development or review of research projects. Funding such educational and training programs for consumers will have broad impact on increasing engagement of consumers with research. Additionally, it is important to recognise that children and young people may have their own interest in contributing to or volunteering in research. As such any educational materials created should address the needs of this population group to understand and engage in research.

Providing paediatric patients with a good research experience early on could help inspire the next generation of clinicians and researchers, and/or decrease fear of participation in research as adults, thereby facilitating research involving children and adults in the future.

- **What are the health and medical research strategic directions and priorities and how might we meet them? (Terms of Reference 5, 12 and 13)**

Developing skills and best practices for research conducted according to Good Clinical Practice guidelines in small populations is critical in the paediatric space, but will be increasingly important for other researchers in the future. As we move to an era of personalised medicine, all trials will face the difficulty of recruiting from small population bases, geographically spread. Developing clinical trial networks able to efficiently access electronic infrastructure and thereby support virtual teams will be necessary to increase capacity for recruitment geographically and streamline clinical trial operations and efficiency.

Wellness as a child and young person creates the foundations for a more healthy adulthood. A healthy start to life is a national research priority area yet limited funding is directed into this area. Ensuring there is solid evidence for how the paediatric population is cared for will give them the best start to life and reduce the long term impact to the health service into adulthood. The PTNA supports the elevation of paediatric care to its own national health priority, and by default national health and medical research priority. Many paediatric treatments currently used in our health system are being used outside their licensed indication as they have been only studied in adults or not in a particular paediatric condition. Children are physiologically not little adults, with different vulnerabilities and susceptibilities that affect both treatment effectiveness and safety profile. As such, it is important that the treatments provided are adequately researched for use in different age groups. The increased emphasis by regulatory authorities in obtaining better evidence for the safety and effectiveness of new treatments demonstrates the important emphasis being placed on paediatric research. By elevating paediatric research to a national health priority, it will facilitate the funding of paediatric research in Australia against the important adult health priorities also identified.

The funding of clinical trial fellowships and training, establishment and funding of paediatric clinical trials centres and clinical trial networks, and education of the community to demystify the clinical trial process and protection, and necessary role they play as participants of clinical research will go a long way to ensuring any project specific funding awarded in the future will be used most efficiently.

- **How can we optimise translation of health and medical research into better health and wellbeing? (Terms of Reference 4, 8, 9, 10 and 11)**

Any investment in developing research skill and capacity can also be leveraged to attract continued investment of multi-national pharmaceutical and medical device

companies, providing additional jobs and opportunities for research. In the case of paediatrics specifically, there is an increased regulatory requirement for the conduct of paediatric studies for new treatments, which represents a growth opportunity for Australia. Currently Australia has a very small share of commercial paediatric clinical trial activity. By investing in our paediatric clinical research infrastructure, Australia could exploit this market opportunity and attract increased investment in research and the health service from the conduct of these trials.

Research is critical to the national health reform objective of creating an agile and self-improving health system. Improving the capacity for research within our health system provides the opportunity to develop research skills and experience in our paediatric clinicians, attract expert clinicians to our health system, and develop a culture of continuous improvement within the health service.

The Commonwealth's e-health reforms provide enormous opportunities to increase research capacity and community engagement in research. Any efforts should consider the capacity for e-health systems to integrate with systems collecting research data and sample, store large volumes of data that can be accessed efficiently and quickly, and engage with, recruit and monitor clinical trial participants. Any discussion of e-health should also consider the opportunity presented by social media channels to improve engagement, understanding, and participation in research by the community.

- **Key recommendations**

The PTNA would like to offer the following key recommendations:

- That clinical research be recognised as an integral component of any advancing, developed health system, as demonstrated by the provision of funding for protected research time for clinician researchers within our health services
- That the invaluable role played by supporting staff be recognised with the creation of a defined career path for clinical trial coordinators.
- That there is financial support to the development of national clinical trial networks and local clinical trial centres that can improve the efficiency of resources, the sharing of best practices, and a facility for developing clinical research careers. The clinical trial network initiatives in the UK (for e.g. <http://www.mcrn.org.uk/>), Europe (add linkage) and locally, the cancer and kidney networks are excellent examples of the added value to health brought about by structured investment in clinical research.
- That child health is elevated as a national health priority, and by default, a medical research priority.
- The national health and e-health reforms are leveraged to facilitate research, particularly where this can facilitate data collection, trial recruitment and social engagement with the community.
- That consideration should be given to the development of innovative partnerships and funding models, or legislative changes that encourage greater investment in paediatric research and development by the commercial sector and greater investment. This could include, for example, provision of tax incentives, a change to the regulatory framework and requirements for the licensing for paediatric indications, in particular in the case of orphan drugs, or where the drugs and their side effects are known, or the drug has high level evidence supporting its use in a given disease.

Thank you in advance for your consideration of the above. Please do not hesitate to contact the PTNA should you have any questions.

Kind regards

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