



11 April 2012

Mr Simon McKeon  
Chair  
Strategic Review of Health and Medical Research  
McKeon Review Secretariat PO Box 4226 MANUKA ACT 2603

Dear Mr McKeon

Children's Cancer Institute Australia (CCIA) welcomes the opportunity to make a submission to the Strategic Review of Health and Medical Research in Australia. We believe that this is a most timely and critical initiative for the future prosperity of our sector and its ultimate goal, improved health outcomes for all Australians.

CCIA is the only independent medical research institute in Australia dedicated to research into the causes, prevention, better treatments and ultimately a cure for childhood cancer. Our vision is to save the lives of all children with cancer and eliminate their suffering. Our mission is to be a leader in preventing cancer and finding ways of curing cancer in children through world-class research. CCIA is located in a new state-of-the-art facility, the Lowy Cancer Research Centre, at UNSW's Kensington campus. It is one of the leading cancer research centres in the world, integrating childhood and adult cancer research.

CCIA is a member of the Association of Australian Medical Research Institutes (AAMRI) and has contributed to an extensive response by this peak body to the Strategic Review. In addition to the AAMRI submission CCIA wishes to highlight some key issues of particular relevance to its own mission.

Clearly, given its size and investment in the health and medical research sector, Australia is performing exceptionally well by international comparisons. Hence, in principle, we believe that solid foundations have been laid for the future growth of the sector. There are however identified shortcomings and opportunities that we would like to outline in response to the questions raised by the review panel:

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

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

Over the past three decades we have pursued our vision by building a critical mass of world class research within an environment that closely integrates basic, laboratory based science within a childhood cancer clinical service delivery framework to ensure maximum and rapid benefits for the health and wellbeing of children with cancer. This research-driven approach to treatment and care developed by CCIA and like-minded organisations in the USA and Europe has resulted in a dramatic improvement of survival of children with cancer

from the dismal survival rates pre-1960 to the present time, when well over 70% of children with cancer are cured - in many ways making it a model for an innovation driven, evidence based, self-improving health system. In fact within the field of paediatric oncology, clinical trials have become the standard of care and are fully embedded in clinical service delivery rather than an "optional extra". There is good evidence that this high level of participation in clinical trials (approximately 60% of patients) is a key driver in the tremendous improvement in childhood cancer outcomes. It is also clear that research has a critical role to fulfill in reducing health care costs. Today approximately one in nine hundred adults is a survivor of childhood cancer, and two thirds of them are suffering from long term, significant side effects of their treatment that require ongoing costly medical care, for illnesses including cardiac disease, metabolic disorders and even therapy-induced second cancers. There is no doubt that the development of safer, targeted drugs, through focused medical research effort, is a key prerequisite for avoiding those outcomes of childhood cancer treatment, and hence delivering major cost, community and individual benefits.

As a specific example of how medical research into childhood cancer can improve health outcomes and deliver huge cost savings, CCIA researchers have developed a novel diagnostic process called Minimal Residual Disease testing, which can predict which patients diagnosed with the commonest childhood malignancy, Acute Lymphoblastic Leukaemia, are at major risk of relapse and are hence in need of more intensive targeted treatment. A recently concluded clinical trial of over 550 Australian children with this disease has shown nearly a doubling of the survival rates in children at greatest risk of relapse, with relapse having been avoided in most patients, thus sparing them and the health system many weeks of costly inpatient treatment. While clearly not all the innovations that have led to improved outcomes for children with cancer have emanated locally, there can be no doubt that CCIA's reputation and capacity for world class research has enabled us to collaborate globally and rapidly implement research findings wherever they are made, as well as pioneering our own major research and clinical innovations.

Another specific area where CCIA has developed significant capabilities with major potential for translating the benefits of medical research into better outcomes, is the field of drug discovery, where the Institute has been successful in attracting substantial funding for capital infrastructure from a number of sources including the Australian Cancer Research Foundation, the Commonwealth's Super Science program as well as the Cancer Institute NSW. This support has enabled CCIA to establish a unique national infrastructure platform, to conduct high throughput screening of chemical small molecule libraries with the aim of developing the next generation of safer, more effective molecular targeted therapeutics for the treatment of children, and also adults, with cancer. While the Drug Discovery Centre is thus now equipped with state-of-the-art world forefront instrumentation, it, like many other national infrastructure facilities, faces the challenge of attracting funding to support the highly skilled specialist staff who are needed to operate these facilities, since there is a critical shortage of funding for such purposes. We believe that it is absolutely paramount that costs for non-physical infrastructure (ie human capital) to be allowable under national infrastructure grant schemes such as Super Science in order to maximize the benefits arising from such key capital investments.

Despite the encouraging trend in improved health outcomes for children with cancer, such progress can only be sustained if a coherent strategic support framework is established that can drive translation across all aspects of the continuum of discovery to clinical practice. We believe that additional targeted infrastructure investment as well as career development support is required to enhance and deepen these collaborations within and across research

hubs. Careers in research remain highly competitive and opportunities are limited - particularly for researchers 5-7 years from completion of their PhD. Further emphasis is needed on providing career support schemes that will ensure continuity and security of funding for the establishment and subsequent maintenance of careers. In addition, dedicated research time for clinicians will also be necessary to enhance capacity and generate critical mass for research translation. Indeed we believe that there needs to be a more rigorous assessment of and commitment to investment in research within the health system itself as a key partner in the translational continuum. While the recent health reforms have flagged federal funding support for research within the hospital sector, to date little evidence can be observed of increases in research investment within hospitals.

As a specific example of the inadequate research investment in the hospital sector, funding for clinical trials nurses and data managers within the paediatric cancer field remains largely dependent on community fundraising, which is inevitably exposed to significant fluctuations in line with the economic climate of the day. This results in an unacceptable degree of uncertainty and instability for such a critical segment of the health workforce, and we believe that increased public funding for such positions as an integral part of health services would contribute significantly to the further growth of paediatric clinical trials, and hence improved outcomes. Similarly, funding for clinical trials conduct (particularly for industry-independent studies) remains scarce, posing a critical roadblock to the further expansion of trials activity. This lack of funding is of particular concern to the paediatric cancer field where there is only limited industry investment into the development of improved treatments, due to the small patient population and hence limited financial returns to pharmaceutical companies, and therefore a heavy reliance remains on investigator initiated and collaborative trials group sponsored studies. Current funding levels supported by the NHMRC are clearly insufficient and need to be increased to stimulate further growth within the sector, and ultimately, to lead to more rapid implementation of evidence into clinical practice, which is absolutely critical, particularly given that childhood cancer remains the most common cause of death from disease in children in this country.

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

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

A key issue for the sustainability of independent medical research institutes such as CCIA is the adequate, secure and long term funding of indirect research costs. There needs to be closer collaboration and engagement between the Commonwealth and the States to ensure that funding mechanisms are synchronized and complementary to cover these costs at the same (adequate) rates across the sectorial variations currently in place. Ideally such indirect costs would be incorporated as a loading on top of the direct research costs awarded to the applicant organization. Furthermore, we view it as absolutely imperative that collaboration is enhanced between funding bodies, which are currently highly fragmented, to avoid duplication and maximise leverage of the research strengths inherent across the nation. A more strategic approach towards a nation-wide coordination of efforts within the field will be critical to ensure that limited resources are allocated in the most effective and efficient manner. We view it as imperative that key funding mechanisms are resourced adequately both in terms of their administration as well as their available grant funding allocation. As noted above, current funding levels are clearly insufficient to sustainably grow the sector, hence a number of strategies should be pursued to facilitate increased

investment, including stronger incentives for philanthropic giving, a more structured approach to promoting Australia internationally as an attractive investment destination for the biotech and pharmaceutical industries, as well as increased public funding. National harmonisation of regulatory processes (particularly for the implementation of a process for the ethics approval of multi-site clinical trials) is also a high priority to improve efficiency and competitiveness of the HMR sector.

Ultimately investment in health and medical research should be outcome oriented and help facilitate a self-improving health system that is driven by a culture of innovation, knowledge generation and its effective and timely application. In order to achieve this it is critical to:

- Continue to fund excellence and focus on areas of existing strengths - yet be responsive to new opportunities and promising emerging fields and approaches such as personalised medicine.
- Facilitate the closer link and rapid translation of research into clinical practice by providing support for effective translational pipelines that link basic research to clinical services and vice versa.
- Encourage efficiencies within the sector and provide incentives for leveraging of research investment

CCIA commends the work of the Review Committee, and looks forward to continued engagement in framing the future direction of health and medical research in Australia.

Yours sincerely

Dr Peter Wejbora  
Associate Director