Improving the treatment and the lives of patients with blood cancers via clinical trial research

Detailed submission: Strategic Review of Health and Medical Research from the Australasian Leukaemia and Lymphoma Group (ALLG)

EXECUTIVE SUMMARY

Health and medical research can be functionally divided into research whose worth is judged by the new knowledge it creates and the ideas for better health that flow subsequently (fundamental research and early translation), and research that is explicitly addressing questions of immediate application to the health of individuals or the health of populations or the performance of the health care system. Clinical trials research typically falls into the latter category, and represents the engine that generates the evidence for best practice in health care in Australia.

In addressing the terms of reference we consider critical points for consideration are that:
1. Clinical trials research must be undertaken to ensure Australia remains a viable research competitor in the national and international markets.

2. Health and medical research will benefit from a separate funding stream, specifically to address matters of clinical trial research. The government could consider a number mechanisms of collaboration and networking to oversight a comprehensive, financially viable and expertly managed national research program.

3. Decisions for future strategic directions must be reviewed and implemented systematically, with the appropriate mix of private, public and industry representation in the process. Current research limitations need to be address so as to avoid future barriers in the conduct of research activities.

4. Application of blanket rules and policy to clinical research setting impairs translation implementation to the health and research sector. Not for profit (NFP) organisations are very focused on community needs, and as such are instrumental in the implementation of health reform that delivers rapid translation of research results to the health care environments for the benefit of the Australian community.

As a leader in the area of haematological clinical trial research, we believe matters of the highest priority are:

- Identification of appropriate funding models to support clinical trial research
- Implementation of appropriate funding models to support clinical trial research
- Removal of inappropriate legislative and bureaucratic barriers where possible
- Redressing the trends for greater reliance on industry to initiate and fund clinical trials at the expense of co-operative group- or investigator-initiated trials addressing public good questions, by bolstering Governmental funding and infrastructure support for quality clinical trial activity
- Linking clinical trial outcomes with implementation by ensuring government and co-operative trials groups interact more effectively not just as funder and researcher, but as commissioners of research and advisors on regulatory change necessary for practice change.

Clinical trials provide not only an immediate benefit to some patients through accelerated access to new therapeutic approaches, but also go part way to the generation of a framework for the delivery of health care that optimises care pathways, skilled labour forces and infrastructure. Our expert advice and comments for consideration by the panel are accompanied with proposals for their implementation.

Q1. Why is it in Australia's interest to have a viable, internationally competitive health and medical research sector? (Terms of Reference 1 and 6)

1. The need for Australia to build and retain internationally competitive capacity across the research spectrum, from basic discovery research through clinical translation to public health and health services research.

6. Strategies to attract develop and retain a skilled research workforce which is capable of meeting future challenges and opportunities.

Cancer is the leading cause of disease burden in Australia and is becoming more prevalent with the aging population[1]. Blood cancers are some of the most commonly diagnosed cancers and accounted for the second most common cause of cancer death after lung cancer (7348 deaths) in 2005. In that year, more Australians died from blood cancers (4248 deaths) than colorectal (3801 deaths), prostate (2952 deaths) or breast cancer (2643 deaths)[2]. Blood cancers are among the most expensive cancers to treat and many types of blood cancers remain incurable. AIHW data shows that myeloid and lymphoid cancers collectively rank as the 3rd most common cancer affecting Australians in terms of incidence and cancer related deaths. The ALLG is the peak clinical trials body supporting blood cancer research in Australia.

Advances in the detection, diagnosis and treatment of blood cancers are vital to improve outcomes for people suffering from these diseases and to reduce the economic and social impact of blood cancers on Australian society. Clinical trials are the most effective way to evaluate new approaches to all aspects of cancer control, including prevention, screening, diagnosis, treatment, health service provision, supportive care, palliative care and psychosocial impacts, and are crucial for providing an evidence base to support changes in practice. However, it is painfully clear that there is a gulf between the generation of evidence and its implementation. This gulf is typically greatest where the evidence from trials appears remote to the Australian experience, either because it is less relevant to the Australian population or practically a poor fit with Australian health care delivery. For this reason, **clinical trials involving Australian patients and being conducted within the Australian health care system are crucial for the rapid application of meaningful advances in cancer management and for maintaining best practice high quality health and medical care in Australia.**

During the 1990's and 2000's there has been greater emphasis by governments on research and development (R&D) and retaining Australia's competitive reputation is crucial for our R&D prospects and investments of the future. Australia's proportion of GDP devoted to R&D is still below that of leading countries in Europe and North America. Given the present global pessimistic financial outlook, future business spending on R&D will come to a stand still if we are not able to, as a nation, commit ourselves to those core research activities of such as clinical trials. Clinical trials provide not only an immediate benefit to some patients through accelerated access to new therapeutic approaches, but also go part way to the generation of a framework for the delivery of health care that also optimises a diversely skilled labour force and infrastructure.

[1] Australian Institute of Health and Welfare 2010. Australia's health 2010. Australia's health series no. 12. Cat. no. AUS 122. Canberra: AIHW.

[2] AIHW (Australian Institute of Health and Welfare) & AACR (Australasian Association of Cancer Registries) 2008. Cancer in Australia: an overview, 2008. Cancer series no. 46. Cat. no. CAN 42. Canberra: AIHW.

To illustrate how clinical trials research in Australia can both contribute substantially to global knowledge and expedite transformation of health care and health outcomes locally and internationally, we are proud to provide the example the ALLG's investment in Chronic Myeloid Leukaemia (CML) research. This has produced:

- Over 40 publications and presentations of clinical trial results that have made the case for fundamental change in the care of CML patients from old-style chemotherapy and bone marrow transplantation to uniform use of targeted therapy with what was a new class of anti-cancer drug - tyrosine kinase inhibitors.
- Multi-million dollar investments by global pharmaceutical companies in Australian research, expanding our market as a viable research environment.
- Guidelines and education exercises that facilitated change in clinical practice for CML in Australia, and internationally based on the outcome of the clinical trials.
- Rapid availability of new medicines on the PBS, based on the obvious applicability of clinical trial data generated in Australia, and facilitated by engagement of Australian clinical trial researchers with the PBAC.
- Refinement on a regular basis of PBS restrictions to maximise benefit and minimise costs in an environment of expensive new drugs and rapidly evolving clinical trial evidence.
- Outstanding success with new laboratory assay development, driving rapid uptake and MBS-funding for tests that have real-time impact on treatment decision and pathways.
- Absolute reduction in the health care dollar spent associated with diagnosing and, treating CML. i.e. with transforming treatment from costly bone marrow transplant care which involves high cost drug usage and lengthy stay in hospital, to treatment with oral medication and attendance to day clinic attendance.
- Ever increasing improvements in the survival (doubled, and still improving) and quality of life for patients with CML compared with standard care one decade ago.

This example in CML research confirmed our international competitive edge; it brought with it admiration from the international community of our capabilities to conduct high quality, intellectually-independent, clean, notable research. The international community has high regard for how Australian physicians diagnose, treat and monitor CML. Despite these successes that demonstrate how application of clinical trials improves patient outcome, maximises efficiency in health care resources utilisation **major barriers exist within the national health framework for effective clinical trials research and these must be addressed in the interest of our community and our researchers.**

a) **Current funding models are ill suited to enable major clinical trial research and threaten Australia's capacity to enter and sustain competitive position globally, and viability of clinical trials consortia.** The current NHMRC model of funding has the effect of limiting severely the chances of a successful application for clinical trials. Trials conducted by the ALLG usually are often more complex than for other cancers, multiple modalities may be employed including chemotherapy, immunotherapy, bone marrow transplant, radiotherapy, surgery and other modern treatments such as epigenetic therapy. Samples of blood and bone marrow are required for scientific studies and tissue banking is routinely embedded in ALLG trials with associated logistical complications. As a consequence of these complexities blood cancers do not fit well within the NHMRC assessment algorithm and do not appear to be well understood by the grant review panels. This added difficulty of obtaining funding from the NHMRC can result in the delays to commencement of trials or even the abandonment of well developed and scientifically important concepts to the detriment of patients in Australia.

Proposal: *The ALLG proposes the panel look at the National Institute of Health (in the USA) as a model of where clinical trialists are utilised in the review panels in clinical trial research applications.* *http://era.nih.gov/roster/*

b) **The demand on limited resources for conduct of clinical trials is ever expanding as a result of increasing requirements of the regulatory environment.** Applications of regulatory conditions add considerably to the workloads of clinical research and administrative staff. This applies additional stressors on the workforce both in the hospitals and in the central administration of the collaborative groups. The workforce in the future will need to be adaptive but also appropriately trained and remunerated to be retained in the clinical trial industry. From 1995 to 2011 the health industry remains under the average wage earnings compared to that of the other industry earnings such as mining, government, and education (IBIS World 2011). Appropriate remuneration for the increased responsibilities in regulatory compliance activities will be critical to retain the current workforce, and to ensure that workloads of the future are adequately allocated in the health and medical research sector.

Proposal: *Review of changes in the scope of work undertaken across clinical and administrative roles in order to fulfil regulatory compliance is critical. The ALLG proposes the panel consider the variation in health and medical research position descriptions and actual position activity across the health sector; this could be undertaken via surveying the clinical trial workforce regarding their role, responsibilities, and remuneration. This data collection could be performed at a national level or at a state level.*

c) **In the spirit of advancing academic endeavours those individuals assisting with research are often donating large amounts of time, whilst hospitals themselves absorb many costs into their already over-strained systems.** Patients for blood cancer trials are recruited at participating hospitals that treat haematological cancer. Many of the hospitals have to limit the number of trials open to patients due to governance, staffing and budgetary considerations, thus restricting the number of eligible patients who would otherwise be offered the possibility of participation in trials. This has a flow on effect of slowing the rate of recruitment and thereby extending the time taken to accrue the target number of patients, and therefore delaying the generation of evidence vital to improving health care delivery. In the UK, government policy includes a target number of patients to participate in clinical trials, and provides financial compensation for high level clinical research performance. The ALLG provides some funding but is unable to offer hospitals adequate financial support that could better enable maximal trial participation.

Proposal: *ALLG relies heavily on its membership, which is made up of most of the Haematologists in Australia and New Zealand who largely provide their services on an honorary basis, to conduct its clinical trials. The ALLG proposes the panel recommend that government funding for clinical care includes an identifiable component for associated research and to determine this extra investment that a review is established to identify the reasonable costs associated with clinical trial activity in hospitals and community settings.*

d) **Projects addressing future health are expected to be complex and those carried out in the Not for Profit (NFP) sector are not likely to have the adequate resources to structure, monitor and analyse them.** The ALLG, as a NFP organisation, frequently has to make its priority sourcing funding for the basic implementation of clinical trial coordination tasks. The lack of government funding for the core conduct activities leaves many research projects waiting excessive time, at risk of failure due to delays. A prime example of this matter is the ALLG's own experience with Bone Marrow Transplant (BMT) research. There is a clearly identified need to address a number of scientific matters in the realm of BMT; however for over 6 years the ALLG has been unable to open a

randomised BMT trial. Seven protocol concepts have ceased development due to either lack of funding source, or failure of the regulatory environment to adopt standard of care therapeutics into practice resulting in inability to access standard medications in the context of a clinical trial conduct. The ALLG, as NFP, has lacked the necessary resources to assist researchers in progressing matters to the government funding agencies and regulatory departments.

Proposal: *The ALLG suggests the panel recommend the NHMRC or a new funding body tasked with funding immediately-applicable health research fully funds the cost of conducting large-scale public good research conducted by the NFP sector.*

Q1 Summary:

Why is it in Australia's interest to have a viable, internationally competitive health and medical research sector?

The pursuit of a viable and internationally competitive health and medical sector will ensure that Australian patients have access to a health care system reflective of appropriate interventions and treatment options relevant for our population. This pursuit needs to be continued at its current high quality health practice and medical service standard.

Clinical research must be undertaken:

I. under the auspice of a functional, national structure
II. with application of pertinent regulation
III. together with appropriately resourced funding partners
IV. by way of appropriate trained, skilled, and supervised staff

Importantly, the ALLG would like to emphasise that any **reforms need to be mindful of the large number of volunteers working for NFP's, and the significant number of hospital staff performing research in their own unpaid time**. Thus future reliance on Australia's perceived viability as a nation capable of producing and sustaining competitive health and medical research should not be reliant on this contribution as given.

Q2. How might health and medical research be best managed and funded in Australia? (Terms of Reference 2, 3 and 7)

2. Current expenditure on, and support for, health and medical research in Australia by governments at all levels, industry, non-government organisations and philanthropy; including relevant comparisons internationally.

3. Opportunities to improve coordination and leverage additional national and international support for Australian health and medical research through private sector support and philanthropy, and opportunities for more efficient use, administration and monitoring of investments and the health and economic returns; including relevant comparisons internationally.

7. Examine the institutional arrangements and governance of the health and medical research sector, including strategies to enhance community and consumer participation. This will include comparison of the NHMRC to relevant international jurisdictions.

1. Financial issues

a) Globalisation: Globalisation does not always result in benefits for Australia. For example some aspects of expected return in international research and global industry involvement have diminished Australian researchers' intellectual property, and acknowledgment as prominent in research results. The impact of the global financial crisis (GFC) leaves us in a difficult situation. The cost of drug development has significantly increased over the past 2 decades. From 1993 through 2004, industry reported a 147 percent increase in research expenses (Pharmaceuticals Industry Council 2009). Global drug companies who have in the past supported our investigator initiated trials have in recent times been delaying negotiations or cancelling committed funds. This exposes projects to a refinancing risk, most of which is mostly irrecoverable. Thus despite time spent in feasibility analysis and design, we are still at risk of failure as a result of international markets. **Without continued and increased investment in health and medical research, Australia's ability to offer its citizens improved treatments and other benefits of clinical will be weakened.**
The ALLG, with 40 years of clinical trials experience, can evidence the value to Australia of our retaining greater control and input to managing and funding research. Although the input of global pharmaceutical companies will always be required, other areas of the clinical research process should remain as much as possible within local hands.

b) Privitisation: Some governments believe opportunities exist to free-up taxpayers' money through privatisation of health assets. The implementation of such strategies should not occur to the detriment of control of service or income generated. Greater engagement with the private health sector should be explored by the government. An integrated (public/private) set of plans to include the private sector in research would be of value as there is certain benefit to the private sector to contribute to research outcomes. Private institutions often have the infrastructure that would compliment the conduct of research activities such as clinical trials. **The economic benefit that clinical trials produce would assist in alliances between the private and public with regard to best practice and standard of care services.** This should be fully set out and the concept of private/public sector research alliance should be explored.

There are possibilities for the government to develop partnerships that capture the 'value' (investment) of multiple funding sources, inclusive of public and private sector, philanthropy and industry investment.
A model whereby private health insurers provide a percentage of an annual premium to the federal government to pass through to clinical trial researchers would both 1. ensure a consistent capture of funds from the private insurers and 2. expand clinical trial participation in the private sector. It could be

suggested that private health funds should be required to contribute to the funding of clinical trials as they are also a major beneficiary of the outcomes i.e. research activities provide results that improve standard of care and reduce health care costs.

Proposal: *The ALLG suggests the panel consider models to ensure research in Australia continues to be led by Australians, and examine prospects between the public and private sector that could recover funding shortfalls of future research.*

2. Funding models and sources

a) Collaborative schemes: Currently, investigator initiated ALLG clinical trials are funded through government (NHMRC, Cancer Australia), philanthropic, commercial and international (non-Australian funding agencies) sources. There is little consistency in access to these funders, and groups such as the ALLG in particular struggle to attract any state based research funds as we are committed to multi-site multi-state research. Health and medical research could be best funded in Australia with the continuation of such schemes as the Priority- driven Collaborative Cancer Research Scheme (PdCCRS). This scheme functionally works well as it pulls together nationally all interested parties to the one theme of research, in this case cancer. It utilises an NHMRC framework of assessment, which is a means for researchers to have aspects of their research considered in a research sense and a disease speciality. However, there are significant limitations with the process and NHMRC scoring framework, which in our experience has not yielded success of recent times.

b) Philanthropy: While philanthropic foundations can be a source of funding for medical research, the situation in regard to such foundations in Australia is not very amenable to them as a strategic funding stream. There is no public reporting requirement for charitable foundations in Australia and therefore comprehensive information is not available making it difficult to estimate the size or total disbursement. Philanthropy Australia estimates, however, that there are over 5000 foundations in Australia disbursing $25 million - $1 billion annually. They indicate that approximately 80% of foundations are in Victoria due to past tax incentives in that state and point out that many trusts are legally limited to making grants within their state of origin. In other cases foundations state that the applicant organisation must be based in Victoria and the project must benefit only people within Victoria. Moreover, foundations must be guided by wishes of individual benefactors as expressed through Will or trust deed (the legal document by which a foundation comes into being). Some foundations can fund all organisations which are charitable in nature, others must fund charitable purposes.

The ALLG, like many other NFP organisations, is constantly trying to reach the right match in the philanthropy and foundation sector. Often philanthropic groups restrict funding to particular community agendas, and do not include medical research in their funding portfolio. Philanthropic groups expect the government to be investing in medical research and will therefore only consider a medical research application if it is accompanied by evidence of government support. This could be due to organisational limitations in terms of being able to assess medical research applications and/or understand the science underpinning the request for funds. For this reason it is critical that there be dialogue between national and state based philanthropic groups and the government research funding agencies. This is a good example of where government funding of research could result in increased leverage of other sources. The government could assist with providing materials and resources to both researchers and philanthropists to adapt and utilise to their given area of research e.g. website and social media supports, online access to frequently asked questions.

c) Fundraising and partnerships: As a result of the highly competitive nature of the grants world, fundraising for commencement and continuation of basic research is a must. However, researchers are often not professionally qualified, trained or resourced to do this. Furthermore, time restraints often limit the attempt. Researchers, unless they are affiliated with large consumer based community fundraising associations, will struggle to achieve successful financial outcomes of any fundraising venture.

A second problem is that the financial structure of hospitals often makes it difficult for researchers to acquire and administer donated funds. There must be better support given to researchers to tap into the fundraising markets, and then to be able to utilise this funding in real-time for their research.

From a different standpoint, philanthropists need to be able to direct financial support directly to those in research. Often consumers are seeking the tangible endpoint of their donated funds, and the research environment does not lend itself easy to promoting and marketing this. Consumer programs within the health system and research organisations need to be equipped to educate consumers on the modes of research to demonstrate to all fundraisers the high value and worth of their individual or collective gift.

Proposal: *There is great opportunity to draw together research groups with community, philanthropy, charity, and foundation groups of like interest. The ALLG suggests the panel recommend the establishment of networks that are aligned to areas of particular interest that will in turn be of support and advocate for research in that particular domain e.g. cancer research groups linked with philanthropic groups that are directly interested in supporting cancer research.*

3. Research issues

a) Investment in researchers: Investment in Australian health and medical research is crucial for maintaining best practice high quality health and medical care and also yields social and economic benefits. Loss of labour resources results in unused industry capacity and nationally we are at risk of this if Australia does not make efforts to invest in funding training and post graduate positions in the research industry. Government researchers as a proportion of national GDP has slowly declined from 10.4% in 2004 to 10.0% in 2006 to 9.0% in 2008. (OECD R&D Stats 27April2011). Clinical trials play a pivotal role within the larger field of medical research, as it is only via the pathway of trials that the breakthroughs in understanding achievements in the laboratory lead to changed clinical practice. Without investment in clinical opportunity for researchers the investment in more basic research is effectively wasted. **Investment in researchers and opportunities to pursue new innovative lines of research will attract and retain the most talented staff, thus maximizing the chance of successful research outcomes.** Greater government investment in clinical trials will return greater potential for leveraging supplementary funding from other sources for those researchers and their future projects. They will also need to be supported by modern sophisticated technology in order to use human resources as efficiently as possible.

b) Supporting clinical research as an integral part of health care delivery:
Unlike laboratory research, public health research and health economics research, the majority of clinical research is led and conducted by individuals who are principally employed to deliver health care. The current NHMRC people support programs either do not apply to such clinically based researchers or they are non-competitive for funding when competing against full-time research staff. An even greater problem is that major health care providers (DOHA, State DOHs, Teaching Hospital Boards and CEs) do not recognise the value of clinical research in tangible ways, such as protected time for research, infrastructure funding. In some ways clinician researchers have been disenfranchised in the last two decades within major teaching hospitals etc. Yet they are the champions for change that must be cultivated within the health care system to bring about evidence-based improvements to health care delivery.

To reinvigorate clinical research, a culture that ensures that research is an integral component of good clinical care and health services delivery must be re-established. To do so in a sustainable fashion will require that under specified arrangements, funding for clinical services will include (or attract) an element (supplement) for related research. For example in hospitals, this funding would be dedicated to clinical research by clinical teams, and quarantined from the funding for direct patient care. To assess the effectiveness of the use of such funds, research KPIs would need to be instituted for hospitals, hospital management, and clinical teams. Such funding could be competitively assigned initially based on recent track record, and adjusted according to performance.

Proposal: *Better coordination and output of research can be achieved with adopting incentive and compensation for institutions that undertake clinical trial research. The ALLG recommends the panel review the current environment of research at a hospital base level, and consider factors that may accelerate and enhance clinical trial conduct. International comparisons of investigator initiated trials funded by government and other sources have had relative success, the NIH model in the UK is a good example of this.*

Q2 Summary:

How might health and medical research be best managed and funded in Australia?

The ALLG stresses that there is a need for funding for clinical trials, that clinical trials are in need of financial support to coordinate the essential development and conduct of research i.e. statistical advice, trial data management. Economic return on investment made, could be literally achievable if the Australian government were to take the lead in directing current research funds to the entire trial project as a program of investment. Based on figures given to us by Minister Butler in November 2011, the NHMRC annual expenditure into **cancer related research was $175m in 2011 which comprises 23% of all NHMRC expenditure, but only a small fraction of this amount was directed to clinical trials**. The ALLG remains concerned that there is little consideration given to investing in clinical trials, even less to trials in haematological oncology.

In a survey of 3484 clinical trials and research projects in Australia, 65% of funds came from Biotechnology, Pharmaceutical and Medical Device companies, 10% from NHMRC grants, 7% from State Governments, 4% from other Federal Government sources and 14% from other sources (e.g. charitable organisations, societies, foundations, collaborative groups). The national need to integrate and share in research opportunity is clearly evident; however this must be led by government who has the capacity to manage the landscape of research partners.

An improvement to institutional arrangements needs to have visibility and assurance of sustainability to researchers in a long term capacity. Implementation of policy is one control e.g. process, research KPIs etc, however there needs to be absolute dollar investment for institutions to undertake research effectively, and that involves funding to laboratories, biobanks (tissue banks), research units, and personnel delivering care. Research is a precise science, as such long term commitments are need to realise the benefit of research investment.

Q3. What are the health and medical research strategic directions and priorities and how might we meet them? (Terms of Reference 5, 12 and 13)

5. Likely future developments in health and medical research, both in Australia and internationally.

12. The degree of alignment between Australia's health and medical research activities and the determinants of good health, the nation's burden of disease profile and national health priorities, in particular "closing the gap" between indigenous and non indigenous Australians.

13. Opportunities for Australia's health and medical research activities to assist in combating some of the major barriers to improved health globally, especially in the developing world.

Between 1982 and 2007 the age-standardised incidence rate for all cancers combined has increased from 383 to 485 cases per 100,000 people (AIWH 2010). By the age of 85 years, 1 in 2 males and 1 in 3 females will have been diagnosed with cancer at some stage in their life and cancer is estimated to be the leading cause of the burden of disease in Australia in 2010, accounting for 19% of the total burden (AIWH 2010). A greater emphasis on clinical health outcomes from NHMRC funding will be a critical requirement to combat the growing burden of cancer in the community. Consideration to appropriately prioritising activities that will nurture Australia's research investment is critical.

Strategic direction:
Naturally the ALLG has an interest to fulfil a primary objective to find cures for cancer; equally it is in the nation's interest to accomplish this. The ALLG acknowledges that improvements in cancer can only be achieved via the delivery of scrupulously performed clinical trial research. The outcomes of the research provide the current direction, and determine the bearing of the next-steps.

Priorities to achieve this:

- Deliver scientifically valid objectives, sound reasoning, and feasibility and be underpinned with the appropriate samples collections and biobanking that build the foundations of stored collections for future research opportunities
- Determine appropriate funding models that enable clinical trial infrastructure and procedures to be performed that will enable their conduct
- Implement and monitor those funding models, to ensure longegivity of process i.e. **clinical trials are long term investments as such government support models must commit to long term implementation so as to realise the benefit.**
- Invest in education, training and remuneration of researchers and their qualified counterparts. This could be sourced from a number of academic and tertiary centres nationally.
- Essential to increasing clinical trial conduct in Australia is the necessary clinical infrastructure capacity and capability issues and a pool of adequately trained and supported clinical trials staff able to affect these goals.
- Formation of networked groups to address services in a collaborative manner – public, private, industry, and cooperative research groups. Economies of scale are to be had via collaboration with our immediate trialist colleagues

The goal is to translate research findings into clinical practice; the way to do this is through clinical trials. Australian participation in trials is 4% compared to the UK which is 11% (Bio21 2010). The NHMRC should aim to promote schemes which increase trial participation rate to a best-in-class target of 12%. Increasing clinical research participation rates will create a 'virtuous cycle' of research translation into health outcomes.

Health and medical research can be functionally divided into research whose worth is judged by the new knowledge it creates and the ideas for better health that flow subsequently (fundamental research and early translation), and research that is explicitly addressing questions of immediate relevance to the health of individuals or the health of populations or the performance of the health care system. NHMRC does seem to be able to deliver at least partially for the former. For the latter, however, major new systems are required, and this can be most simply achieved by the creation of a new funding organisation that draws funding from the health care sector….as the engine room for R&D. It must also be recognised that the current system which funds medical research (by NHMRC/ARC) and health care delivery separately (through activity-based funding) has stifled clinical research and led to the withdrawal of clinicians in major teaching hospitals from research, and threatened the viability of national clinical trials consortia.

Proposal: *whilst the government considers the range of directions for health and medical research of the future, the common fundamental strategy must be evidence based and deliverable. Clinical trials are addressing a range of endpoints, of which the impacts of every day practice will be impacted upon. Clinical trial conduct as a means to translate research findings in to everyday clinical practice will benefit the hospitals and the community, and a greater review of the successes and barriers of clinical trial conduct in Australia is warranted.*

Q3 Summary:

What are the health and medical research strategic directions and priorities and how might we meet them?

Decisions for future strategic directions must be reviewed and implemented systematically, with the appropriate mix of private, public and industry representation in the process. Current research limitations need to be addressed so as to avoid future barriers in the conduct of research activities. A need for a new funding body that caters for large scale clinical trials is of major importance, and would free up the NHMRC to focus on training and funding support to researchers involved in the earlier phase and pilot studies as project grants.

Q4. How can we optimise translation of health and medical research into better health and wellbeing? (Terms of Reference 4, 8, 9, 10 and 11)

4. The relationship between business and the research sector, including opportunities to improve Australia's capacity to capitalise on its investment in health and medical research through commercialisation and strategies for realising returns on Commonwealth investments in health and medical research where gains result from commercialisation.

8. Opportunities to improve national and international collaboration between education, research, clinical and other public health related sectors to support the rapid translation of research outcomes into improved health policies and practices. This will include relevant international comparisons.

9. Ways in which the broader health reform process can be leveraged to improve research and translation opportunities in preventative health and in the primary, aged and acute care sectors, including through expanded clinical networks, as well as ways in which research can contribute to the design and optimal implementation of these health reforms.

10. Ways in which health and medical research interacts, and should interact, with other Government health policies and programs; including health technology assessments and the pharmaceutical and medical services assessment processes.

11. Ways in which the Commonwealth's e-health reforms can be leveraged to improve research and translation opportunities, including the availability, linkage and quality of data.

There is a fundamental importance of having clinical services linked to research and development activities from the perspective of education of both scientists and medical professionals. This link is essential in an area where our understanding is changing so rapidly due to application of new technologies. Training the future clinicians (medical, nursing etc) will optimise our position of being at the fore-front of developments in our respective areas be that research in cancer, immunology or haematology.

The ALLG has concerns that much of the infrastructure around clinical trials activity in particular is funded through the states. Many ALLG Members report having to argue at this level (to state and local hospitals) for any level of support. With the move towards the efficient price model for funding hospitals this activity runs the risk of being lost completely as it does not relate directly to measurable out-puts. Industry puts a percentage figure into R&D and our state funded hospitals are increasingly looking to either shed this or out-source it (to the university sector, institutes etc) moving it away from the clinical interface where it adds so much value.

For medical research to deliver value, it must be applied. Large scale randomised clinical trials of interventions, public health research, health economics research, and health care systems research all generate as their endpoint knowledge that is immediately applicable to how individual patients should receive care or how the health of the general population can be improved or how health care can be delivered more efficiently or cost-effectively.

The current organisation and funding of such research is fragmented and ineffective. The lack of translation of research output into improved systems and improved health outcomes can largely be explained by the current uncoupling of this type of research from the health care delivery system and particularly from the gatekeepers for change in the health sector. This uncoupling has been entrenched by systematic ring-fencing of funding and responsibility into silos. For example, the Federal Government functionally separates funding and responsibility for medical research from health care delivery. The same occurs in many State jurisdictions, and also within most major hospitals. Consequently, there is little buy-in by health care providers to the research and researchers are not required to design research that answers questions of high priority for the health of the Australian community.

An obvious structural change that would overcome this major barrier to research application and which would deliver greater transparency for the community about where the research dollar is being spent, is the creation of funding stream [perhaps named the Council for Health Investment and Economic Framework: Research (CHIEF: Research)] which is constituted and funded by the major health care delivery stakeholders. This agency would be responsible for funding large scale late-stage clinical trials, major public health studies, technology assessment research and health care delivery research. A significant proportion of its budget could be aligned with national health priorities, and should include commissioned research. It would naturally focus on very large-scale research and research that addresses very specific questions about health care delivery or illness prevention. It must also fund infrastructure for key participants e.g. national consortia for clinical trials, public health alliances etc. As its second major function, the Agency would be responsible for recommending and enabling implementation of evidence-based changes to the health care system.

Proposal: *Creation of a funding body should aim to initially provide detailed and justified advice to the Federal and State Governments as to what percentage of health budgets must be allocated to late stage R&D and implementation. The same body can then monitor the impact of this new investment, and recommend adjustment every 5 years or so. The UK has effectively done this, with some positive outcomes.*

Q4 Summary:

How can we optimise translation of health and medical research into better health and wellbeing?

Clinical services must be linked to research and development. Training needs to be in the forefront. There is a serious risk that research will lose out as hospitals implement efficient price models as it does not relate directly to measurable outputs. **Large scale randomised clinical trials generate important endpoint knowledge that is immediately applicable to individual patients and the population in general**. If this type of research continues to be uncoupled from the health delivery system, translation of this knowledge will be virtually impossible. Government needs to change from the current situation of functional separation of responsibilities for medical research and health care delivery. One solution would be to create a funding stream which s constituted and funded by major health care delivery stakeholders. As its second major function, such an Agency would be responsible for recommending and enabling implementation of evidence-based changes to the health care system.

VALIDATION OF SUBMISSION:

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