



Australian Institute for Bioengineering and Nanotechnology (AIBN)

Mr Simon McKeon
Chairman,
Strategic Review on Health and Medical Research in Australia

Dear Mr. McKeon and panel members,

Thank you for the opportunity to contribute to this review process. The Australian Institute for Bioengineering and Nanotechnology (AIBN) at the University of Queensland has a number of successful health and medical research programs which are exemplary because they demonstrate the importance of the discovery engine, the value of platform science, and the translation of research to innovative health devices and solutions tailored for Australian health priorities. **The key recommendations from the Australian Institute for Bioengineering and Nanotechnology to the McKeon review panel are:**

1. Capitalise on and further develop the current capacity in the Australian research community by fostering basic, clinical and applied/translational research under the Health and Medical research banner.
2. Identify the value of a highly skilled workforce, factor in success in the research sector by allowing for growth in the people-support schemes, identifying and fostering innovative research leaders, accompanied by a standardised National fellowship scheme to redress the current gaps in funding and scheme structure for early and mid-career salaries.
3. Recognise the value of platform sciences, and develop long-term investment strategies in the enabling facilities that service the Australian research community, government health departments and the biotechnology business community.

We have addressed the terms of reference of the review panel in the following document.

Yours Sincerely

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AIBN response to the McKeon Review of Health and Medical Research.

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

Australia is currently well positioned with a vibrant health and medical research community. This sector drives outcomes for local health priorities by:

- Protecting Australia's capacity to rapidly respond to emerging health threats.
- Supporting access to global healthcare advances for the Australian public through active participation in international partnerships
- Supports development of new healthcare products in the context of the Australian healthcare system and socioeconomic, cultural and environmental framework
- Provides global citizenship in responding to third world disease

Protecting Australia's capacity to rapidly respond to emerging health threats. A strong medical research sector in Australia is essential: it provides us with the skills and systems to be proactive with preventative medicine policies, respond to emerging medical threats and ensures the development of protocols, therapeutics and devices that address Australian health issues. Our ability to respond to infectious disease rely on local capacity to identify, diagnose, monitor, contain and treat these infections. This requires expertise in the basic sciences of immunology and cell biology, zoology, virology and microbiology. It requires critical mass in platform technologies including expertise in cutting edge approaches to genomics, bioinformatics, biomolecular engineering and protein expression, purification, characterisation and scale-up.

Capacity to respond to urgent health issues cannot be 'bought-in'. The barriers to technology adoption are high in the absence of a vibrant research community and a skilled workforce. We cannot outsource platform technologies because we require expertise in the underlying principals of these technologies for innovation and application to emerging diseases. A healthy medical research community requires an active foundation in discovery / exploratory sciences, supports the emergence and uptake of next-generation technologies and provides a critical mass of highly trained individuals who are able to innovate and direct world-wide trends, as well as adopt best practise from the international forums.

Supporting access to global healthcare advances for the Australian public through active participation in international partnerships The Australian context is an exciting one: our institutes have brought together leading researchers from around the world, we attract global collaborations and international students, which bring linkages and opportunities between Asia, Europe, North America and Australia. AIBN's experience offers a snapshot into the benefits of an international profile: Sixty-three of AIBN's researchers formally collaborate with research groups in more than twenty different countries, and receive funding from international biotechnology firms, overseas charitable organisations and US/European philanthropists. Over the last 5 years AIBN has increasingly attracted overseas students to our research laboratories: in 2007 thirty-seven percent of AIBN students were from overseas, whereas in 2011 sixty percent (72/120 students) were international students. This exchange between international research communities facilitates the exchange of ideas and outcomes, and places Australian laboratories in a highly sought-after position on the international stage.

Supports development of new healthcare products in the context of the Australian healthcare system and socioeconomic, cultural and environmental framework. A vibrant research community brings investment from industry. The AIBN experience is that local and international businesses are interested in partnerships that increase their capacity (such as access to platform technologies) and

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competitiveness (such as partnered IP development). Vaxxas Pty Ltd is an example of the translation of AIBN research, and represents one of Australia's largest investments in a startup biotechnology company, attracting \$15 million from local and international investors. The company will develop a revolutionary needle-free vaccine delivery system, and will translate pioneering Australian research and development of the Nanopatch to human clinical trials. The investment is led by OneVentures, with co-investors Brandon Capital, the Medical Research commercialisation Fund and US-based HealthCare Ventures (that has invested \$US1.6 billion in more than 100 companies over 25-plus years).

Provides global citizenship in responding to third world disease. Australian research has driven innovation in the health practises of developed nations, and provided new ways to tackle third-world diseases. Our ability to respond to infectious disease rely on local capacity to identify, diagnose, monitor, contain and treat these infections. This requires expertise in the basic sciences of immunology and cell biology, zoology, virology and microbiology. We need to be aware and responsive to global threats to be good global citizens, and to protect homeland security.

The anti Hendra virus monoclonal antibody m102.4 case study:

This case study is an example of the need for strong collaboration between clinicians and other technical groups in rapidly responding to emerging infectious disease threats.

Hendra ,and the closely related Nipah virus, are highly pathogenic zoonotic viruses whose predominant natural reservoirs are flying foxes. Hendra virus spillover events to horses have occurred over a period of eleven years, and on seven recognised occasions , transmission from horses to human. Of these seven human infections, four have been fatal.

Dr. Geoffrey Playford, Director, Infection Management Services, Princess Alexandra Hospital Brisbane, led the management of a number of the infected individuals, and managed to source an anti-hendra virus monoclonal antibody developed for research purposes by Dr.Chris Broder, from The Uniformed Services University of the Health Sciences in the USA. The antibody has been shown to protect ferrets and monkeys from Hendra virus challenge.

The Queensland government was keen to have supplies of m102.4 on hand for use as an experimental drug in case of increased spillover cases in humans. The Government contracted the National Biologics Facility (NBF) in AIBN to develop a process able to produce m102.4 of clinical purity, and to produce a number of batches of the antibody to stockpile for emergency use. The strong collaboration between Drs Playford and Broder and the group at the NBF led by Drs Munro and Chin meant that high quality antibody was produced in a relatively short time period. The NBF has been established with funding from the federal government (NCRIS scheme), and from the Queensland government.

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

AIBN strongly believes that translational research is a critical component of health and medical research in Australia and recommends that this imperative be recognised in government funding programs. AIBN supports the development of a long-term government strategy to invest in health and medical research. This strategy must capitalise on the current momentum in health and medical sciences and recognise the social, financial and intellectual contributions that a vibrant Health and Medical Research Sector offers to the Australian community. It must recognise that globalisation requires growth in Australia's research sector to remain competitive in the intellectual economy, to protect our capacity to innovate in an international context. It must recognise that critical mass of researchers across disciplines is essential in protecting and further developing a quality framework for Australian healthcare.

Recommendations:

- Support cross disciplinary research which sits at the translational interface between basic research, device or process development, and clinical practise.
- Develop the platforms and systems to implement personalised medicine
- Continue to invest in the basic research as the engine that drives innovation in clinical practise
- Remove the divisive policies of the National funding agencies that currently limit researchers who aim to cross the basic/applied research divide.

Support cross disciplinary research which sits at the translational interface between basic research, device or process development, and clinical practise. The AIBN believes that the current definitions of Australian medical research often ignores the contributions of cross-disciplinary research to translational outcomes. We need an environment which fosters basic research in cell and developmental biology, physics, engineering, mathematics and molecular studies, as these disciplines feed into the applied research of specific diseases. We support medical research that addresses the burden of disease in Australia, which projects changes to that disease burden over the coming decades, and which insures itself against emerging infectious diseases.

Develop the platforms and systems to implement personalised medicine. Two of the most significant trends in medical research over the past couple of decades are (i) personalised medicine (the right drug for the right person at the right time) and (ii) translational medicine (direct, two-way linkage between basic research, applied research and clinical practice). The two trends are interconnected, in that strong translational medicine is required for the development and delivery of personalised medicine. Without a continuing (and growing) investment in all facets of Australian medical research (basic, applied and clinical) the availability of the benefits of personalised medicine will be delayed for Australian citizens. Personalised medicine has to be delivered in the context of the Australian healthcare system and within the local ethical, socioeconomic, cultural and environmental framework. This can only happen if there is a strong, underpinning base of experienced basic, applied and clinical researchers with access to state-of-the-art equipment, facilities and technologies. Furthermore, a strong and active translational research base supports the testing and early adoption of new healthcare treatments, providing Australian citizens with timely access to innovative products and treatments.

Continue to invest in the basic research as the engine that drives innovation in clinical practise. The application of stem cells to regenerative medicine leverages on the fundamental research of reprogramming cells. The application of personalised medicine to cancer patients leverages on the

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chemistry, nanotechnology and computing research that drive the technology to faster, cheaper and more reliable platforms. Gene therapy opportunities are expanded by fundamental research into microRNA regulation of the genome. Basic research must be supported within an Australian system, and the division between basic and applied research is somewhat artificial in this context.

Remove the divisive policies of the National funding agencies that currently limit researchers who aim to cross the basic/applied research divide. The divide between ARC and NHMRC funding schemes is throttling the translational interface between engineering and medical disciplines. The current developments in the ARC system are widening this gap by introducing quotas restricting NHMRC grant holders in the number and type of ARC schemes that they can apply for. Researchers are forced to choose between ARC or NHMRC fellowship schemes - but given the low success rate of either scheme, this is forcing researchers to make an unequitable gamble between basic or medical research. Those researchers who sit at the intersection of disciplines, striving to drive basic research to clinical translation through invention of a platform, process or device are penalised. We argue that these are the very researchers who should be fostered and encouraged within the system.

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

(Terms of Reference 2, 3 and 7)

The AIBNs recommendations are:

- Provide a financial environment which facilitates co-investment by philanthropists and industry.
- Provide a funding framework that allows for 'incubation' of projects until they are ready for private investment.
- Develop an at-call fund to be used as leverage in response to investment opportunities outside the normal funding cycle.
- Build capacity in cross-disciplinary research through new funding models
- Build a critical mass of skilled scientists
- Remove the ARC/NHMRC divide in fellowship career structure and funding policy and redress the funding shortages in scholarships and fellowships

Provide a policy and funding environment that supports industry and philanthropic investment.

AIBN researchers actively seek funding from a variety of sources, and our research is currently funded through philanthropic, business, state and federal government. We recognise that investment by business and philanthropy requires a healthy research sector: government funding plays a key role in foundation research, recruiting and maintaining highly competitive staff, attracting students, and in providing the infrastructure for platform research.

The expectation of the research community is that State and Federal governments will provision for growth in the research sector, just as it plans for growth in other industries. On-going investment in traditional project and people support schemes is essential. Equally, long-term funding for enabling technologies through dedicated facilities requires funding for equipment as well as highly skilled staff to enable the use and adoption of this equipment to the broader research community, including industry and clinical partners.

Provide a funding framework that allows for incubation of projects until they are ready for private investment. One of the challenges faced by institutes such as AIBN is how to nurture innovative projects through to the point where external, private investment is available (and appropriate). Clearly, private investment is essential for the development and commercialisation of products arising from technologies coming out of public investment in health and medical research. However, the timing needs to be right. Premature private investment can be detrimental, as is investment that comes too late. If Australia is to gain maximum benefit for its investment in medical research, then it needs to nurture products that arise from that research. The current funding models do not provide such a 'nurturing' environment. Research institutes and universities are often forced to prematurely create 'spin out' companies in order to obtain small amounts of seed capital for product 'proof-of-concept'. The relatively small amounts of seed capital available, along with the burden imposed by a corporate structure, sets these companies up for failure right from the start. Funding programs that support projects through to proof-of-concept within a research institute context would increase the likelihood that public funded medical research would lead to commercial products. 'Incubation' of innovative projects within academic institutes not only de-risks the project so that private investment is more likely, it also increases the quantum of private investment that can be obtained. The Vaxxas Pty. Ltd. case study (page 7) is a perfect example of an exciting technology platform that was taken to animal proof-of-concept (within AIBN) and was then able to secure a major (\$15M) Series A investment.

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Building capacity in cross-disciplinary research through new funding models. The AIBN experience in linkages between philanthropy, business and government illustrates the necessity of ongoing investment by all three sectors. Although health and medical research is a major theme at the institute, NHMRC contributes modestly through fellowships, student scholarships and project grants. Our experience with the NHMRC system argues that new models of cross-disciplinary funding are necessary, which consider team metrics and infrastructure capacity, and have a focus on platforms that can be applied across many, not just one, disease. The scale of these projects often falls between a typical project grant and a bigger program grant.

We suggest that providing a third funding arm that sits between ARC and NHMRC aimed at emerging technology, blue sky science, and cross-disciplinary platform sciences would redress the current funding gap in NHMRC and the philosophical gap between ARC and NHMRC. Track record assessments are used to judge feasibility of the project given the research team. Therefore we argue that these should rely less on the publication pedigree of senior academics, but also considered a breadth of evidence of impact in the field - such as technology innovation, collaboration and leadership in enabling research - criteria which may be best exemplified in early career researchers.

Develop an at-call fund to be used as leverage in response to investment opportunities outside the normal funding cycle. Government support frequently provides necessary leverage for philanthropic or business buy-in. The funding cycle is too slow for institutes who wish to take advantage of international funding opportunities that may require government leverage. An at-call special initiatives fund would benefit strategic Australian researchers engage in research investments that are not part of a typical funding calendar.

Build a critical mass of skilled scientists. It is clear that collaborative, cross disciplinary research teams are necessary to exploit many of the technological developments in health care. However this also requires individuals who are highly skilled specialists. Therefore AIBN believes that an Australian research strategy must continue to support research higher-degree training, and continue to provide a career structure for early career through to senior health and medical researchers. Providing talented people with the means to innovate should be complimented by strategic funding initiatives for team research.

Remove the ARC/NHMRC divide in fellowship career structure and funding policy. Future fellowships from the Federal government, and Smart Futures from the QLD government provide exemplar investment in talented researchers, and provision of seed project funding for those researchers. The Future-fellowship scheme has been very successful at attracting skilled researchers back to Australia from leading international laboratories. The scheme is due to terminate after the next round (2012). The AIBN urges the continuation of this scheme, and the adoption of the principal of investment in people with seed funding for NHMRC fellowships, particularly those aimed at supporting early-mid career researchers.

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

(Terms of Reference 4, 8, 9, 10 and 11)

The AIBN recommendations are:

- Prioritise the workforce: invest in skilled scientists
- Retain and grow capacity: clinical standard manufacturing and screening platforms
- Retain capacity to respond to emerging health threats

Prioritise the workforce: invest in skilled scientists. Fund high calibre innovators to undertake highly experimental research: blue-sky research such as the human-genome project, solving the antibiotic crisis, transformative technologies for theranostics require strategic investment in people rather than projects. Fund technical experts into research-hubs in the form of core facilities; these provide the engine room technology and allow the application of cutting-edge technologies to a cross-section of medical research problems. This technical expertise falls outside of current fellowships, it is a technical career structure that is currently missing from any of our funding models. Of particular concern to AIBN is the funding gap imposed on research groups and researchers successful in winning Fellowships. Fellowship salaries should be fully funded.

Retain and grow capacity: develop local capacity for clinical standard manufacturing and screening platforms. The potential and real impact of platform technologies on health outcomes, across a range of diseases, cannot be overstated. Increasingly these platforms come from research that is funded by the ARC. The recently-introduced ARC ruling restricting applications for those who successfully work across both the ARC and NHMRC domains will discourage multidisciplinary approaches to health outcomes.

The Vaxxas Pty Ltd case-study:

The Vaxxas case study is a good example of high quality medical research being translated to a commercial entity for the evaluation of clinical potential. Vaxxas aims to replace the needle and syringe traditionally used for vaccination with a patch smaller than a postage stamp, the *Nanopatch*. The *Nanopatch* has thousands of small projections designed to deliver the vaccine to the abundant immune cells in the skin

The basic research behind the *Nanopatch* started with Professor Mark Kendall returning from Oxford to AIBN/UQ with exciting novel ideas for improved vaccine delivery. In collaboration with a group of excellent medical researchers, he established a major multidisciplinary research group developing the *Nanopatch*, with federal government support in the form of NHMRC and ARC grants, and international funding support from the Bill and Melinda Gates Foundation, PATH, and the Medical Research Council. The research has been highly productive and has resulted in a strong suite of patents. A key element in the development of the *Nanopatch* was that the group had access to a specialised piece of equipment, a Deep Reactive Ion Etcher, installed in AIBN as part of the Australian National Fabrication Facility (ANFF), funded under the NCRIS scheme as national research infrastructure.

The success of the animal trials to date with *Nanopatch* attracted the attention of a group of Australian and US venture capitalists, who formed a consortium to invest \$15million in Vaxxas Pty Ltd. The investment in Vaxxas, one of Australia's largest investments in a start-up biotechnology company, will fund the company to carry out human clinical trials of the new technology