Australian Medical Research and Innovation Five Year Strategy and Priority Area: Medical Innovation

The BioMelbourne Network fully supports the establishment and the continuing capitalisation of the Medical Research Future Fund as a dedicated vehicle for investment in health and medical research and medical innovation in Australia. This submission proposes strategies for the critical priority area of medical innovation, which we define as the translation of research discoveries into development pathways that deliver new products and services which provide measureable clinical, economic & health outcomes. We recognise that the MRFF will fund additional priorities that lie outside the scope of this submission which may be complementary to our vision for a thriving health innovation ecosystem in Australia.

The challenge: To ensure that Australia can translate a research discovery through the product development lifecycle here in Australia to deliver social, health and economic benefits to the nation. Alignment with Current Challenges - Facilitate the translation of research into health outcomes; Support research and innovation from concept to delivery

Australia has relatively low levels of translation of research discoveries into patient-ready products and services, the well acknowledged "valley of death" or "chasm of commercialisation" as described in a number of previous reviews of Australia's health and medical research system. When considering health and medical R&D, Australia has invested in and delivered outstanding "R", but has failed to adequately resource and progress the "D". Across the world, support for regulatory-standard product development has leveraged the skills and capabilities of industry partners to drive successful research translation. Australia's vibrant biotechnology, medical technology & pharmaceutical industry has a depth of skills, expertise and "know how" that can advance the translation of research into health outcomes. **The MRFF strategy should provide the bridge in the pathway from research discovery to patient by dedicating a fixed level of funding in direct financial support of research translation and product development in these priority areas**:

- Formal pre-clinical development
- Early stage manufacture, prototyping and formulation
- Clinical trials
- Industry workforce capability
- Health innovation system reform

Strategy building blocks: Aims - A translation pathway that maximizes opportunities for success; Leveraging and enhancing collaboration and integration; economic benefits. Mandatory Considerations - How to deliver practical benefits from medical research and medical innovation to as many Australians as possible; How to ensure that disbursements complement and enhance other assistance provided to the sector

It is essential that the MRFF be used to complement and enhance other support provided to the sector, and to undertake activity that is additional to that currently being funded. Once medical research outputs move into development phase, there is limited funding available through publicly funded research councils, which typically lack the continuity, scale or consistency to efficiently progress translation. Research outputs prior to preclinical development are often viewed as "too early" to attract investment from venture capital or angel investors. The only current option arises from the Entrepreneurs' Programme – Accelerating Commercialisation, which has limited funds and is not specific to the health and medical research sector.

The MRFF disbursements should be strategically positioned downstream of research councils (NHMRC/ARC) to support the further development of research outputs and create a translational research pipeline of health innovation products. Translational outputs from the MRFF can then feed into funding programs such as the BTF or other investment mechanisms/existing commercialisation programs. This will bridge the translational gap and enable research outputs to reach patients, creating social, health and economic benefits for Australia.

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Solutions: The translation of research outcomes to improve the health of all Australians requires a supported and well-funded medical innovation ecosystem which fosters collaboration and partnerships. The MRFF should 1) support multidisciplinary R&D teams, bringing together industry, SMEs, startups, clinicians and academics with diverse backgrounds to address unmet health needs; 2) support the spin-out of medical research & technology into Australian start-up companies; 3) accelerate research translation through engagement with early stage and later stage Australian biotech & medtech companies who provide the vehicle for medical innovation. A focus on translational research should provide support to both research organisations and companies seeking to develop products that deliver impact for patients and the economy.

Priority 1) Formal preclinical development. There are currently very limited incentives to support formal pre-clinical development, such as pivotal toxicology & non-clinical safety pharmacology studies, which may include both *in vitro* and *in vivo* studies. The three main barriers faced are: 1) Funding to support the early protection of valuable intellectual property; 2) Access to expertise to advise on the preclinical data required to support clinical trial applications, to develop a feasible and efficient business plan to conduct clinical studies; 3) Accredited facilities in Australia that operate to regulatory-standard Good Laboratory Practice (GLP) standards to undertake the work here, rather than offshore. The MRFF is ideally placed to fill this gap and provide increased access to expertise, develop GLP facilities and increase knowledge transfer by creating funding mechanisms to support the preclinical development activities which generate the data required to undertake clinical trials. Filling this gap would provide mechanisms for collaboration to develop knowledge and skills for product-focused preclinical studies. One mechanism to address this would be to establish a "preclinical accelerator" or "medical innovation hub"; through the engagement of a dedicated cohort of advisors with knowledge and networks across the gamut of pharmaceutical and medical technologies, to facilitate connections between technology developers and the appropriate service providers or experts in the field. This could be further enhanced by embedding companies or experts with product development expertise within preclinical facilities in research institutes, thus facilitating outcomes-focused early stage translational research through robust study design and execution.

Priority 2) Early stage manufacture, prototyping and formulation. Early stage manufacturing is a crucial component of translating research products into outcomes. It requires significant skill, quality systems and process development input and brings a significant focus to the cost effectiveness of the processes to ensure a commercially viable product can be produced. Formulation of drugs requires experience, with a focus on the route of administration and similarly, medical device prototyping needs to consider end user experience and adherence to standards to ensure clinical success. The final formulated materials/prototype medical devices should be manufactured for use in preclinical studies as well as clinical studies. Incorrectly formulated drugs or devices that need further design modifications can negate subsequent development activities, a costly consequence as reformulating/redesigning products wastes significant resources and can bring about the premature end of the development of a promising new drug or device. One mechanism to address this challenge would be the introduction of Development vouchers that allow academics, SMEs and startup companies to access development services from qualified facilities at universities or institutes, or registered service providers to accelerate the development of products that will have health benefits for Australia. A voucher model could also encompass preclinical and clinical work in the translational research space. This scheme could be modelled from successful State Government of Victoria Innovation voucher program, and would drive collaboration and partnerships between academia, SMEs and industry.

Priority 3) Clinical trials. Clinical trials are the value-adding component of development and translational research providing pivotal safety and efficacy data for new medical innovations, and directly link research

outcomes with patients. Clinical trials are a key competitive advantage for Australia, in terms of developing the value of Australian IP onshore, as well as providing patients with early access to emerging therapies and treatments. Australia has world class clinical trials infrastructure and the MRFF should support and enable a greater number of investigator-lead and sponsored clinical trials to be undertaken in Australia, particularly in phase 2a/2b trials, where patients are enrolled to assess efficacy and safety.

Priority 4) Industry workforce capability. Multiple, specialised disciplines are required to work together in development of new medical products. The biotechnology and pharmaceutical industry is the repository of development expertise and access to this expertise is crucial to support our national development and research translation goals. Two areas that the MRFF strategy should address: 1) providing the academic research workforce with exposure to industry and ideally providing industry experience during their training. International fellowships that enable medical researchers to undertake secondments and training with multinational corporations in the global medical technology and pharmaceutical sector would foster product development and translational research skills and allow fellows to return to Australia with these crucial skills from international industry experience. Also, providing support for students and companies to engage with the diverse number of local industry internship programs that are already established, would create scale and accelerate skills development 2) Establishing a post-graduate employment program to assist with workforce growth and ensure access to talent for high growth-potential companies. Many large corporations in areas such as legal and finance offer graduate and post-graduate employment programs that provide 12-24 months of in-company training to develop the future workforce for that sector. This scale is not achievable for individual SMEs working in the biotechnology, medical technology and pharmaceutical sector. Creating a sector-wide graduate employment program would enable high-growth potential SMEs to employ highly educated, trained research graduates thus creating jobs and retaining & training the future innovation workforce in Australia. It would also build the capability and expertise needed to execute the MRFF strategy.

Priority 5) Health innovation system reform. There is an urgent need to adapt the Australian healthcare system to be able to integrate new technologies into standard of care practices. Emerging areas of technology, such as cellular therapies, regenerative medicine, personalised genomics and customised medical devices will not be seamlessly integrated into patient care pathways without addressing regulatory, and particularly funding (including lack of mechanisms to determine health-economic outcomes) and operational barriers (such as changing locations of care, new supply chains and logistics skills) to ensure benefits can be delivered to patients. Funding from MRFF could be used to: 1) Develop and test (including training) new deployment models for modalities such as cellular therapies and mobile health technologies; 2) Design new funding models for curative therapies and continuous patient monitoring technologies; 3) Fund health economic impact studies e.g. by procurement of new technologies for limited periods to enable health systems and product sponsors to jointly evaluate cost effectiveness. In addition to earlier, streamlined access to breakthrough technologies, these measures could create an ecosystem that enables Australian companies to develop the evidence required to enable stronger partnering deals or global market penetration, thereby retaining research at home and delivering greater economic returns alongside health outcomes benefits to Australians.

Measures of success for the MRFF: Increased number of products based on Australian health and medical research in the product development pipeline. Increased numbers of clinical trials. Increased manufacturing and R&D activity. Enhanced collaboration and partnerships across the innovation ecosystem. Measureable economic returns from investment attraction, job creation and product export. Increased patient access to therapies based on Australian health and medical research & improved health outcomes for Australians.