APPROVAL PROCESSES FOR NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES IN AUSTRALIA

Submission to the House of Representatives Standing Committee on Health, Aged Care and Sport

October 2020

RESEARCH AUSTRALIA

CONNECTING ▪ ENGAGING ▪ INFLUENCING

ABOUT RESEARCH AUSTRALIA

We are the national peak body representing the whole of the health and medical research pipeline.

Our vision: Research Australia envisions a world where Australia unlocks the full potential of its world-leading health and medical research sector to deliver the best possible healthcare and global leadership in health innovation.

Our mission: To use our unique convening power to position health and medical research as a significant driver of a healthy population and contributor to a healthy economy.

Our goals:

Engage

Australia in a conversation about the health benefits and economic value of its investment in health and medical research.

Connect

researchers, funders and consumers to increase investment in health and medical research from all sources.

Influence

government policies that support effective health and medical research and its routine translation into evidence-based practices and better health outcomes.

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Summary of recommendations

Earlier and better diagnosis of rare diseases to improve access to clinical trials involves expanding the capacity of the existing National Health Genomics Policy Framework for the systematic, equitable and timely delivery of genomic services, such as genetic testing (diagnostics) and gene therapies (treatments) and genetic counselling to Australians with, suspected of having, or with an increased chance of a rare disease. **It also requires investment in rare disease registries to facilitate the identification and recruitment of eligible clinical trial candidates.**
If we are to achieve the objective of commercialising more drugs and novel medical technologies where there is an unmet need, the guidelines for funding programs need to be reviewed to ensure that the guidelines better support the research and commercialisation process.
Commercialising more drugs and novel medical technologies where there is an unmet need, requires an 'end to end' review of funding programs, identifying what funding and incentives currently exist and where the gaps are. **Consideration then needs to be given to what action can be taken to closing these gaps, including modifying existing programs to better support commercialisation, and providing a more streamlined progression through the pipeline for research that has commercialisation potential.**
Where commercialisation is a goal of funding, projects that secure funding for a clinical trial need to be supported to develop a commercialisation strategy from the outset. This will influence the design of the clinical trial, what data is captured and how, creation of an IP and regulatory strategy, and the investigation of commercial partners. Expenditure for this purpose needs to recognised as a legitimate research cost that can be funded from the grant.
The feasibility of expanding the remit of the NHDISC to include clinical trials data and inclusion of the TGA in its membership should be considered as a way of making Australia a more attractive location for clinical trials.
Research Australia submits that the Committee should consider the opportunities precipitated by COVID-19 to consolidate the opportunities for remote recruitment, consent and administration of clinical trials as standard practice in Australia.
There are further opportunities to align the TGA's processes with those of overseas regulators. In particular, Research Australia notes that the there is an opportunity to align reporting to the TGA of clinical trials data and adverse events with data models and standards being adopted internationally. **The capacity for the sponsor to report to the TGA using the same data and reporting standards used for the FDA could be an important incentive to recruit patients for clinical trials in Australia and to subsequently seek registration of a new medicine here.**

APPROVAL PROCESSES FOR NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES IN AUSTRALIA

SUBMISSION TO THE HOUSE OF REPRESENTATIVES STANDING COMMITTEE ON HEALTH, AGED CARE AND SPORT

Introduction

Research Australia welcomes the opportunity to make a submission to the Committee in response to the Inquiry into Approval Processes for New Drugs and Novel Medical Technologies in Australia.

Modern life is underpinned by science, and nowhere is this more evident or important than in medicine. Enormous advances have been made in the last century in the development of new medicines and medical technologies, which have saved innumerable lives and improved the quality of life for tens of millions. This progress is continuing, with new knowledge being acquired and applied every day in ways that bring new drugs and medical technologies to the patient.

Keeping up with this increasing pipeline of new products is an ongoing challenge for our regulators, our health professionals and our health system. It is therefore timely that the Standing Committee on Health, Aged Care and Sport is conducting this Inquiry into these approval processes.

Opportunities exist to change our approval processes to benefit patients and better support research and development in Australia. Faster and more effective approval processes mean new medicines and technologies reach patients faster. Improving the environment for clinical trials enables Australian patients to benefit from the latest medicines and technologies developed overseas while also helping Australian health and medical research to flourish in a competitive and lucrative world market. Research Australia's submission seeks to identify these opportunities with the twin objectives of improving Australians' health and prosperity.

Terms of Reference

Research Australia's submission addresses each of the Terms of Reference in turn.

The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies.

There are many new drugs and technologies that cross over traditional boundaries. Some new treatments incorporate drugs and delivery technologies, or combine diagnosis, monitoring and treatment. The recent experience around autologous treatments, which has allowed therapies that used an individual's own stem cells to proceed without effective oversight or regulation, are an example of the problems that can be created when we draw the boundaries too rigidly, creating loopholes that can be exposed and/or place patients at risk.

There is also a blurring of boundaries between consumer devices and medical devices and this is likely to continue, particularly with the growth of telehealth and the rise of products designed to be used in the home setting to monitor health and/or symptoms. Regulatory overreach could be detrimental to consumers and patients by stifling innovation and making products more expensive, while a lack of oversight can place lives at risk. Any response needs to be proportionate to the risk of the therapy/ device.

This is no easy task, and there are no simple solutions. It requires a regulator that is well placed to collect information about emerging trends in Australia and overseas and is able to consult quickly and effectively with product manufacturers, innovators, health professionals and consumers.

Another requirement in respect of innovation relating to rare diseases is a diagnosis. An early and definitive diagnosis is necessary to enable an individual to be identified as a potential candidate for a clinical trial. While still not a straightforward process, recent technological developments, particularly in relation to genomic sequencing, have demonstrated the effectiveness of this approach. [1]

Research Australia submits that earlier and better diagnosis of rare diseases to improve access to clinical trials involves expanding the capacity of the existing National Health Genomics Policy Framework for the systematic, equitable and timely delivery of genomic services, such as genetic testing (diagnostics) and gene therapies (treatments) and genetic counselling to Australians with, suspected of having, or with an increased chance of a rare disease. It also requires investment in rare disease registries to facilitate the identification and recruitment of eligible clinical trial candidates.

[1] https://www.news-medical.net/news/20200624/Whole-genome-sequencing-can-improve-diagnosis-and-treatment-of-patients-with-rare-diseases.aspx

Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions.

The journey from initial research discovery to a new drug, therapy or technology is a long one. In the classic model, the pharmaceutical company is responsible for development of a drug, and its testing and commercialisation. The significant investment costs are recouped from the profits from the product's subsequent sale.

In other cases, such as rare conditions with an unmet need, and personalised and off patent drugs this model does not always work; the prospective profit may not be sufficient to warrant undertaking the initial research and/or commercial development process. Here there is a case for a degree of public subsidy or funding, particularly in the early stages of development. If and when a drug can be shown to be effective, some of this cost may able to be recouped through licensing or other means, but essentially it requires public intervention if the benefit to patients is to be realised. The actual manufacture and distribution of the new drug or medical technology typically still involves a commercial manufacturer.

In Australia, we have some programs and opportunities where government and/or philanthropic funding is available, but these are fragmented. Funding and incentives are available for some stages along this journey, but the connections between these different stages are tenuous, or don't exist.

We need to take this journey and turn it into a process, where the ultimate objective of developing a new product is considered appropriately at the outset, and where progress towards this objective is better planned for and evaluated at each stage, with progress to the next stage assured if the appropriate requirements (scientific and commercial) have been met. Turning the current journey into a process requires a new mindset, changes to what is funded, and when.

Two current grant opportunities from the Medical Research Future Fund (MRFF) provide an illustration of how we fund and incentivise different parts of a journey rather than a process.

The MRFF's 2020 Childhood Cancer Research Grant Opportunity seeks to *'support Australian medical research and medical innovation projects that improve our understanding of the aetiology, biology and progression of cancer in children and adolescents.'*[2]

The guidelines make it clear that Partnerships and co-investment are encouraged, including with commercial entities such as pharmaceutical companies. This grant opportunity does not fund clinical trials, instead focussing on research at an earlier stage, but which could potentially lead to a clinical trial.

The MRFF also funds later stage research. A current example is the funding for clinical trials under the Rare Cancer, Rare Disease and Unmet Needs (RCRDUN) initiative. The program guidelines

[2] Australian Government Department of Health 2020, Medical Research Future Fund – Emerging Priorities and Consumer Driven Research Initiative 2020 Childhood Cancer Research Grant Opportunity Guidelines

also state that it encourages partnerships with commercial entities such as pharmaceutical companies. This would seem to be an ideal program to provide new treatments and improve the lives of people with these conditions, except that the guidelines specifically disadvantage treatments that are likely to be able to be commercialised.

The following are two of the requirements against which applications are assessed.

'*You should demonstrate this by providing details of how your research proposal will be directly relevant to the objectives and desired outcomes of this grant opportunity, specifically:*

…..

-clinical trials that test the effectiveness of interventions for which a commercial return is unlikely to be achieved due to low patient numbers or where it is not possible to obtain an enforceable intellectual property right

– engagement of partners (e.g. commercial, health service delivery, multi-disciplinary, policy) that will support implementation of study findings into practice, as quickly as possible.'[3]

The requirement that a commercial return be unlikely raises the question of how the funding will benefit any patients other than those who participate in the clinical trial, unless the treatment being tested is suitable for non-commercial translation, such as adoption of a new model of care. (The guidelines specifically refer to 'health treatments, drugs and devices' as being eligible for funding.)

While it is reasonable to exclude clinical trials that can readily secure commercial backing, there is a sound rationale for public funding of clinical trials which are not attractive to commercial players at this point but where, should the trial prove successful, it can lead to a product that could be commercialised. There are many instances of treatments for conditions that could not secure commercial funding for initial clinical trials but were picked up at a later stage when the commercial potential was demonstrated. In the case of this RCRDUN grant opportunity, the potential for commercialisation if the clinical trial is successful needs to be evaluated as part of the application process for funding (where a commercial product is the path to the patient). This potential needs to be seen as a positive rather than a negative; an important means by which the clinical trial will deliver better health outcomes.

The support for commercial partners on the earlier stage Childhood Cancer Research Grant Opportunity, and the preference shown for testing interventions for which a commercial return is unlikely in the RCRDUN opportunity is an example of how we currently fund a journey, with many breaks and uncertainty, rather than a process.

While the two funding programs referred to above are both from the MRFF, there are other funding programs that are relevant to the research, commercialisation and development of new drugs and novel medical technologies, including programs funded by the Department of Education (ARC funded programs and Research Support Program); programs funded by the Department of Health through the National Health and Medical Research Council; and programs provided by the Department of Industry, Science, Energy and Resources.

[3] Australian Government Department of Health 2020, Medical Research Future Fund - Clinical Trials Activity Initiative 2020 Rare Cancers, Rare Diseases and Unmet Need General Grant Opportunity Guidelines

Research Australia submits that if we are to achieve the objective of commercialising more drugs and novel medical technologies where there is an unmet need, the guidelines for this and any similar programs need to be reviewed to ensure that the guidelines better support the research and commercialisation process.

Just as importantly we need to ensure that appropriate support and incentives are provided at all stages of the research and development process, and that success in an earlier stage helps 'qualify' a project for further, later stage funding.

Research Australia submits that this requires an 'end to end' review, identifying what funding and incentives currently exist and where the gaps are. Consideration then needs to be given to what action can be taken to closing these gaps, including modifying existing programs to better support commercialisation, and providing a more streamlined progression through the pipeline for research that has commercialisation potential.

We also need to ensure that the outcomes specified in funding programs to support the commercialisation of new drugs and medical devices are appropriate. For example, a program that is funding a Phase 1 clinical trial should fund the activity and research required to enable a successful trial to be ready to proceed to a stage 2 commercial clinical trial. This requires consideration of international regulators, such as the USA's Food and Drug Administration, which has specific requirements that must be met by a Phase 1 clinical trial before it can progress to Phase 2. Failure to do so can mean that a Phase 1 clinical trial needs to be repeated before progressing, significantly reducing the commercial viability of the research.

Research Australia submits where commercialisation is a goal, projects that secure funding for a clinical trial need to be supported to develop a commercialisation strategy from the outset. This will influence the design of the clinical trial, what data is captured and how, creation of an IP and regulatory strategy, and the investigation of commercial partners. Expenditure for this purpose needs to recognised as a legitimate research cost that can be funded from the grant.

If a product is subsequently successful in securing commercial funding for further development, it is preferable that this occur in Australia. To this end, Research Australia welcomes the Government's decision announced in the budget that will reverse many of the changes it had proposed to the Research and Development Tax Incentive (RDTI) Scheme. The RDTI Scheme provides an incentive for further commercial investment, and the removal of the caps in particular could support the significant investment required for Phase 2 and Phase 3 clinical trials to be undertaken on a commercial basis in Australia. This, together with other measures to support early stage research and align Australia's processes with international standards can help invigorate clinical trial activity and the development of medicines and devices in Australia.

Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies.

The reform of the Australian clinical trials environment has been ongoing for over a decade. While progress has been made in many areas there is still more work to be done. When it comes to ethics approval, the current National Mutual Acceptance scheme has been an improvement, but more work needs to be done to achieve a truly national and all-inclusive scheme. More can also be done to create a 'single system' post the ethics approval, with the adoption of common technology platforms, processes and reporting requirements by all parties, including state regulators.

At a Commonwealth Government level, Research Australia notes with approval the recent funding provided to the Therapeutic Goods Administration (TGA) to modernise its IT systems. This provides an opportunity to improve the provision of clinical trials data to the TGA and also the reporting of adverse events.

There are currently multiple systems, specifications and standards used across Australia and internationally for the collection and reporting of health information, including for clinical trials reporting. While a single system might be unattainable, better harmonisation of systems and improvements in interoperability within Australia could provide significant efficiency benefits for Australian clinical trials as well as the health system more broadly. This could help make Australia a more attractive location for international clinical trials.

The National Health Data and Information Standards Committee (NHDISC) provides advice to the Australian Institute of Health and Welfare (AIHW) for its work in developing and maintaining national health data and information standards and related national health information infrastructure, in the context of the National Health Information Agreement (NHIA).[4] It has representation from multiple Commonwealth Government Departments and agencies and from each state and territory.

The NHDISC does not currently include representation from the Therapeutic Goods Administration or consider data collected and reported for clinical trials. There is an opportunity to better incorporate the collection and reporting of data for clinical trials with other health information routinely collected in our health system. Better integration of reporting for clinical trials with systems used for other purposes in the health system has the potential to improve interoperability and efficiencies. Redevelopment of the TGA's IT systems provides an opportunity to include it in this process.

Research Australia submits the feasibility of expanding the remit of the NHDISC to include clinical trials data and inclusion of the TGA in its membership should be considered as a way of making Australia a more attractive location for clinical trials.

The COVID-19 pandemic has had a significant effect on Australia's health system but has not overwhelmed it in the way it has elsewhere in Australia. The early lockdown in Australia disrupted research but also led to new innovations, including more 'remote' recruitment of participants for

[4] https://www.aihw.gov.au/our-services/committees/national-health-data-and-information-standards-com

clinical trials. This innovation is complemented by a current initiative in Australia to normalise the use of electronic consent for participation in clinical trials, currently being led by CT:IQ.[5]

The COVID-19 pandemic has also accelerated the introduction of telehealth, 'normalising' remote engagement for patients and clinicians. This also provides an opportunity for clinical trials to expand the use of remote engagement with patients, potentially enabling more people, particularly in rural and remote Australia, to participate in clinical trials. Improved efficiencies in the recruitment for, and administration of, clinical trials in Australia, and improved reach in to rural and regional Australia have the potential to make Australia a more attractive location for clinical trials.

Research Australia submits that the Committee should consider the opportunities precipitated by COVID-19 to consolidate the opportunities for remote recruitment, consent and administration of clinical trials as standard practice in Australia.

Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.

Research Australia acknowledges the steps taken by the Australian Government and the TGA in recent years to promote international cooperation in relation to the regulation of therapeutic goods.

Research Australia has already noted the benefits that could flow from better harmonisation of systems and improvements in interoperability within Australia.

Research Australia submits there are further opportunities to align the TGA's processes with those of overseas regulators. In particular Research Australia notes that the there is an opportunity to align reporting to the TGA of clinical trials data and adverse events with data models and standards being adopted internationally. The capacity for the sponsor to report to the TGA using the same data and reporting standards used for the FDA could be an important incentive to recruit patients for clinical trials in Australia and to subsequently seek registration of a new medicine here.

[5] https://ctiq.com.au/implementing-econsent/

Conclusion

Health and medical research, and the development of new drugs and medical technologies, are advancing rapidly. It is critical to Australia's future health and prosperity that our processes for the approval of new drugs and technologies, and for the approval and conduct of clinical trials to test new drugs and technologies, keep pace. There are opportunities to make better use of technology, to better integrate the TGA with Australia's health systems, and to support more harmonisation with international regulators and regulatory processes, including through the use of common data standards.

Research Australia trusts that this submission has been useful, and we would be pleased to provide the Committee with further information.

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