

Submission

2016 National Research Infrastructure Roadmap

Capability Issues Paper

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General Questions

Question 1: Are there other capability areas that should be considered?

- These appear to be well-rounded and multi-disciplinary

Question 2: Are these governance characteristics appropriate and are there other factors that should be considered for optimal governance for national research infrastructure.

- Again, these appear to cover overarching governance characteristics.
- Perhaps consider a cross-jurisdictional element when appropriate for particular infrastructure.
- For example, PTNA is currently partially funded by NCRIS and endeavours to include representation from each Australian jurisdiction.

Question 3: Should national research infrastructure investment assist with access to international facilities?

- While international linkages are essential to paediatric clinical trials, priority for investment should be locally focused. Australia lacks the sustainable infrastructure that paediatric clinical trials requires in order to meet the needs of Australian patients and communities. However – see next question.

Question 4: What are the conditions or scenarios where access to international facilities should be prioritised over developing national facilities?

- Global initiatives to improve paediatric clinical trial infrastructure (in Australia and beyond) and to ensure the longevity of this research discipline would benefit from international prioritisation.
- Examples include international clinical trial network partnerships and collaborations where the global impact is greater than any individual country could contribute. Such a partnership is the newly formed Global Pediatric Clinical Trials Network < <https://critical-path.org/critical-path-institute-establishes-the-global-pediatric-clinical-trials-network-pre-launch-consortium/> > . A European example is the European Clinical Research Infrastructure Network <http://www.ecrin.org/> , already a successful academic clinical trials accelerator, it will begin developing a pan-European paediatric research network.
- Enhancing these linkages would potentially lead to smoother drug development pipelines, academic relationships, internationally collaborative R&D and a steady flow of clinical trials to offer Australians. The ultimate outcome will be improved health for all Australian

children and a decreased burden on the health system from preventable and treatable illnesses.

Question 5: Should research workforce skills be considered a research infrastructure issue?

- Infrastructure is only useful if there is expertise to use it. People need to be trained and educated in order to maximise the full potential of the infrastructure. For clinical trial this means that clinicians and their research teams need to be trained on correctly formulating research questions right through to interpreting the results. In a complex and highly regulated clinical research environment, research teams must understand the circumstances in which they are working. It is difficult to identify and access the training and education needed across Australia. While some cities have a number of choices for clinical research training, other cities and most rural areas have none.
- In addition, paediatric research presents unique challenges of its own. Children are more heterogeneous than adults, varying greatly in size and physiologic maturity. Outcome measures often need developing to fit the child's age and dosing also needs to be age and maturity appropriate. These all increase the complexity and costs of studies, which may need to be repeated in multiple age groups. Paediatric illnesses can be specific to children, many are very common while many are rare. It is well established that paediatric illness can have life-long implications – medically, socially and economically. Medicines for children are uncommonly tested in children even though modes of action, doses and regimen may differ significantly from adult medicines. With a shift toward increasing high-quality research in children, there is a growing need for specialised, skilled and trained paediatric researchers.

Question 6: How can national research infrastructure assist in training and skills development?

- Following on from Qn5, provision of accredited training and education that is accessible in every Australian state is an important research infrastructure consideration. Accrediting existing courses and enabling providers to develop new material, run regular, low-cost courses and to reach out to locations that have hitherto missed out could result in an early win.
- Clinical trials as a discipline has the capability of attracting huge amounts of investment into Australia. This will increase the number of jobs available in the field. Australia has to be ready for this imminent growth.
- Clinical trials in children pose unique challenges. This requires an investment in developing innovative methodologies and strategies for design and recruitment.
- For example, PTNA licences a variety of paediatric clinical research material from its members but lacks the infrastructure and national expertise to deliver them in all jurisdictions. Training and skills development needs to be accessible to be effective.

Question 7: What responsibility should research institutions have in supporting the development of infrastructure ready researchers and technical specialists?

- Ensuring that all staff members that are involved in clinical research are properly qualified, trained and/or supervised is the responsibility of the research institute. For organisations that undertake clinical research this is largely the responsibility of the clinical governance

office and the clinical research governance office in conjunction with monitoring by the human research ethics committee secretariat. Often the research governance and ethics offices are supported partially by the institutional and partially by fee-for-services models. Maintaining and monitoring the required standards for infrastructure-ready researchers and trial specialists can be difficult even for the best intentioned institution if the training and education are cost-prohibitive, geographically inaccessible or otherwise not available.

- PTNA will be able to play a role in monitoring and setting quality standards for the sites/institutions that use the infrastructure.

Question 8: What principles should be applied for access to national research infrastructure, and are there situations when these should not apply?

- While we agree with the tiered approach to user access according to public sector, public sector international and private sector, the missing category is public-private partnerships. In the clinical trial setting there are variety of collaborative combinations which can vary between fully government-funded research through to fully commercially-funded research. The missing category catches the collaborations that would fall in between, which on a sliding scale can be more public or more private.
- Access should be based on the intended outcomes with public good at the high access end and shareholder profit at the other
- Australian research should be given high priority
- International access should be given to research that will benefit Australians and/or contribute a research body of knowledge
- Priority access should be given to those with no alternatives (eg geographic isolation or esoteric requirements)
- Rates should be appropriate for the tiers of user categories
- Rates and access policies should be easily available, uncomplicated and transparent

Question 9: What should the criteria and funding arrangements for defunding or decommissioning look like?

- If infrastructure is not providing a return on the investment within a given time, which will differ for each piece of infrastructure, disinvestment should be considered. However, the reasons for inadequate usage should be determined prior to disinvesting or decommissioning (eg expertise to operate the infrastructure, location, lack of marketing, access policies, cost of use vs cost of maintenance and operations, alternatives available, technology changes since commissioning, implications for existing users, data migration issues, sale of equipment and so on).
- Importantly the stage of development, visibility and user uptake should be considered for newer infrastructure which have not had the advantage of time or means to build its user base.
- The cost of decommissioning needs to be taken into regard.

Question 10: What financing models should the Government consider to support investment in national research infrastructure?

- Much like capital works in construction, public-private partnerships (PPPs) remove some of the risk from government.
- Matched funding as per previous NCRIS grant rounds
- Tax incentives for industry partners
- Partnerships with philanthropic organisations
- Greater transparency regarding what is not funded – industry and charitable organisations may be able to ‘fill the gaps’
- Requirement for financial investment for large upfront amounts to ensure funds are being leveraged and optimised (eg investment interest).

Question 11: When should capabilities be expected to address standard and accreditation requirements?

- Government funded infrastructure should meet high standards and be accredited within a reasonable period of time. This needs to be factored into the amounts invested in the infrastructure as accreditation can be expensive.
- However, some infrastructure may meet the needs of the users without the need for accreditation, this also needs to be considered for each piece of research infrastructure. When is accreditation perfunctory? When does it add value?
- Accreditation should not prohibit the infrastructure from being used, however it should clearly state that it is not accredited.
- It is important that sites that participate in paediatric clinical research meet the standards required of such activity. PTNA could play a role in setting and monitoring these standards.

Question 12: Are there international or global models that represent best practice for national research infrastructure that could be considered?

- While other powerhouse countries rally to improve their competitiveness in paediatric clinical trials through collaboration, Australia risks being left behind if it does not have the infrastructure to meet the increasing need for paediatric clinical research and innovation.
- The closest model to the Australian environment is the **European Paediatric Clinical Trials Research Infrastructure -EPCTRI ECRIN (Europe)** – included in the **European Strategic Forum on Research Infrastructures (ESFRI) 2016 Road Map**, https://ec.europa.eu/research/infrastructures/index_en.cfm?pg=esfri-roadmap. The EPCTRI model includes a central operating hub plus a network of sites. Each site meets a minimum standard for participating in paediatric research. PTNA is well positioned to form the central operating hub. In addition, PTNA’s governance structure substantially meets the criteria of the National Research Infrastructure Roadmap Issues paper (section 3.2) and can easily be tweaked (eg add a ‘private’ component).
- Europe, Canada, US and Japan all have commitment from their governments and are now working together. There is a ground swell of enthusiasm from Australian clinicians and researchers to join the global movement; and these countries are imploring Australia to collaborate to ensure the high quality research is undertaken and applicable world-wide. However, Australia currently lacks the organised paediatric research infrastructure to participate.

Question 13: In considering whole of life investment including decommissioning or defunding for national research infrastructure are there examples domestic or international that should be examined?

- Health Technology Assessment Programme (UK) assesses the broad impact of infrastructure including the costs.

Question 14: Are there alternative financing options, including international models that the Government could consider to support investment in national research infrastructure?

Shared models between government and others, such as:

- Canada: <http://www.cctcc.ca/> - The CCTCC is a collaboration between the Canadian Institutes of Health Research (CIHR) (government), Innovative Medicines Canada (industry), and HealthCareCAN (healthcare organisations)
- UK: <https://www.crn.nihr.ac.uk/> - NIHR Clinical Research Network – supports commercial and non-commercial clinical research
- USA: <https://c-path.org/about/> - public-private partnership between academia, industry and the FDA

Health and Medical Sciences

Question 15: Are the identified emerging directions and research infrastructure capabilities for Health and Medical Sciences right? Are there any missing or additional needed?

- While many of the capabilities touch on clinical trials there is no mention of supporting or establishing clinical research infrastructure. Clinical trials are the coal face of research translation into practice. The emerging trends all have a focus on translation which is vital however as a field of research itself, clinical research is often overlooked between basic science and clinical care. In addition, paediatric clinical research is often put in the too-hard basket because of its complexities. However, building capacity in paediatric clinical research, starting with trials, is imperative in developing a comprehensive pipeline from bench to bedside and should be supported as a cross-disciplinary pillar of health and medical science.
- Developing and investing in paediatric clinical trial infrastructure in Australia will secure Australia's place on the world stage while at the same time ensuring a national benefit for generations to come.

Question 16: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- <http://www.ecri.org/> - European Paediatric Clinical Trials Research Infrastructure - European clinical research infrastructure network (EPCTRI ECRIN)
- Global Paediatric Clinical Trials Network is in set-up: <http://c-path.org/critical-path-institute-establishes-the-global-pediatric-clinical-trials-network-pre-launch-consortium/>

Question 17: Is there anything else that needs to be included or considered in the 2016 Roadmap for the Health and Medical Sciences capability area?

- Clinical Trials , in particular paediatric clinical trials - See Question 15 above. PTNA strongly encourages the **inclusion of paediatric clinical trial infrastructure as an emerging trend** in health and medical science in the National Research Infrastructure Roadmap.
- As sub-set of clinical research, paediatric clinical research lacks the infrastructure that adult research enjoys through alternative structures such as colleges (Royal Australian College of Surgeons, Royal Australian College of General Practitioners etc). Child health is crucial to our nation's future. As well as child-specific disease that can have a lasting impact on a child's future, it is also now well recognised that many adult diseases have their origins in childhood. In spite of its importance, paediatric medicine is one of the medical disciplines with the least high-quality evidence to inform practice. It is difficult to undertake clinical research in children due to the need for parental consent, the heterogeneity of paediatric physiology and pharmacology, the shortage of validated outcome measures, the long timeframe needed to measure important outcomes (waiting years or decades to detect the childhood impact on later life), the need for large volumes of patients for rare childhood diseases and the technical difficulties of obtaining adequate clinical specimens. Without dedicated resources, paediatric clinical research is usually placed in the "too hard basket" by both industry and government funding groups. Paediatric research is a very high-gain activity but is also a relatively high-cost activity.
- PTNA is partially funded through NCRIS. Without this funding the trust and enthusiasm to work together across Australia could not have been achieved in paediatric clinical research. To ensure Australia is ready to embrace the growth in paediatric clinical trials, the infrastructure needs to be in place, the experts need to be developed and trained and patients and families need to be involved. PTNA has the foundations of being the binding element that can secure Australia's position as a leader along with its European, American and Canadian counterparts.
- The Medical Research Futures Fund consultation has also identified paediatric clinical trials infrastructure as a growing need in Australia, regardless the infrastructure will only be optimised if the disparate parts are linked across jurisdictions and action is taken expeditiously.
- Through linking all of the paediatric infrastructure through a hub like PTNA, it can act as a first-port-of-call for all paediatric clinical trials matters be they education and training, data collection and use, access to infrastructure and expertise, quality and standards, transparency, marketing of capabilities, translation of outputs into outcomes, regulatory and legal matters, resourcing, interaction with industry etc. While other infrastructure like Academic Health Centres are excellent local initiatives, NCRIS's increased investment in PTNA is crucial to overcoming jurisdictional boundaries to deliver truly national paediatric research infrastructure.

Environment and Natural Resource Management – N/A

Question 18: Are the identified emerging directions and research infrastructure capabilities for Environment and Natural Resource Management right? Are there any missing or additional needed?

- [No answer provided].

Question 19: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- [No answer provided].

Question 20: Is there anything else that needs to be included or considered in the 2016 Roadmap for the Environment and Natural Resource Management capability area?

- [No answer provided].

Advanced Physics, Chemistry, Mathematics and Materials – N/A

Question 21: Are the identified emerging directions and research infrastructure capabilities for Advanced Physics, Chemistry, Mathematics and Materials right? Are there any missing or additional needed?

- [No answer provided].

Question 22: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- [No answer provided].

Question 23: Is there anything else that needs to be included or considered in the 2016 Roadmap for the Advanced Physics, Chemistry, Mathematics and Materials capability area?

- [No answer provided].

Understanding Cultures and Communities – N/A

Question 24: Are the identified emerging directions and research infrastructure capabilities for Understanding Cultures and Communities right? Are there any missing or additional needed?

- [No answer provided].

Question 25: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- [No answer provided].

Question 26: Is there anything else that needs to be included or considered in the 2016 Roadmap for the Understanding Cultures and Communities capability area?

- [No answer provided].

National Security – N/A

Question 27: Are the identified emerging directions and research infrastructure capabilities for National Security right? Are there any missing or additional needed?

- [No answer provided].

Question 28: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- [No answer provided].

Question 29: Is there anything else that needs to be included or considered in the 2016 Roadmap for the National Security capability area?

- [No answer provided].

Underpinning Research Infrastructure – N/A

Question 30: Are the identified emerging directions and research infrastructure capabilities for Underpinning Research Infrastructure right? Are there any missing or additional needed?

- [No answer provided].

Question 31: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- [No answer provided].

Question 32: Is there anything else that needs to be included or considered in the 2016 Roadmap for the Underpinning Research Infrastructure capability area?

- [No answer provided].

Data for Research and Discoverability

Question 33 Are the identified emerging directions and research infrastructure capabilities for Data for Research and Discoverability right? Are there any missing or additional needed?

- Clinical trials management systems, data capture and corporate management systems are as important to clinical research as the expertise to use the software. WebSpirit, a clinical research data capture system, is one example of an application licenced to the Paediatric Trials Network Australia, made possible through significant investment through NCRIS.
- WebSpirit has the potential to be the premier data capture system used for all types of clinical research if focused software development work is undertaken. Storage space and hosting of servers for this one system poses a logistical and financial challenge.

Nonetheless, WebSpirit addresses the data capture need but clinical trials management systems and corporate management systems are a common lacuna across all types of clinical research.

Question 34: Are there any international research infrastructure collaborations or emerging projects that Australia should engage in over the next ten years and beyond?

- [No answer provided].

Question 35: Is there anything else that needs to be included or considered in the 2016 Roadmap for the Data for Research and Discoverability capability area?

- See Question 33 –further investment in clinical research data capture systems, and Clinical Trial Management Systems and Corporate Management Systems.

Other comments

If you believe that there are issues not addressed in this Issues Paper or the associated questions, please provide your comments under this heading noting the overall 20 page limit of submissions.

- As stated above, clinical research as a field of research is missing from roadmap. While it is both affected and effected by other capabilities such as those identified under Health and Medical Science and Data for Research and Discoverability it lacks the recognition it deserves as a cross-disciplinary mechanism for producing evidence to translate into clinical practice, prevention and education.
- Infrastructure for developing investigator-initiated research is not well supported in any State or Territory in Australia. Where infrastructure does exist it is often cost-prohibitive for users or geographically inaccessible. In addition, commercial clinical research is also undertaken for public good, not just to subsidise trial centre cashflow. Products researched in Australian populations have the potential and intention of being marketed and accessible in Australia. The needs of commercial and non-commercial research differs however basic building blocks need to be in place to undertake either or both.
- As clinical research has not been well supported thus far, it is almost a green field for infrastructure investment. It offers the opportunity for a relatively high return on investment and value for money in terms of better health outcomes for Australians and lower costs to the Australian health care system.
- The time is ripe for Australia to invest in paediatric clinical trials infrastructure, like PTNA, to join Europe, Canada and the US in the global march toward better health for children through research.