

Review of the National Innovation System

Australian Stem Cell Centre Submission

13 May 2008

Declaration of Interest

The Australian Stem Cell Centre Ltd is funded by the Commonwealth Department of Innovation, Industry, Science and Research as well as the Australian Research Council. The Australian Stem Cell Centre is a not-for-profit company limited by guarantee and has developed corporate partnerships with a number of organisations. For a list of university and institutional stakeholders, go to www.stemcellcentre.edu.au.

Abbreviations

ASCC / the Centre	Australian Stem Cell Centre
NH&MRC	National Health & Medical Research Council
ICSCN	International Consortium of Stem Cell Networks
SNAP	Stem Cell Network of the Asia Pacific Region
DIISR	Department of Innovation, Industry, Science and Research
ARC	Australia Research Council

Submission Details

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About the Australian Stem Cell Centre

The Australian Stem Cell Centre (ASCC), a Biotechnology Centre of Excellence, is an internationally prominent organisation focussed on undertaking targeted stem cell research and translating the outcomes to therapeutic benefits for patients in Australia and across the globe with additional economic benefits for Australia.

The establishment of the Australian Stem Cell Centre was a key initiative of the Commonwealth Government's *Backing Australia's Ability* policy, announced in 2002, that sought to accelerate innovative and internationally competitive research endeavours in the field of stem cell technologies in Australia. A second round of funding under *Backing Australia's Ability – Building our Future Through Science* brought the total Commonwealth Government funding commitment to AUD \$98.6 million with a further AUD\$11 million from the Victorian Government and \$3.2 million from Monash University.

The ASCC headquarters is located on the Monash University campus in Melbourne with a second major node newly established at the University of Queensland in Brisbane. The ASCC is currently funded by the Department of Innovation, Industry, Science and Research (DIISR) as well as the Australian Research Council (ARC) through until June 2011.

The ASCC model is based on a foundation that supports the fundamental principle of scientific excellence in parallel with commercial opportunity and advantage. With an independent skill-based Board the ASCC has been able to identify and coalesce Australia's scientific strengths in stem cell research into a key therapeutic focus selected after considerable diligence examining scientific, commercial and legal factors. The ASCC is committed to a vision that is aimed at developing and delivering therapeutic benefits, with our first therapeutic focus being in the area of haematology – a competitive strength in Australia for many years. This vision is complemented by a dedicated multidisciplinary research structure that currently and in the future will continue to address fundamental scientific challenges in the field of stem cell research and provide the basis for the development of new future therapeutics.

Biotechnology Centre of Excellence (BCE) funding and the ASCC model together are beginning to address the gap in support between research discovery and the commercial and clinical delivery. A major component of the ASCC is a focus on the requirements for translation including: directed research, intellectual property and regulatory strategies, business development and eventually the implementation of clinical trials. The positive partnership between the ASCC, the Government, stakeholders and scientists is a significant feature of the ASCC and essential if BCE funding is to maximise the dual goals of research and translational excellence.

Stem cell research and regenerative medicine hold enormous therapeutic promise. The broad application of stem cell technology to a vast array of human diseases and conditions is possible. Many centres around the world are exploring the potential of stem cell technology. However, very few are applying the same degree of strategic focus to the selection and development of projects into programmes focused on a common therapeutic goal. Australia's approach to funding and organising a national stem cell initiative is unique in comparison to other international examples in this sector.

This submission is based upon our specific experiences in establishing and operating the ASCC. We recognise that innovation is a very broad system and highlight that our comments in this submission are restricted to our experiences in medical research and the biotechnology sector. During this time we have identified a number of key success factors as well as specific barriers in achieving the success of the Centre. Based on an analysis of these factors we have developed our recommendations.

Success factors

The Australian Stem Cell Centre has experienced the following to be critical success factors that most influence the process of innovation and the achievement of success.

- **High quality people** – Innovation is people, hand in hand with research excellence, the organisation must ensure it has the ability to attract and retain high quality people with diverse skills such as science management, intellectual property management and business development. In addition to recruitment the ASCC is committed to ongoing training to continue to develop critical skills and retain talent. High quality people will be dependent upon levels and longevity of funding, quality infrastructure and a clear vision and mission that is achievable and realistic.
- **Research excellence** – An essential component of the science strategy of the ASCC is to select and support research of the highest calibre. Research excellence ensures a quality platform from which the best students and the ongoing training of skilled scientists can grow, which will contribute significantly to building the intellectual capacity of the organisation and the sector itself.
- **Strategic focus** – A critical component of innovation at the ASCC is our strategic focus in areas of competitive strengths which will deliver therapeutic outcomes. Strategic focus also facilitates the evolution and development of multidisciplinary networks working towards a common goal across institutional, technological and geographic boundaries.
- **Independent board** – Though funded by government, the independence of the ASCC's skill based board ensures objectivity, accountability and access to skills that ensure a clear focus on the strategic goals of the organisation to deliver therapeutic benefits and achieve long term sustainability.
- **Excellent relationships** – Partnership with government and the requirement to report to multiple agencies requires a highly functional relationship with government that is mutually beneficial and shares a common vision. The ASCC has experienced the benefit of such a relationship with government.
- **Continuum of support from research discovery through to commercialisation** – Through combining research excellence with commercial competitiveness, the ASCC has built a robust bridge from discovery research to commercialisation. In support of this, the innovative funding of the Biotechnology Centre of Excellence program, which specifically funds the ASCC, has helped address a significant gap in the translation process.

Major barriers

The following are major barriers to innovation and translation success in light of the experiences of the Australian Stem Cell Centre over the past five years.

- **Short term perspectives must be converted to long term vision and support to achieve maximum success in the full spectrum of innovation.**
 - Short time frames severely reduce the ability to attract and retain key talent.
 - Students often report feelings of uncertainty and hesitation when considering embarking upon a PhD due to concerns about continuity of funding to projects or laboratory.
 - Long term stability engenders a strong sense of security and commitment from employees which translates to higher productivity.
 - Lack of stability creates difficulties in developing the shared vision.
 - Short time frames can only realistically achieve near term goals which fall short of the critical proof-of-concept or preclinical stage of developing therapeutics.
 - Successful translation of research discoveries into therapeutic outcomes and commercial success requires long term funding support if national returns on investment are to be achieved.
- **Competition not collaboration**
 - Collaboration between groups is often impeded by a highly competitive environment where research activities are frequently only partially funded. Groups that are ideal collaborators often need to compete against each other for funding.
 - Collaboration takes time to mature and needs to be supported with adequate long term funding to enable teams to weave a relationship that is mutually beneficial. This can often be a particular barrier to attracting international collaborators.
- **Lack of coordination across the Innovation System leads to inefficiencies, gaps in funding coverage and duplication.**
 - Long term innovation funding should be from one government agency to ease the burden of reporting on recipients and to ensure a common vision. For example, stem cell research falls across several portfolios and funding agencies – ARC, DIISR, NH&MRC as well as the Department of Health. In the case of the ASCC, in cooperation with the Commonwealth, we have been able to report to three Federal and one State agencies using one report.
 - The vision and the expected outputs may differ between agencies, therefore considerable coordination at a government level and at the research recipients end is required. This results in significant administrative overhead and the inefficient use of scientists talents.
 - Lack of coordination across government agencies can also lead to funding silos. It is difficult to grow major activities when our ability to compete for funding is prohibited.
 - On occasion duplicate organisations can be established or funded for political or social reasons with no coordination applied at a government level to ensure the greatest return on the whole-of-government investment in the field. This encourages duplication of infrastructure and resources.
- **Translational Gap.**
 - The high cost of preclinical and clinical trials need to be recognised by all participants in innovation and must be met if new therapies are to be developed. By providing sufficient funds for these translational activities, the opportunity to accrue commercial benefits in Australia will increase and return on investment improved.

- There is a lack of availability of high risk capital. There is a dearth of such sources in Australia. There is a need to access significant Venture Capital investment if we are to succeed in our commercialisation efforts.
 - No coordinated funding for translation has been established to create critical mass and experience in this area.
 - Lack of creative funding opportunities for translational activities leads to poor investment strategies.
 - BCE funding is a significant initiative that has made a positive impact in addressing the funding gap.
- **Limited availability and access to expertise.**
 - Intellectual capital is Australia's greatest deficit. Not having the critical skills to undertake specific scientific discovery through to translation and commercialisation has a significant negative impact upon success.
 - A disproportionate emphasis on attracting 'high flyers' into innovation sectors from within Australia or abroad does not always correlate with quality nor innovative thinking.
 - The need for an education strategy which will develop the next generation of home grown talent to achieve critical mass in innovation is essential.
- **Institutional and technology-centric paradigm.**
 - This creates a highly competitive environment between researchers within institutions and between institutions.
 - This results in no incentive to create collaborative networks coupled with considerable difficulty in achieving agreement.
 - "How much can we get out rather than how much can we put in" is exacerbated by under funding of university research and their consequent inability to contribute towards partnerships.
- **Publish versus patent tension.**
 - Collaborations between industry and academia will almost always encounter the inherent tension between an academic requirement to publish versus the industry requirement to patent a discovery.
 - Australian universities and research institutes are excellent research engines and experienced at knowledge transfer but not uniformly experienced nor motivated to commercialise research results.
 - Translation is not a priority for many universities and research institutes, nor do they adequately provide incentives to translate research discoveries into therapeutic and commercial benefits. There is frequently a disparity in the vision and definition of success between academic institutions and commercial organisations.
 - Cultural disparity between organisations can result in cultural barriers. Rather than embracing the continuum of research to commercial goals cultural barriers can result in fragmentation and failure.

Recommendations

Building an Australian Knowledge economy and supporting a national innovation system will require a number of issues to be addressed: investment in enhancing our intellectual capital; building collaborative knowledge-based networks with world-class infrastructure; removing barriers to translation success; investing in public education to build a culture of support ; working across technology, cultural and geographic borders and recognising the importance of globalisation.

The Australian Stem Cell Centre recommends that:

- I. **The industry sector, government and academia unite to invest in building, attracting and retaining greater intellectual capital.**
 - **Build skills in science, commercialisation & management.** The greatest challenge to the biotech sector is the need to build greater intellectual capital in this country. Industry, academia and government must collaborate as well as work individually to encourage more undergraduates into careers that will contribute to a knowledge economy. We must also encourage the attraction of overseas skills, whether expat or other, to enhance skill capacity. Talent management and skill enhancement that supports an innovation economy are pressing needs. The lack of such talent is a major barrier to scientific and commercial success for many research organisations and biotechnology companies now and in the future.
 - **Establishment of a national innovation education strategy.** The ASCC advocates the development of a national innovation education strategy that aims to create a workforce that can address the needs of a knowledge-based economy. There is opportunity to engage with the industry sector in funding post graduate scholarships and internships, particularly in areas of science and technology as well as fields that support translation including strategic planning and management. Exchange across national and international borders should be encouraged. Australian post graduates should be openly encouraged to travel overseas and provided with an incentive to return with their newfound skills. Australian industry, academia and the government should all encourage long term learning opportunities and skill enhancement, particularly in innovation industries.
 - **Appropriate incentives for commercialisation success.** It is equally as important that the biotechnology industry and research organisations develop systems that support commercialisation by identifying and implementing appropriate rewards and incentives for scientists recognising inventorship and successful translation. Public engagement and support of innovation is an essential factor to encourage and support these activities by creating positive reinforcement and social recognition of achievement.
 - **Invest in building positive social environments that serve to attract and retain intellectual capital.** Optimising the social environment to embrace innovation should not be underestimated nor considered the 'soft' requirement. Organisations and leaders need to develop appropriate social, political and cognitive strategies to create an environment that is conducive to innovation. The medical research field, specifically stem cell research, benefits in a myriad of ways when the social environment is supportive; students are encouraged to work in the field, it is helpful in attracting and retaining key talent, it attracts additional investment and the public show their support for government sponsored programs and new legislation allowing the science to progress.

II. Australia should encourage the development of collaborative and connected knowledge networks. The focus in Australia should move from an institutional or technology-centric model to a network or community-centric model of innovation.

- **Adopting the new network-centric paradigm requires moving away from individual silo's of research and development within relatively isolated institutions.** This must be replaced by coordinated, open, collaborative and multidisciplinary networks-centric models that work towards achieving a common goal that is often focused on a specific therapeutic interest. Such knowledge networks should be enabled to evolve across institutional, technology and geographic borders.
- **Encourage social capital in knowledge-based networks.** Social capital in knowledge-based networks fuels collaboration and the exchange of ideas, which enhances the innovation process significantly and accelerates the pace of advancement within the network.
- **Regional innovation systems, particularly bioregional innovation systems, which take advantage of unique regional knowledge, infrastructure capabilities and access to joint funding schemes, should be encouraged.** A regional bio-economy would also seek to offer much wider opportunities for students as well as systems for skill development. Regional innovation systems would share the risk across stakeholders and develop positive regional relationships.
- **Scientific communities should embrace global networks with enthusiasm.** The emergence of a new international, network – centric paradigm has significantly assisted the rapid rate of scientific development as is occurring in the stem cell field. Globalisation brings vast benefits, particularly in the enhancement of knowledge and skill development.
- **The Australian Government should continue to support focused Biotechnology Centres of Excellence (BCE).**
 - To build critical mass and strategic focus in areas of international competitive advantage.
 - Long term, to enable better returns on the investment, both in building capacity in skills and infrastructure, as well as in commercial returns.
 - The ASCC believes the BCE program has had a significant and positive impact on addressing the translational challenges in stem cell research. The program is appropriately focussed to achieve a commercial and clinically beneficial outcome.
- **The Australian Stem Cell Centre is an example of a network arrangement with the emergence and strengthening of the Melbourne node of the ASCC and the newly launched Queensland node.** These activities demonstrate the power of 'clustering' or networking participants. This enables knowledge sharing, delivery of significant infrastructure capabilities and partners to achieving a defined and common goal.

III. Generate and provide access to innovative funding opportunities that specifically support (a) long term 'frontier research' goals and activities and (b) translation.

- Long term and substantial funding should be prioritised to those major projects and initiatives that:
 - (a) Address national research priorities.
 - (b) Are collaborative and multidisciplinary.
 - (c) Create research and industry communities with a common goal.
 - (d) Have a governance structure that allows stewardship of research outcomes to translation with near, intermediate and long term product development opportunities. There must be a common purpose and specific achievable goals to galvanise and drive priorities.
- **The government needs to provide access to funding and investment opportunities specifically quarantined or designed to support translation.** Being very pragmatic, one of the most important components in translating biomedical technologies to treatments is the availability of funding. Australia abounds in biomedical research discoveries. The Australian research community has well established its productivity and international competitiveness. This enormous cache of innovative research and discovery is valuable in its own right, however translating discovery into treatments which would enhance this value many fold, is very difficult and expensive. The challenge is to bring innovative funding solutions to this translation process to enhance our chances of success. Funding must be sufficient to enable progression of a lead product at least through Phase I clinical trials. This will significantly mitigate risk and improve the attractiveness of an initiative to third party investment. Significant funds need to be applied to developing a research engine that will produce the next generation of products to decrease long term risk to achieve a sustainable initiative. The research engine needs access to competitive funds if it is to grow. Any major biomedical innovation initiative must be at least equal to and preferably of greater attraction for third part investment compared to the more traditional investments in mining, retail and industry.
- **Government should act to facilitate third party investment in the innovation economy.** The cost to develop one drug today (including failures) is approximately USD\$800 million and takes 12-15 years. Without a major pharmaceutical research or biotechnology industry in Australia, third party investment funds must be sourced. It is not the role of governments to fully underwrite such an expense, but governments can act to facilitate such a third party investment by essentially being the 'glue' funding that brings sectors together. In the absence of a pool of private high risk capital, Government will need to come up with ways of plugging the innovation funding gap. Whilst there are several Government programs aimed at stimulating the Venture Capital sector, the job is not yet complete and the sector remains underdeveloped. The Government should persevere with the existing programs and consider new initiatives, particularly with regard to the specific difficulties faced by the biotechnology sector because of its long lead times.

The ASCC's model of innovation

There are key features of ASCC structure and implementation which are addressing the delivery of innovation from basic research to therapeutic and commercial success. These include:

- From its beginnings as an initiative across government, the ASCC has been innovative through its strategic focus on translating leading edge research to therapeutic benefits and commercial success.
- The ASCC Board is independent and skill based covering the continuum of basic research to full commercialisation.
- The ASCC interacts with stakeholders under a defined agreement. This agreement enables the ASCC to own and develop IP from the research it funds at stakeholder institutions.
- The ASCC conducts research in both ASCC facilities and in stakeholder institutions thereby balancing basic research with more applied or directed research.
- The ASCC has highly strategic and focussed research activities selected and managed through rigorous science management systems.
- The ASCC has selected, through extensive due diligence, haematology as its major therapeutic focus. This is outcome driven research with near, intermediate and long term product perspective. This is a high risk strategy but is essential if we are to compete with more extensively funded stem cell research activities around the world. With additional funding the ASCC can enable additional therapeutic areas to be implemented with the same rigour. These products will deliver new therapies which will revolutionise the delivery of health care. A near term target is the manufacture of neutrophils (white blood cells) to protect patients from infection when undergoing high dose chemotherapy for cancer. The intermediate term products enable new ways to manufacture blood cells for transfusion to address the international crisis in blood donation and an innovative approach to bone marrow transplants. Long term projects focus on the manufacture of blood from human embryonic stem cells. These products address multi-billion dollar international markets and by their nature require knowledge exchange across research and industry sectors and the focussed integration of public and private sectors to achieve success. These are high technology products requiring a significant expansion of skilled human capital to support such commercial endeavour.
- The ASCC is constructing a bridge from basic research to commercialisation. This bridge includes the integration of strategic planning, science and intellectual property management, business development, clinical and regulatory expertise and experienced product delivery capability. Such a bridge is a unique innovation enabled by BCE funding.
- The ASCC is now forming a for-profit commercial initiative to attract corporate partner investment and venture capital funding. With appropriate ongoing support from government for the basic research engine that will remain within the ASCC Centre of Excellence structure, this initiative can become a definable national model for the implementation of innovation in the translation process and turn an Australian weakness into a true strength. The indices of success in this endeavour are specific, measurable, achievable and targeted. As such, all participants are accountable in their roles. This is not research for the sake of research but translation of research to achieve major patient benefits and commercial prosperity.

Specific comments with regard to the Terms of Reference

1. Identify a set of principles to underpin the role and participation of the public sector in innovation.

- Innovation in biomedicine should be recognised as a continuum from basic research to therapeutic delivery and should be funded as such without gaps in support.
- There must be clarity of the role of the various public sector organisations and recognition of their specific objectives.
- There must be coordination and integration of public sector organisations to achieve the task of building intellectual capital and supporting knowledge-based networks.
- Develop funding regimes to overcome the institutional and technology centric competitive model and foster network centre initiatives
- Government funding should be used as 'glue', binding together knowledge-based networks supporting basic research engines, and fostering translation of the outputs of these engines in a manner that underpins a national innovation agenda.
- An incentive structure must be in place to support public sector participation as a partner in innovation.

2. Develop a set of national innovation priorities to complement the national research priorities, ensuring the objectives of research programs and other innovation initiatives are complementary.

- National innovation priorities should be in the development of innovative systems and platforms that support the commercialisation of quality research outcomes. A whole of government approach is recommended with coordination of innovation priorities across government departments.
- In areas where Australia has the potential for global leadership, foster governance arrangements, (such as the ASCC), that have the ability to bridge basic research to therapeutic outcomes and draw together research expertise to deliver commercial / beneficial outcomes for society.

3. Identify regulatory and other barriers to innovation and recommend ways to minimise these.

- Short term, fragmented support of innovation should be converted to long term network centric initiatives focussed on deliverable outcomes.
- Multiple portfolios of funding agencies with different agendas should integrate as a single innovation entity.
- The translational gap must be recognised and addressed by organisations with the capabilities to bridge the gap.
- Cultural disparity and barriers between sectors such as academia and industry must be addressed by promoting and supporting a continuum of innovation delivery.

4. Examine the scope for simplifying and reducing program duplication and ensuring that any support provided is well targeted and easy to access.

- The support of a single area of technology (e.g. stem cell research) by multiple government agencies with different agendas results in fragmentation and competition. In addition, duplication (e.g. the Australian Stem Cell Centre and the National Adult Stem Cell Centre) can occur with funding from different Departments. A "one stop shop" for innovation programs to meet specific client needs and to achieve greater synergy for all agencies involved would be a significant advance.

- Allow entry for major initiatives such as ASCC to competitive funding if they are to grow.
- 5. Consider the appropriateness, effectiveness and efficiency of the Research and Development (R&D) Tax Concession Scheme in promoting innovation and make recommendations to improve innovation outcomes.**
- The ASCC is currently a not-for-profit organisation and hence has not focussed on a tax concession scheme. However in the near future we will form a for-profit commercial entity which will benefit greatly from a Research and Development Tax Concession Scheme. This however will not solve the innovation funding gap problem which requires Government support in developing a pool of high risk private capital in Australia.
- 6. Consider ways to improve the governance of the national innovation system to support higher expectations of government agencies and industry**
- Whole of government coordination is required at a Federal level.
 - There is a need for greater communication and coordination between Federal and State funding to ensure synergy and recognition of both State and Federal Departments in the initiative.
- 7. Assess the appropriateness, effectiveness and efficiency of the cooperative Research Centres (CRC) Program and make recommendations to improve innovation outcomes.**
- The ASCC has several components to its governance and structure which we feel are aligned with our vision to deliver therapeutic and commercial benefits from stem cells and related technologies. These include independent Board, ability to develop and own IP from funded projects at stakeholder institutions, ability to select projects based on both scientific excellence and alignment with strategic goals, ability to define and focus on a targeted result and ability to support and direct our internal research groups. These and other attributes of the Centre we believe maximises our potential for success.

Scope of Submission

The intent of this submission is for the Centre, in accordance with its obligations to the Commonwealth, to take a leadership role in the development of Australia's research expertise in the biotechnology sector and to propose changes which will enable Australian researchers to remain part of, and so reap benefit from, the exciting advances in medical research that are occurring in stem cell research. Our submission describes and discusses matters relevant to the Review of the National Innovation System largely insofar as such scientific matters pertain to stem cell research and related technologies.

Consultation Process for the Preparation of the submission - The Australian Stem Cell Centre is, by deliberate design, a collaborative environment that encourages consultation. At every level the Centre invites independent review of its procedures and processes to ensure the outcomes are consistent and unbiased. This process is vital to the identification and advancement of high quality science: it is also critical to the spirit of collaboration in general and the essence of the Australian Stem Cell Centre.

This submission has been a collaborative effort bringing together the thoughts and opinions of ASCC executive management, Board Members and administrators, all with a common goal to ensure Australia's position at the frontline of stem cell research and innovation.

Inevitably this paper cannot precisely represent the specific views of each person associated with the Centre. The Centre's stakeholders and wider scientific community each hold their own independent view. The Centre's role is to reasonably represent all of its stakeholders; therefore this submission endeavours to reflect the majority view. The Centre respects the views of individuals and individual organisations.

Once the initial draft was complete the paper was then reviewed by a representative group of senior scientists and the executive management group. Following this, the draft was presented to the Board of Directors of the Australian Stem Cell Centre for comment and approval. The document was then sent to representatives of the ASCC's ten stakeholders for their information. Any suggestions or comments were considered by the ASCC and included in the document if appropriate. Once approval was granted by the ASCC Chairman, Professor Vicki Sara, the paper was cleared for public distribution.

The distribution of this submission includes:

- the Secretariat of the Review of the National innovation System;
- all stakeholders of the Australian Stem Cell Centre;
- all externally funded scientists and employees of the Australian Stem Cell Centre; the Australian stem cell community and industry, and
- the general public via the Australian Stem Cell Centre website (www.stemcellcentre.edu.au) and the Innovation web site administered by the Secretariat.

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