Summary of workshop and outcomes

'Innovating the Marketplace with Stem Cells'

The NSW Stem Cell Network hosted a one day workshop, which attracted 131 registrants from 76 organisations representing sectors as diverse as, commercial and academic research/management, investors, policy makers, lawyers, patent attorneys, venture capitalists, regulators, clinicians, GPs and cell therapy providers. Integration of these sectors is crucial to bringing stem cell therapies to patients.

Twenty one speakers described the progress of stem cell research and innovation in Australia and on a global perspective, outlining the issues faced by the Australian Industry and the cooperative nature of strategies required for successful delivery of stem cell therapies to patients. Breakout sessions, cabaret style cross disciplinary allocated seating, an open floor panel session and informal end of day refreshments encouraged communication and collaboration towards solutions for commercial, educational and regulatory restrictions facing the stem cell industry.

The day was praised by many and described as, "one of the best meetings of its kind that I've been to in 17 years" Peter Mountford

Weaknesses in the Australian stem cell Industry;

Australia is competing against super hubs who have market resources and geographical advantages that we as a single country can never match. The scale and experience of these hubs provides a self-reinforcing advantage over Australia. We must collaborate to be able to compete more effectively and realize the full value of our contribution to global markets.

Sophisticated infrastructure with expensive running costs is essential for delivery of most treatments (cell collection, expansion, patient delivery, storage, imaging etc.) and government now, and in the future, is likely to have to bear the burden of supply. Globally governments are investing further into the innovation process as the lack of an industry model and extensive risks are deterring large industry investors. Government support is critical for effective translation of therapies through such facilities and, in the absence of industry investment, there is a significant opportunity for government led cooperative development.

Strengths in the Australian stem cell Industry;

Governments are investing in the innovation process including funding clinical studies. Australian R&D performance is strong, we have respected scientists and a non-threatening international reputation. We are in a position to attract clinical trials with our CTN strategy and R and D tax incentive. Further the autologous medical exemption offer an avenue for fast translation of Autologous therapies under the clinical care and treatment of a registered medical practitioner. With our stable economy, exceptional international linkage, world leading market commercial engagement (Mesoblast), highly experienced cell processing organizations (Cell Therapies Pty. Ltd.), our 24 hour geographical linkage and respected, non-threatening national capacity, we are uniquely placed for leading a new global cooperative strategy. Australia has strong patient advocacy groups and exceptionally well connected, global representatives in all sectors of the innovation system.

Opportunities in light of global limitations

Even the largest technology states are seeking mechanisms to mitigate risks, primarily a lack of industry investment and foreseeable high treatment costs, posed by end-stage market-led development. The current market led development strategy will clearly struggle to produce affordable solutions and is threatened by the inability to protect intellectual property of cellular efficacy in an innovation sector evolving via diverse incremental improvements. There is no established industry, industry model or entrenched global leaders.

Many nations are investing strongly in the development of regenerative therapies and it has been recognized that the challenges faced by smaller, high technology states are not unique to Australia. Emerging countries facing a different set of challenges are seeking innovative strategies to participate and invest. Undeveloped nations face an ever increasing challenge. Solution providers (i.e. Australia's opportunity to design cooperative frameworks) will find large and receptive markets.

Significant potential for public good supporting developing nations by developing cost effective solutions and global collaborative infrastructure based on government rather than industry support. Clearly the benefits or regenerative therapies are not purely economic and hence are of great interest to many sectors of society.

Calls to action

Based on presentations and discussions on the day the following summarises the calls to action towards building the Australian stem cell industry and reducing the barriers towards getting innovative treatments to patients.

Cooperative development model – "Maybe we should invest less money in stem cell research and more money in developing a viable industry model" (Peter Mountford)

- Understand and align stakeholder incentives - Two proposed R&D programs of an all of government strategy (1) "Many Products Local" – A regenerative medicine industry model by design-Australia benefits via lead time advantages (choosing preferred cooperative partners) and gaining an increased level of global engagement (2) "Academic Social Responsibility" – A new social innovation concept to help address global challenges. Foreign Affairs investment into developing global industries to meet the needs of smaller high tech countries as well as emerging and developing nations.

Points summarised from discussion throughout the day

1. Infrastructure - Support for service provider clinics for cellular therapies that facilitate close collaboration between clinicians and researchers, expedite regulatory burdens and co-ordinate specialised care needed for patients requiring stem cell therapies, similar to the alpha stem cell clinics at CIRM, California. Government funded facilities are essential for lowering industry risk through service integration. As a result of the workshop a committee is being formed to submit a proposal for funding to set up an alpha stem cell clinic in NSW.

2. Facilitating collaboration and knowledge exchange between the sectors – annual national industry meeting. Harness patient advocacy networks to aid in engagement of relevant

bodies. Establishing a national register of relevant researchers, clinicians, investors, ip firms, and biotech companies. As a result of the workshop closer interactions between Australian stem cell groups are being discussed.

3. Medical exemption guidelines for informing patients for a better understanding of treatment being offered and limitations including lack of efficacy studies, non/homologous, non/integrative, protective/regenerative, autologous/allogenic/xenogenic. Requirement for specialist referral for procedure to ensure support for rigorously tested treatments (Orthocell model). Enforce self regulation by organisations offering cellular therapies under medical exemptions ensuring rigorous scientific research to validate each non-homologous use and control of profits made in the absence of clinical data collection.

4. Researchers must realise the role they play in research translation and keep an open cross sector vision to facilitate innovative and new approaches. Systems to ensure financial viability of maximum efficacy therapies is needed to ensure maximum patient benefit as the driver of basic research. Early Mid Career Researcher training schemes - entrepreneurial PhDs co-supervised by commercial or clinical bodies. Industry and/or clinical sign off of proposal on translational grant applications ensuring market or compassionate grounds exist and products are clinically, regulatory and financially viable. Translational success needs to be rewarded in lieu of peer review publication in academic grant applications.

5. Invest in developing a successful business model

    a. Industry supported models are unable to mitigate cell therapy associated risks even in major markets. New model should encourage international collaboration for strength in competing with superhubs while ensuring that Australia maintains sufficient IP, R and D and affordable therapies. Government supported models and orphan disease programs enable compassionate exemptions to lower financial and regulatory burdens - skip phase 3 trials (Japan model) - treat control group 24 weeks after treatment group (MS model). AIM patent box may better support later stage clinical and commercial application.

    b. Models need to be developed that create symbiosis of commercial and compassionate interests. This must be driven by government support as evidenced by the lack of progress in market driven models.

“The time has now come for greater urgency in ensuring significantly enhanced translation from the ‘bench to the bed’” Alan Trounson.

“We need new models for funding and managing research” Alan Trounson.

“The risks are high and this time all nations face a development challenge not unlike our own valley of death. We should design, deliver and lead solutions.” Peter Mountford

As quoted by patient advocate Dr Paul Brock “As Christopher Reeve said ‘Our houses are already on fire; we need you to put them out now!’”

‘The Future is Now!’ Martin Pera.