Introduction

1 What is your name?
Name:
AusBiotech Ltd

2 What is your email address?
Email:

3 What kind of organisation do you work for?
Other

Mission Statement

4 Is the above Mission statement appropriate for the vision and goals of the Mission?
Please provide comments here to the question around the appropriateness of the Mission statement for the vision and goals of the Mission:
Yes.

5 Can the Mission statement be strengthened?
Please provide comments here around how the Mission statement could be strengthened:
There is merit in considering whether the statement can be adjusted to reflect the potential to operate in the global context and make a contribution to global health outcomes. Further, it could acknowledge Australia’s desire to be a centre for stem cell-related jobs, given the enormous potential for high-value jobs that the sector can deliver.

Vision

6 Is the above Vision statement appropriate for the investment being made towards the Mission?
Please provide comments here to the question around the appropriateness of the Vision statement:
Yes.

7 Can the Vision be strengthened?
Please provide comments here to the question around the appropriateness of the Vision statement:
The Vision could be strengthened by specifically calling out successful commercialisation (i.e. product approval) as an outcome we are aspiring to, which would be objective validation of the Vision being realised (in contrast to the one-off individual patient use exemptions or limited clinician “practice of medicine” interventions).

Scope

8 Does the proposed scope of the Roadmap address the scope of stem cells therapies that should be addressed in the context of the Mission?
Please provide comments here to the question around the scope:
AusBiotech believes the Roadmap provides for appropriate stem cell therapies that should be addressed but that it should not be limited to research (as per ‘Scope of the Mission’).

While we acknowledge the funding is being drawn from the Medical Research Future Fund and the research focus of that fund, if the goal of the Mission is the development of innovative treatments, then funding should also support development of research (see ‘Funding Principles’). In this context industry is critical, as industry will be required to help deliver the ‘innovative, safe and effective stem cell treatments’ to patients that the Mission aspires to.

Ideally, the scope would include differentiated progeny of stem cells and exosomes.

Funding Principles

9 Are the key funding principles appropriately articulated?
Please provide comments here around the appropriateness of the principles:
Yes. Please also consider including the need for funded research to address unmet needs and be differentiated from existing/competitor treatments and have the potential to be commercialised (or enable commercialisation).

10 Should any funding principles be further strengthened in other areas of the document?

Please provide comments here to the question around strengthening of the principles throughout the roadmap:
‘Multidisciplinary collaboration’ is not an end in itself. If it assists with commercialisation, then it should be facilitated. Funding should be linked to the potential for commercialisation, for example, the team’s track record of commercialisation in this space, and allow enough scope for development collaboration to be included.

11 Are there other principles that should be followed?

Please provide any additional principles that would strengthen the Roadmap:
N/A

Funding priorities

12 Do the six funding priorities capture the key areas of focus for the sector?

Please provide comments around the appropriateness of the six key flagships:

a. Targeted research funding:
Research on ethical, societal and regulatory issues appears to be captured in the funding priority ‘Ethics, engagement and policy.’

The emphasis on moving from preclinical to clinical is justified providing all preclinical requirements for IND have been met (e.g. Quality and Manufacturing Controls; Pharm/toxicology; Biodistribution; tumorigenicity).

In addition to patient advocates helping to define the target product profile (amongst other important roles), key members of the team should have an industry track record in stem cell development be it Investigational New Drug (IND)/CTN clearances, Biologic License Application approvals, etc. To achieve the Roadmap’s aims, individuals with proven expertise in the development, manufacturing, regulatory, quality, clinical, and commercial dimensions of stem cell therapies are needed.

b. Clinical translation:
See above regarding the recommendation to satisfy all preclinical requirements for an IND rather than taking advantage of the more liberal regulatory environment in Australia allowing arguably premature conduct of clinical trials. This is an important safety concern.

c. Ethics, engagement and policy:
In addition to engaging patients and advocates, given the Roadmap’s objectives, it would be beneficial to engage health economists with expertise in reimbursement and market access and regulatory specialists with international experience to ensure safe and effective treatments reach the Australian public.

d. Capacity building and workforce
We suggest linkages between business programs (e.g. MBAs) and research teams to foster entrepreneurial activity. The greatest capacity building will come from hands on experience working in teams/companies developing stem cell-based products and bringing them to market. Providing financial means for researchers to commit a significant amount of time to developing their research so that their other research interests don’t languish, or they can employ others to maintain momentum in their lab will reduce the opportunity cost of their involvement in commercialisation.

e. Commercialisation
We welcome the explicit identification of ‘commercialisation’ as one of the funding priorities. The priority should more explicitly identify industry as a key driver of commercialisation and the necessity of engaging industry in this process.

Industry is an integral part of developing stem cell products and without industry as a key partner, patients will not be able to have access to the ‘innovative, safe and effective stem cell treatments’ the Vision aspires to.

f. Infrastructure
Seed funding of preclinical CROs (e.g. that provide animal models to conduct required GLP studies for pharm/toxicology, biodistribution and tumorigenicity) would be an important value add to the sector.

13 Are there any specific areas of actions that can be suggested under each priority?

Please provide any further areas of action for each flagship:
See responses to previous question.

Also, see ‘Regenerative Medicine: Opportunities for Australia’, developed by MTPConnect and L.E.K. consulting in conjunction with CCRM, AusBiotech and other stakeholders. Key actions identified in this report that align with the Mission’s objectives include the need to create a clear market access pathway and securing long-term investment in the sector (inclusive of private investment and international venture capital).