Institute for Molecular Bioscience
The University of Queensland
St Lucia, Qld, 4072

Elton Humphery
Secretary, Community Affairs Committee

Dear Sir,

Re: **Legislative responses to recommendations of the Lockhart Review**

I am writing to you in response to your email of 18th September calling for written submissions in response to the recommendations of the Lockhart Review.

Background to my involvement and interest in this topic
I am a molecular biologist with a specific interest in the kidney. Over the past five years, I have moved into the area of investigating Chronic Kidney Disease, a condition which is growing at the rate of 8% per annum, can not be cured and can currently only be treated by dialysis or transplantation. The number of Australians being treated with dialysis or a kidney transplant has increased more than fourfold since the 1980s. By 2010, the direct costs to the health sector of providing these services to current and future patients are likely to exceed $4.25 billion. Hence, there is a desperate need for alternatives. In order to progress the development of such novel treatments, I founded a multidisciplinary collaborative consortium of researchers based at the University of Queensland and Monash University entitled the Renal Regeneration Consortium (http://www.renalregeneration.com). The objective of this consortium in the long term is to develop novel cellular therapies for the treatment of Chronic Kidney Disease. We have not focussed on one approach but have recognised the scientific fact that we are not yet in a position to say what sort of stem cells will be able to do what sort of regeneration. Hence, we are working with both adult and embryonic stem cell lines. Much of our work to date has been funded by the National Institutes of Health, USA, and hence we have been restricted in the human ES cell lines that we are able to use. We now have funding from the Australian Stem Cell Centre to continue aspects of the work.

From this preamble, it is clear that I am a scientist who is very interested in pursuing research on stem cells. I am also a scientist with a clear passion to do research that can be translated into the clinic. To this end, I have filed a number of patents and formed a start up company, Nephrogenix Pty Ltd.

The potential outcomes of stem cell research
It is my honest opinion that much of the public discussion about therapies from stem cells is premature. While the long-term objective is obviously some advancement in medical science, it is possibly more likely that research into stem cells will not lead to the cures that we currently imagine. I would argue that there is likely to be two often undiscussed outcomes of this research that may well have far greater implications for human health and a much greater chance of being delivered. The first of these is increased biological understanding. Our fundamental understanding of how a cell is directed to become a specific type of cell remains vague. The fact that there is an ability to change the fate of a cell such that it takes on another form is only just becoming accepted in cell biology and this has come out of recent advances in stem cell science. How and when and why this happens during normal processes of

response to injury or disease can start to be addressed by investigating processes such as how a nucleus is reprogrammed during somatic cell nuclear transfer. This will have very broad implications for our understanding of biology and medicine. It may also ultimately allow us to avoid the derivation of a blastocyst at all, which would be a position morally acceptable to all. The second outcome of stem cell research that is often overlooked is the development of such cells as screening tools. To have a supply of potentially patient-specific human cells to screen compounds in development for human use is very likely to revolutionise the pharmaceutical and biotechnology industries and lead to increased safety in new products.

The current legislative position in Australia and the implications of the Lockhart Committee recommendations

The ethical decisions reached by the *Prohibition of Human Cloning Act 2002* and the *Research involving Human Embryos Act 2002*, these representing the current state of law in Australia, deem that it is acceptable to harvest human ES cells from human blastocysts collected for IVF with the consent of the owners and under a licence. This acknowledges that, while not the opinion of all in our country, our society does not regard the blastocyst as having equivalent rights to an implanted embryo, fetus or a postnatal human being. A blastocyst is a ball of undifferentiated cells with no capacity to self-sustain or to differentiate without successful implantation into a womb. It is a seed. It has potential and no more. We condone the discard of such tissues as a part of IVF, hence there is no additional ethical dilemma in using these cells for some other purpose. That decision is now history and I believe was based on sound judgement with wide-ranging public input. It is also reflective of the opinions reached in many countries around the world.

The Lockhart committee reaffirms the acceptability of our existing legislation with respect to the regulated derivation of new human ES cell lines. The key recommendation of contention resulting from the Lockhart committee is the recommendation to lift the ban on somatic cell nuclear transfer. I do not see any additional ethical dilemma in the legalisation of this process. In contrast, I strongly uphold the need to ban reproductive cloning, as is also reinforced by the Lockhart Committee recommendations.

Why is there no additional ethical dilemma with somatic cell nuclear transfer? Our society not only accepts but strongly supports the rights of its citizens to have access to Assisted Reproductive Technologies (ART), including In Vitro Fertilisation (IVF). The processes of ART involve the destruction of blastocysts created via the in vitro unison of an oocyte and a sperm. This destruction occurs during the derivation and culture process, during the training of technicians and in the process of discarding unwanted excess blastocysts. Somatic cell nuclear transfer involves the reprogramming of an oocyte via the removal of its nucleus and the introduction of a nucleus from an adult cell. The introduced nucleus is reprogrammed by the environment of the oocyte and the resultant cell behaves like a fertilized zygote, dividing to generate a blastocyst. In this way, a human embryonic stem cell line could be generated from a specific genetic background. There is no additional ethical dilemma here. The ethics is not any different to that for the derivation of a human ES cell line from an existing blastocyst.

Why do we need more hES cell lines and why can't we just keep making them the way we have?

This question is regularly raised in relation to the issue of whether or not to accept the Lockhart recommendations. The answer to why we need more ES cell lines

remains the same as it did at the time of the previous debate. The existing lines were derived at a time when the procedure was suboptimal. Many of these are no longer of use due to chromosomal instability. Essentially none of them were derived in a manner that would allow for their use in clinical trials. Why has this not changed since 2002? It is in the process of changing, but the process of deriving a human ES cell line is technically challenging and there are still very few people on the planet able to do it. This will take time to change. One of the most important objectives of the Federally-funded Australian Stem Cell Centre is capacity building in the area of stem cells such that there are more people able to derive, culture and investigate these cells. The question of why we need to use SCNT is usually answered by saying that this will give us an opportunity to derive autologous human ES cell lines to treat a specific person with a specific condition. It is much too early to know whether this will even be scientifically or commercially feasible, In fact, it is possible that the latter is the greater obstacle to this ever being delivered. However, the derivation of hES cell lines in this way will enable us to increase our understanding of normal development, abnormal development, nuclear activity and our ability to reprogram one cell type into another. This understanding will be of great importance to the development of new treatment techniques and the manipulation of cell type during disease. To be able to develop a human ES cell from a patient with a disease of development is likely to give us significant insight into what has gone wrong in embryonic patterning. Such understanding can never be gained by simply harvesting existing hES cells from an IVF blastocyst.

The continuing need for consistent federal legislation.
I have recently returned from the United States of America where I travelled for two months as an Eisenhower Fellow investigating the barriers to translation in the area of cellula therapies and regenerative medicine. I have written an opinion piece on this issue in an European scientific journal, EMBO Reports, which I would be willing to supply if required. I also spent some time talking with US senators and congressmen both for and against the legalisation of the derivation of human ES cells in the US. These talks confirm for me the belief that Australia has taken the right route by enacting federal legislation in the area of manipulation of the embryo. This has not necessarily placed us at an advantage, although some have claimed this. However, it has ensured that there is equitable access to the technology for both academic researchers and those within private commercial entities. I would encourage the Government to continue to act in a consistent manner with respect to its enactment of legislation and its interpretation of ethics. If they do this, they will support the recommendations of Lockhart.

Kind regards,

Professor Melissa Little
Institute for Molecular Bioscience
University of Queensland
St. Lucia, 4072, Qld
Australia

Ph +61 7 3346 2054
Mobile 0427770386
FAX +61 7 3346 2101
email: m.little@imb.uq.edu.au