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National Health and Medical Research Council

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Dear Sir/Madam

Regulation of autologous stem cell therapies - Discussion paper for consultation

Thank you for providing the Office of the National Health and Medical Research Council (ONHMRC) with the opportunity to provide a submission to the Therapeutics Goods Administration (TGA) on the regulation of autologous stem cells therapies.

NHMRC is Australia's leading expert body for supporting health and medical research, developing health advice for the Australian community, health professionals and governments, and for providing advice on ethical behaviour in health care and in the conduct of health and medical research.

In December 2013, NHMRC published two resources on stem cells treatments:

- *Stem Cell Treatments – a Quick Guide for Medical Practitioners*
- *Stem Cell Treatments – Frequently Asked Questions*

Although not exclusively tailored to autologous stem cells treatments, these resources provide medical practitioners and patients with information on the stem cells treatments that are available, and the risks involved in undergoing unproven treatments. The comments in this submission are based on these resources. The submission does not attempt to provide evidence, either way, about the safety and efficacy of such treatments.

Copies of both resources are attached to this submission for your reference.

ONHMRC appreciates the opportunity to provide a submission to this consultation. If you would like to discuss these comments in more details, please contact Jillian Barr, Director, Ethics and Governance at ethics@nhmrc.gov.au.

Professor Warwick Anderson AM

Chief Executive Officer
March 2015

ONHMRC Submission to: Regulation of autologous stem cell therapies - Discussion paper for consultation

Overview

Given that autologous stem cell therapies are not currently regulated under the *Therapeutic Goods Administration Act 1989*, key concerns for ONHMRC in relation to their use are:

- medical practitioners can directly access and use autologous human cells without the pre-market scrutiny applied to medicines and devices
- there are no obligations on either companies providing these 'products' or medical practitioners to report serious adverse events, and therefore no consolidated knowledge of adverse events
- direct-to-consumer advertising is permitted, and concerns have been expressed to the TGA and in public forums that this type of advertising can be inappropriate.

This does not attempt to provide evidence, either way, about the safety and efficacy of autologous stem cells treatments.

Medical practice and research

In the case of autologous stem cell treatments, the treatment is being undertaken as part of medical practice using the patient's own cells. However, it could be considered that those providing unproven autologous stem cell treatments are essentially conducting clinical research, and those receiving autologous stem cell treatments are participants in that research. In this case, however, research is being conducted without the regulatory oversight or rigorous ethical review normally expected, for example:

- ethical review and approval by an appropriately constituted Human Research Ethics Committee,[1] including the mandatory reporting of serious adverse events
- compliance with established and accepted scientific and ethical standards for clinical research, including the *National Statement on Ethical Conduct in Human Research, 2007 – updated March 2014*,[2] the *Australian Code for the Responsible Conduct of Research, 2007*[3] and the *Note for Guidance on Good Clinical Practice CPMP/ICH/135/95*.[4]

NHMRC's position is that new medical treatments should be tested through clinical trials to show that they are safe and effective before being made available to the public.[5] The reality is – other than the use of haematopoietic stem cell transplantation for blood and certain immune related disorders – the majority of stem cell treatments are still in the early stages of research and development and have not been demonstrated to be safe and effective.

Informed consent

As with any unproven treatment, or participation in a clinical trial or clinical research, informed consent should be obtained prior to access to unproven autologous stem cell treatments. Informed consent requires an adequate understanding of the purposes, methods, demands, risks and potential benefits of the research,[6] however, it is noted that the need for informed consent is not addressed in the TGA discussion paper.

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[1] Refer to paragraph 5.1.30 of the National Statement.
[2] http://www.nhmrc.gov.au/_files_nhmrc/publications/attachments/e72_national_statement_march_2014_141126.pdf
[3] http://www.nhmrc.gov.au/_files_nhmrc/publications/attachments/r39_australian_code_responsible_conduct_research_150107.pdf
[4] https://www.tga.gov.au/sites/default/files/ich13595an.pdf
[5] http://www.nhmrc.gov.au/health-topics/stem-cell-treatments
[6] Refer to paragraph 2.2.2 of the National Statement.

Direct-to-consumer marketing

If regulation of this industry by the TGA is not supported as an outcome of this review, then it is paramount that practitioners are held to Australian Consumer Law. That is, where practitioners make claims about their treatments, they should be able to substantiate them. Concerns may arise where consumers are misled or deceived into believing that certain treatments are safe and effective when this has not yet been established. It important that potential recipients are made aware that not all stem cell treatments available in Australia have been tested for safety and efficacy and information on the risks and benefits of these therapies needs to be comprehensive and accessible to potential recipients.