Regulation of Autologous Stem Cell Therapies – Discussion Paper v1.0
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This submission is by Miltenyi Biotec Australia Pty Ltd, a subsidiary of Miltenyi Biotec GmbH.

The Miltenyi Biotec mission is to improve scientific understanding and medical progress by providing products and services that advance biomedical research and cellular therapy.
Honouring this mission drives our commitment to support the translation of basic research into therapy in the areas of immunology, cancer, neuroscience and stem cell biology.

The excellence of our products is augmented by the reliability of our support services, giving added value to our customers.
Cooperation and open dialog with external researchers enable the development of pioneering instruments.

One of our principal tasks is to design and coordinate clinical studies to test the therapeutic utility of Miltenyi Biotec's clinical products in areas such as regenerative medicine and stem cell therapy, with an emphasis on haemato-oncological, infectious, and cardiovascular disease, and immunotherapy. Working with many internationally recognized professionals in centres throughout the world, we carry out trials that range from safety/feasibility (phase I/II) to pivotal stage (phase III) and post-market surveillance trials.

In Australia, our CliniMACS® System used in the production of pure cell products for cellular therapy applications is registered with TGA. Our CD34 reagent for the selection of haematopoietic stem cells is also on the ARTG, and we have a range of similar reagents, which although not registered, are available to recognised cell therapy centres via the Special Access Scheme (SAS) or Clinical Trials Notification (CTN).

We are concerned that the current 4(q) exemption allows our equipment to be used to deliver unregistered therapeutics outside a clinical trial and without SAS approval exposing patients to health risks, as well as potentially compromising the regulatory status of our products both in Australia and overseas.

The current 4(q) exemption is ambiguous and has led to legal dispute between Miltenyi Biotec and at least one private autologous cell therapy company.

For the purposes of this submission we would like to limit our comments to the option which we feel would be the most appropriate level of regulation for cellular therapy products.

The preferred regulatory option for autologous cells is option 4.

Option 4 allows for self-regulation of autologous cells provided there is no more than minimal manipulation and/or are for homologous use only.

There would be adverse event reporting and advertising control for all products.

Option 4 would bring the regulations closer to those of the FDA.

It is imperative that there is a level of peer assessment – this is lacking in Options 1,2 and 3. Option 5 can be perceived as being overly restrictive, and would inhibit the development of new cellular therapies.

Our current customers – the cell therapy centres within the Australian public health system already follow a system of regulation through NATA, TGA and FACT accreditation and peer review, and it is not unreasonable to expect all private centres involved in cell therapy to follow suit.

Other issues:

If Option 4 is implemented, it is essential that the definition of products as being Class 1 or no more than “minimally manipulated or homologous use only” be assessed by an external agency such as TGA and not by the provider of the cellular therapy.

TGA’s definition of minimally manipulated is acceptable.

The current definition of homologous use is: **“**The repair, reconstruction, replacement, or supplementation of a recipient's cells or tissues with a biological that performs the same basic function in the recipients as in the donor”.

We would propose that this definition is altered to include wording to the effect “that the tissues or cells are implanted in the same organ system from which they originated.”

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