ACI NSW Agency for Clinical Innovation

3 March 2014

ACI/D15/1707

Therapeutic Goods Administration
Biological Science Section
Office of Scientific Evaluation
Therapeutic Goods Administration
PO Box 100
WODEN ACT 2606

Dear Sir/ Madam

RE: TGA Regulation of Autologous Stem Cell Therapies: Discussion paper for consultation

Please find the below the submission by the Agency for Clinical Innovation - Blood and Marrow Transplant (BMT) Network Council in regard to the proposed options for regulation of currently exempt autologous stem cell therapies.

Background

The Agency for Clinical Innovation - BMT Network Council is made up of clinicians involved in the complex medical process and care of patients requiring use of haematopoietic progenitor cells as part of their treatment. This includes members from every adult and paediatric hospital in NSW involved in the provision of BMT services. Representatives include haematologists, transplant physicians, general & specialised nurses, cellular therapies laboratory staff and representatives of associated organisations such as the Australian Red Cross Blood Service (ARCBS), Australian Bone Marrow Donor Registry (ABMDR) and the Australasian Bone Marrow Transplant Recipient Registry (ABMTRR). These representatives are intricately involved in the regulation and monitoring of transplant activity. In addition, there is a specific quality management service required for ongoing accreditation under National Association of Testing Authorities (NATA) and Foundation for the Accreditation of Cellular Therapy (FACT) as well as licensing under the TGA.

Current practice

It is essential that any regulatory reform recognises autologous haematopoietic progenitor cell (HPC) transplantation as the established best practice for a range of malignant conditions and that it enables research and development that may establish new indications for its use including in autoimmune diseases such as Scleroderma and Multiple Sclerosis. This could be ensured by clarifying the existing exemption to include autologous HPC transplantation for established and emerging indications following guidance by international transplantation bodies such as the BMT Society of Australia and New Zealand (BMTSANZ) and the European Society for Blood and Marrow Transplantation (EBMT). This clarification would ensure not only that autologous HPC transplantation is evidence-based and peer-reviewed but also that evolving indications occur within the context of a clinical trial.

With regards to autologous ‘stem cell’ therapies (particularly derived from lipoaspirates) being offered for other indications we note the importance of enabling innovation while at the same time ensuring that this occurs within a framework that is likely to yield evidence of safety and efficacy. In addition, it is important that adverse events are reported, collated and, if necessary, acted upon. The current regulatory exemption allows the use of such treatments with minimal supporting safety and efficacy data. In addition, there is no

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restriction on advertising of the unproven benefits of autologous ‘stem cell’ therapies to Australian consumers.
A requirement to meet applicable standards would seem appropriate to bring the autologous stem cell sector into line with other cellular therapies (e.g. HPC). Such standards should include requirements for donor evaluation/testing, product processing, labelling and issue as well as quality control evaluation and microbial screening.
Given this, we would support at a minimum Option 4 of the discussion paper, where minimally manipulated autologous stem cells for homologous use (in a single procedure by a physician) would be regulated under the Act as a Class 1 biological. More than minimally manipulated autologous stem cells (such as mesenchymal stem cells) for non-homologous use would be fully regulated under the Act as Class 2-4 biologicals.

Recommendations
The Agency for Clinical Innovation - BMT Network Council support

i. That haematopoietic progenitor cells (HPCs) continue to be included in the Item 4(q) of the Therapeutics Goods (Excluded Goods) order No.1 for reconstitution of blood after treatment of cancer

ii. That use of HPCs for newly established indications such as autoimmune disorders be included within Item 4(q) of the Therapeutics Goods (Excluded Goods) order No.1

iii. That option 4 for regulation of autologous stem cells be adopted as a minimum standard of regulation for currently used stem cell therapies

iv. That AHPRA be requested to review the advertising promoting autologous stem cell therapies to determine if there are advertising breaches; and

v. That consideration is given to a requirement for independent oversight in addition to the self-certification requirement in Option 4 for low-risk products.

Should the TGA require any further information in regard to the above please do not hesitate to contact [redacted].

Yours sincerely

[redacted]

[redacted]

Cc: [redacted]