'Regulation of autologous stem cell therapies'

While we agree that Option 4 would be an appropriate minimum level of regulation to balance the potential risks to patients with clinical autonomy and judgement, we would prefer to see Option 5.

Our concerns about the current regulation are the same as those raised in the Discussion paper, namely that the safety and efficacy of autologous stem cell treatments are not demonstrated either in preclinical research or in clinical trials. This leads to concerns that people are paying for unproven treatments that are advertised on websites, often through testimonials of individuals purporting to have had relief of symptoms or cures.

- There is no guarantee that autologous therapies are safe. Just because cells come from the same patient it cannot be presupposed that when they are placed into a different location they will be safe.
- Without preclinical evidence and clinical trials there is no guarantee that the cells will do anything. In this case the practitioner is fraudulent in offering a stem cell therapy, even if they add riders and small print to their advertising.
- Often a "stem cell therapy" does not comprise "stem cells" but an extract (usually of adipose tissues). There is no quality control and no identification of the percentage of "stem cells" in the mix. They might be as accurate to say they are offering "fat cell therapy", in fact, this would be more accurate.
- All cell therapies should be subject to proof of safety and efficacy via clinical trials.

The question of whether these therapies should be regulated as Class 1 biologicals is an important issue. This Class does not require efficacy requirements, nor manufacturing requirements, except for “minimal manipulation”. In our view this Class is appropriate for early phase clinical trials to allow new therapies to be explored for safety and efficacy. It is not appropriate for treatments of individuals who are not enrolled in a clinical trial. It is not appropriate to allow any medical practitioner to provide “stem cell therapy” based simply on the ability to isolate cells and on a belief that they may be therapeutic, when they have not been proven therapeutic. It is not appropriate, just because medical practitioners wish to give such a treatment.

Our strongly held view is that Option 5 is much more appropriate a regulatory protocol than Option 4. This would prevent medical practitioners from charging patients for unproven and potentially dangerous treatments. This protects the patients and prevents the unscrupulous from financial gain.

It is important that the regulations do not prevent new stem cell therapies from being tested in clinical trials. Furthermore, in order to promote new therapies, “stem cell-” and other cell-therapies should be regulated in different ways. Some may be effective as mixtures of cells whereas others may require purification. Hence, regulations should allow for differences among individual cell therapies.

The biggest barrier to autologous cell therapies becoming regular treatments, apart from the scientific proof of efficacy, will be the cost of individualised treatments. It is not helpful to the field to regulate cell therapies in exactly the same way as devices or drugs. This is especially true of autologous cell therapies. Autologous therapies are going to be too expensive if every individual’s batch of cells had to be considered a "product" with the associated batch quality controls before transplantation. This will make such therapies too expensive. On the other hand all autologous cells for therapy should be subject to manufacturing standards, such as GLP, without requiring GMP certification. One way to control the quality would be to control the processes under which cells are generated from the biopsy tissues. The efficacy of the "cell product" would be associated with the procedure for isolating them rather than with proof of identify and quality as required for other “products” such as should be required for cells sold from batches and used in non-autologous therapies.

In Summary our recommendation is Option 5, which requires (Discussion document page 8):

- Advertising to health practitioners only,
- Act standards,
- Adverse effect reporting,
- Safety requirements,
- Efficacy requirements, and
- Manufacturing standards (that may differ depending on cells and indication).