Regenerative Medicine: Hype and Hope or Safety and Efficacy?

Regenerative medicine, and the underlying stem cell technology on which it is based, offers considerable hope to patients suffering from trauma and acute or chronic disease. Despite this, regenerative medicine can be highly controversial in terms of claims and weaknesses relating to safety and efficacy, the regulatory aspects, the ethical and social aspects, the commercialisation of stem cell technology and – most importantly – the scientific and medical basis of the proposed technology. Regenerative medicine is in its infancy and we must all be very aware that at present, hype and hope are the backbone of the technology. When safety and efficacy are the backbone, then we will truly be in a new trusted area of clinical practice which patients can access with confidence.

The issue of patient safety and treatment efficacy in regenerative medicine is arguably the most important factor in the future of the technology and at present we are in a position of extremes. This is because technology such as bone marrow stem cell transplantation, peripheral blood stem cell technology (using mobilised bone marrow stem cells) and cord blood stem cell transplantation are practised globally with a high level of safety and efficacy. There are many centres of excellence around the world where experts carry out these transplants with extensive regulatory guidance. Patients enjoy optimised safety and efficacy when they are treated by these experienced teams in a perfect setting.

In stark contrast, there are a rapidly increasing number of stem cell-based 'treatments' for which there is little or no safety and efficacy data. These are often provided by stem cell 'clinics' and prey on vulnerable patients who are often looking for a 'cure' when traditional medicine has been unable to help. This is the dark side of regenerative medicine.

The safety and efficacy of treatments offered using stem cell-based regenerative medicine is defined and controlled by the relevant regulatory authorities. Once again, as with safety and efficacy, the regulation of regenerative medicine technology falls into two extremes.

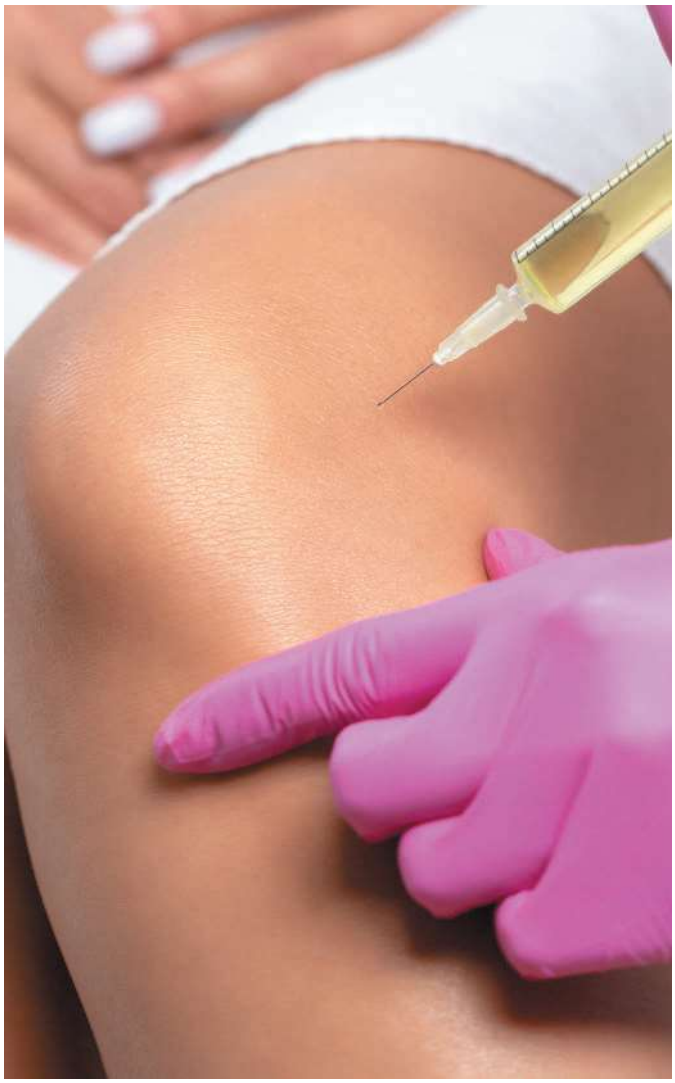
The first extreme is in countries such as the UK and USA, where regulation is well developed and therefore patients are protected and can undergo regenerative medicine treatments with confidence. In the UK, for example, there is the Human Tissue Authority (HTA), the Human Fertilisation and Embryology Authority (HFEA) and the Medicines and Healthcare Products Regulatory Agency (MHRA). These organisations come together to regulate every aspect of stem cell technology, making the UK one of the safest places in the world to be treated using regenerative medicine technology.

The second extreme is in other countries of the world such as India and China where regulation, if it exists, is poor – and the result is that many patients in such countries receive untested and potentially unsafe 'treatments'. This means that in these countries, 'treatments' can be offered which place patients in potential danger and this has been illustrated only too well by reports of patients suffering life-changing damage following poorly regulated 'treatments'. There has also been a considerable rise in 'medical tourism' where patients travel to a country and as part of their visit receive 'treatment' using stem cells. This is a dangerous practice which all patients are well advised to avoid, but the problem is that false information and false promises lure vulnerable patients to have treatment. One of the ways in which we can try to reduce this problem is by patient education, so that patients know what to expect, to ask the right questions and to turn away when things look questionable or even dangerous. We must try to address the problem of patient education by providing clear, understandable advice written with no jargon for the general reader. This will be extremely helpful for anyone considering undergoing a regenerative medicine treatment.

There is, unfortunately, another extreme when considering the regulation of regenerative medicine and this is sadly in places in the world such as South America and many small islands, where there is no regulation of regenerative medicine at all. This total lack of regulation means that anyone can set up a 'clinic' and offer 'treatments' and when doing this, they need not pay any attention at all to the safety and efficacy of the 'treatments' being offered. This is an extremely dangerous situation for patients and we must all try to discourage patients from attending any form of unregulated regenerative medicine 'clinic'.

Regenerative medicine is no different from other medical specialities in that it can raise ethical and social concerns. Ethical issues in regenerative medicine come in many forms; for example, if it is proposed to use human embryonic stem cells as a treatment, then this raises issues about the use of a human embryo to create stem cells. This example not only raises ethical concerns but also, for many people, religious concerns. Embryonic stem cell technology has in fact developed extremely slowly since it was first proposed, and this is largely because of technical problems, but the underlying ethical and religious objections have also contributed to the slow uptake of the technology. There is also the fact that there are not many human embryos available to use to create embryonic stem cells and the technology could therefore never be available on a mass scale. The ethical aspects of regenerative medicine technology also arise in the use of donor stem cells of all types (to ensure the wellbeing of donors) and also in the use of gene insertion technology to produce induced pluripotent stem cells from somatic cells. We all must keep ethical implications in mind when either carrying out or recommending regenerative medicine in the same way as we do in all clinical practice.

The social implications of regenerative medicine are more complex. There is, first, the very obvious fact that most regenerative medicine procedures in most countries can only be obtained by payment and are therefore largely limited to the rich. Payment-only regenerative medicine procedures immediately exclude many people, which may be seen as social injustice, but this is in fact no different to the existing global social injustice in healthcare which we all seem happy to accept. This does not mean that this social injustice is either fair or correct, it just means that regenerative medicine seems to follow the same path as the rest of clinical



medicine. Whether this is a good or bad thing needs further debate. This social injustice may also increase the health status of the wealthy, making the difference between the wealthy and the poor even more extreme than it is today. This is clearly a bad state of affairs, but once again this is a generic problem and not one specifically related to regenerative medicine. It will require a global effort to correct these inequalities.

There are other more subtle social implications associated with specific areas of regenerative medicine and arguably the most important of these is 'anti-ageing'. Ageing is a natural process, based around the ageing of stem cells, which is essential for the ongoing survival of the human race. If we do not have ageing and death, or make significant reductions using regenerative medicine, then planet earth would very quickly become totally overloaded and we would all die. The use of regenerative medicine technology in 'anti-ageing' procedures needs careful consideration and, in my opinion, should not be used. If 'anti-ageing' was successful it could be the beginning of the end for the human race.

The unregulated commercialisation of the stem cell technology used in regenerative medicine is a considerable and increasing problem to us all. There is an analogy within the pharmaceutical industry which is heavily commercialised but equally heavily regulated. This is not the case for Regenerative Medicine which is becoming increasingly commercialised but has little or no regulation on a global scale. The problem is accentuated by business workers who see stem cell technology and regenerative medicine as an easy way to make very big profits in countries where there is little or no regulation. They prey on vulnerable patients who are willing to pay large amounts for untested and unproven 'treatments'. This activity threatens the viability of regenerative medicine as a safe and trusted procedure, but at present there is little which can be done to reduce this unethical and unsafe practice. Our only hope at the moment is to provide clear advice about regenerative medicine to potential patients and to increase the amount of stem cell education provided in schools and colleges.

Finally, we must all be aware of, and guided by, the stem cell science which underpins regenerative medicine. It is absolutely essential that any stem cell-based therapy must have a very clear evidence base composed of peer-reviewed publications and completed clinical trials. Such treatments can then be offered to patients with optimised safety and efficacy. Patients who wish to explore possible regenerative medicine procedures which have yet to go through clinical trials are well advised to enrol as volunteers in clinical trials. This will not ensure their absolute safety because all clinical trials carry risk, but those risks are mitigated to minimise any potential harm to volunteers. This is much better than paying profit-motivated businesspeople to receive untested and unsafe 'treatments' which could result in life-changing damage.

Regenerative medicine holds considerable hope for the future but there are many hurdles to be cleared before the technology becomes commonplace in clinics and hospitals. These are scientific, medical, business and ethical hurdles and they cannot be rushed if we are going to provide a safe, effective and trusted regenerative medicine service in the future.

Peter Hollands

Peter trained at Cambridge University under the supervision of the co-inventor of IVF and Nobel Laureate Professor Sir Bob Edwards FRS. His PhD was in stem cell technology with a focus on the transplantation of stem cells from the developing fetus. His post-doctoral position was as a Senior Embryologist at Bourn Hall Clinic which was the first IVF clinic in the world. Peter has been the Scientific Director of Cells for Life in Toronto and Smart Cells in the UK and was HTA Designated Individual for Smart Cells. He has carried out research in stem cell technology and has written numerous papers and book chapters on stem cell technology. He has been an invited speaker to many international conferences including personal invitations to speak twice at the Vatican, the UK House of Lords and The Canadian Parliament. Peter also has experience in creating new stem cell technology laboratories and the related accreditation and regulatory aspects of stem cell laboratories. Peter has been the Group Chief Scientific Officer of the worldwide stem cell services company WideCells Group PLC and a Quality Manager for the Fertility and Gynaecology Academy in London. He now works as a freelance Consultant Clinical Scientist. Peter has written a book on stem cell technology for the general public called 'The Regeneration Promise' which will be published in November 2020. This is the first of a series of books on medical science. Peter was awarded a Visiting Chair in Regenerative Medicine from Kolkata School of Tropical Medicine in November 2017. This was in recognition of his collaborative work in stem cell technology in Kolkata, India.

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