INTRODUCTION

Translation and commercialization of regenerative medicines

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Historically, human healthcare has evolved within two main disciplines—medicine and surgery. Medicine has primarily focused on the use of chemistry to attempt to restore, correct or modify physiology in order to achieve alleviation of symptoms and in some instances (e.g. bacterial infections) cures. By the end of the last century, biological medicinal products had also become increasingly important comprising maybe 10 per cent of all marketed medicines. Surgery, in contrast, is by and large a physical science and employs engineering and material technology to address anatomical defects, abnormalities and trauma by physical repair. These very different modes of treatment have resulted in distinct funding structures, business models, regulatory pathways and reimbursement routes, which have evolved to allow the commercial success of medicines and medical devices.

Regenerative medicine brings together these and other disciplines to create hybrid products for which neither commercial pathways are applicable and brings also additional unique challenges, for example, the logistics of delivering autologous cell products. Regenerative medicine products, unlike most medicines or medical devices, promise cures in many cases; such high expectations of product performance bring similarly high expectations from investors, regulators and healthcare providers. This series of papers explores the various issues that face regenerative medicine as a new multi-disciplinary field.

Various scientific obstacles stand in the way of the successful translation of regenerative medicine: within this Theme Supplement there is good evidence that progress is being made with a number of these. The source of the cellular starting material can greatly impact quality and safety; stem cells appear to offer a panacea, but also pose many safety concerns that have been difficult to address. Guillot and colleagues (Abdulrazzak et al. 2010) discuss the biological characteristics of stem cells from foetal, cord blood and extraembryonic tissues, and conclude that these cells are an intermediate stage between adult stem cells and embryonic stem cells and hence may have important clinical applications. The commercial use of committed stem cells might be expected to pose fewer risks but in addition the research surrounding them adds significantly to the overall understanding of stem cell lineages. Ilic and colleagues’ (Stephenson et al. 2010) important contribution emphasizes the need to fully characterize stem cells prior to banking or expansion. Their proposed new quality standard could aid and accelerate the banking process and facilitate appropriate selection of stem cell lines by end-users.

Identifying the appropriate cell or cell population is only one part of the problem, it is also necessary to provide the right microenvironment and signalling to allow them to repair or regenerate damaged tissue. Brown and colleagues (Kureshi et al. 2010) propose two-dimensional and three-dimensional surface modifications of natural and man-made collagens and other substrates to ensure a resemblance to what happens in vivo. Kelleher & Vacanti’s (2010) impressive contribution deals with potential advances on tissue engineering by applying the latest nanotechnologies to enhance the regenerative response by accurately guiding cells and their differentiation on the acellular device. The commercial possibilities are far-reaching.

The repair, replacement or regeneration of any complex tissue is highly dependent on vascularization, and thus control of vascularization remains a major limitation. Watt et al. (2010) discuss the successes and
hurdles encountered by researchers and clinical practitioners in understanding vascularization of tissue repair or regeneration in their review on endothelial progenitors and their important role in vascularization. Traditional pharmacokinetics and pharmacodynamics are simply not applicable to cell-based products; in their place, there is the need to assess the biodistribution of the cells. Kooreman & Wu (2010) review the recent advances on molecular imaging to track transplanted stem cells, and analyse homing, engraftment and avoidance of teratoma formation. This paper clearly shows that a number of solutions are now available to enable developers to collect critical safety data.

The impact of regenerative medicine is not just as a new discipline, but as a new way of thinking. A good example can be found in the paper by Jamil-Copley & Kanagaratnam (2010) who propose an alternative man-made device to the pharmacological use of warfarin to prevent stroke in patients with atrial fibrillation.

Looking forward to commercial success, Nerem’s (2010) excellent review deals with the potential emergence of regenerative medicine as an industry. His excellent analysis of the current state of the industry concludes there are indications that this, and related technology, may still achieve its potential and address the needs of millions of patients worldwide, in particular, those with needs that currently are unmet. Combined with Polak’s (2010) comprehensive summary of the latest achievements, both in basic science and in translational research, this paints a truly optimistic view for regenerative medicine.

Prescott (2010) proposes that the overall objective of regenerative medicine is not just the delivery of the product into the clinic but also to patients on a routine basis. Such a goal typically requires a commercial vehicle and substantial levels of investment in scientific, clinical, regulatory and business expertise, resources, time and funding. Regulation is a necessary hurdle, both to safeguard public health but also to provide credibility to the industry. Bravery’s (2010) contribution addresses the regulatory mythology that is hindering translation, not just for product developers but also because these uncertainties deter investment. He concludes that the regulatory pathways are clear but that the data requirements are not, owing to poor understanding of the underlying science that can only be resolved through basic research.

In conclusion, this Theme Supplement highlights that good focused science is progressing the field and moving it forward to the point of commercial realization.

REFERENCES


