Cell and gene therapy - worth the wait?

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How best can we translate promising inventions in the lab into clinically-meaningful therapeutics for the benefit of our patients? In 2015, genetic and cellular therapies have reached a confusing crossroad. Since 1989 over 1500 Phase I/II studies of direct in vivo and cell-mediated gene therapy in diverse diseases have been completed (1). Substantial evidence of improved clinical outcomes has been shown in haemophilia B, immune deficiencies, haemoglobinopathies, immuno therapi es and blindness. In the field of cellular therapeutics, applications have expanded beyond their foundation in autologous and allogeneic hematopoietic cell transplantation to mesenchymal and other adult cell therapy trials. If pluripotent cells can be differentiated ex vivo to recreate and repair mature human tissues and organs then regenerative medicine will become a reality. However embryonic stem cells have been mired in controversy and clinical development has been forestalled (2). Indeed the spectacular fall of the Japanese scientist who claimed to have induced pluripotency in regular body cells using mild acid is not uncommon in the scientific community (3). Medical and, in particular, stem cell tourism has become a billion dollar industry with increasing examples of false claims. Unregulated, untested or unsafe stem cell ‘therapies’ place the field at a challenging crossroad.


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