

Special edition of the SAMJ devoted to cell and gene therapy

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Special edition of the SAMJ devoted to cell and gene therapy

Imagine a world in which disease management is highly personalised. Drug treatments would be specific to the individual concerned, taking into account genetic background and environmental factors. Therapies would be effective, tailored to the individual's needs, with few or no side-effects. Given that at present many of our treatments are based on a one-size-fits-all approach, which clearly has its limitations, personalised medicine is an important ideal to pursue. The highly publicised notion of precision medicine on the other hand aims to identify differences at a population level, guiding the application recent technological advances.

Hematopoietic stem cell transplantation (HSCT) is universally applied and has been performed successfully for several decades. Several new therapies have recently been introduced into the clinical arena and many more are on the horizon. Two areas which have received a great deal of media attention are chimeric antigen receptor T cells (CART cells) and genome editing. The highly promising results obtained with CART cells and the implications for South African patients have recently been reviewed in the *SAMJ* by Pepper *et al.*^[1] The term genome engineering encompasses a variety of techniques which result in alterations of the genome. This can be used for research or therapeutic purposes. When applied for the latter, it falls within the ambit of gene therapy. This technique was recently applied to children born to an HIV-positive father in China. The aim was to render the children resistant to HIV. Many ethical and scientific questions were raised following the birth of these babies, culminating in a call by prominent scientists and several organisations to place a moratorium on genome editing involving gametes (egg and sperm).

In this special issue of the *SAMJ*, the authors have reviewed current and imminent cell and gene therapies. Grobbelaar *et al.*^[2] review HSCT in South Africa (SA) and highlight limitations to its effective and equitable access. Mellet *et al.*^[3] review human leukocyte antigen (HLA) diversity and its clinical applications in SA, including those related to HSCT, while Wolmarans *et al.*^[4] discuss the heterogeneity of cell-therapy products and the implications thereof. One of the areas in which important progress has been made with gene therapy is primary immune deficiency (PID); Erjaee *et al.*^[5] review PID and its treatment on the African continent. Ely *et al.*^[6] review the current status of gene and cell therapy in SA, while Thomson *et al.* provide a perspective on the South African National Blood Service's view on cellular therapeutic services and products. Exciting new developments in the field of gene-modified hematopoietic stem cells (HSCs) are reviewed by Alessandrini *et al.*^[7] while Naidoo *et al.*^[8] provide an overview of therapeutic genome modification using neurodegenerative and infectious diseases as examples. Fanucci *et al.*^[9] discuss bioprinting and its implications for the SA clinical environment. Attention is given to specific organ systems by Alessandrini *et al.*^[10] who discuss the current status of stem cell therapy for neurological disorders, while Niesler *et al.*^[11] review cellular regenerative therapy for acquired non-congenital musculoskeletal disorders. The high prevalence of HIV in South Africa merits a discussion on the effect of the virus on haematopoiesis and in particular on HSCs,

the quintessential example of adult stem cells; Durandt *et al.*^[12] have addressed these issues.

Finally, Skeen *et al.*^[13] report on an original study conducted in SA on people living with spinal cord injuries who received 'stem cell therapy' for their lesions. This study highlights the dark side of stem cell therapy, namely stem cell tourism, in which emotionally vulnerable patients and their families are exploited, both emotionally and financially, and which runs the risk of discrediting the cell and gene therapy fields.

An exciting future awaits us as cell and gene therapies come to the fore. Impressive results have been obtained with CART cells and haematological malignancies, and with gene therapy for genetic disorders (haemophilia, PID). Gene therapy for diseases that are highly prevalent on the African continent are imminent: infectious diseases (HIV) and haemoglobinopathies (sickle cell disease and thalassaemia) being good examples. It is hoped that this special edition will provide readers of the *SAMJ* with insight into these rapidly evolving fields and the implications for our patients.

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