The assessment and appraisal of regenerative medicines and cell therapy products: an exploration of methods for review, economic evaluation and appraisal

Robert Hettle,[1] Mark Corbett,[2] Sebastian Hinde,[1] Robert Hodgson,[2] Julie Jones-Diette,[2] Nerys Woolacott[2] and Stephen Palmer[1]*

[1]Centre for Health Economics, University of York, York, UK
[2]Centre for Reviews and Dissemination, University of York, York, UK

*Corresponding author

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Plain English summary

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Regenerative medicines replace or regenerate human cells, tissues or organs to restore or establish normal function. Potential breakthroughs are eagerly anticipated and expectations are high because of the possibility of cures (or substantial improvements) for diseases that are deemed chronic or fatal. Regulatory pathways are evolving to facilitate the early approval of such promising new therapies. However, the assessment of the long-term costs and benefits of such therapies is more difficult than for conventional treatments.

In response to an inquiry by the House of Lords into regenerative medicines, an expert group (Regenerative Medicine Expert Group or RMEG) developed an action plan for the NHS. The RMEG proposed that the National Institute for Health and Care Excellence (NICE) commission a 'mock technology appraisal' to assess whether changes to its methods and processes are needed.

This report presents the findings of independent research commissioned to inform this appraisal. We reviewed evaluations of regenerative medicines by NICE and other groups as well as conducting reviews of the existing literature concerned with the challenges of assessing the therapies. In addition, an exemplar case study of chimeric antigen receptor (CAR) T-cells for acute leukaemia was constructed to inform the deliberations of an expert panel set up by NICE.

Our research found that, although evidence about regenerative medicines is expected to be associated with much uncertainty in determining the long-term costs and benefits to patients and the NHS, the existing methods available to estimate the implications of this uncertainty are sufficient. Ways of sharing the risks between the NHS and the therapy manufacturers should be investigated further.

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Editorial contact: nihredit@southampton.ac.uk

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This report

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