National Institute for Health and Care Excellence (NICE)

Response to Consultation on IMI Advanced Therapies Concept Paper

General Comments

1. NICE welcomes the IMI concept paper "facilitating the translation of advanced therapies to patients in Europe" and considers that the IMI platform has considerable potential for enhancing ATMP research and development.

2. The key challenges identified are all considered relevant and in the areas of most interest to NICE (the clinical development and pricing, reimbursement and access parts of the concept paper), also comprehensive.

3. NICE has been active in planning for the expected need to evaluate increased numbers of ATMPs. In particular, in collaboration with the University of York, a study exploring the assessment and appraisal of regenerative medicine and cell therapy products was undertaken. We consider this work to be relevant to several areas considered in the concept paper. Two reports were published on the NICE website in March 2016 – a detailed technical report produced by the University of York and a short overview report produced by NICE (https://www.nice.org.uk/about/what-we-do/science-policy-research/nice-research). Within the next 6 months or so, a final, peer reviewed version of the York report will be published as part of the HTA Monograph Series (http://www.journalslibrary.nihr.ac.uk/hta).

4. In addition, NICE Scientific Advice (a consultancy service from NICE to help life sciences companies integrate HTA considerations in clinical development plans) has undertaken advice projects for ATMP companies and developed an educational seminar focused on gene and cell therapies. We consider that scientific advice, covering both regulatory and HTA issues to be very important in supporting translation of advanced therapies to patients.

Comments relating to specific parts of the concept paper

5. The final paragraph of section 1 indicates that due to complex and challenging value propositions for ATMPs, the evaluation of such treatments requires a paradigm shift in the manner in which such evaluations are performed. The NICE / York study outlined in 3 above, concluded that the NICE appraisal methods and decision framework are applicable to regenerative medicines and cell therapies. The study also highlighted several challenges associated with these products where methodological development is needed. We fully support encouraging more research in the pharmaco-economic evaluation of ATMPs but it is important not to prematurely conclude that current frameworks are inappropriate for these products.

6. Paragraph 2 of section 3.2 includes highlighting the importance of regulatory consultation. We consider that this should be expanded to highlight the importance of scientific advice covering regulatory and HTA issues. By considering HTA and payer perspectives at an early stage, there is an increased opportunity for efficient clinical development that generates the key evidence required to support all aspects of the pathway to patient access.

7. Paragraphs 5 and 6 consider the importance of assessing the value of various data bases and we fully support this. There may be synergies with the existing IMI GetReal project and upcoming IMI big data for better outcomes (BD4BO) projects.

8. The table in section 3.4 is a very useful summary of the considerations and challenges associated with evaluating ATMPs. Several of these issues are considered in the University of York report highlighted in 3 above. There is, in particular, comprehensive consideration of the implications of single arm studies and strategies to minimise bias in the estimation of outcomes.

9. Based on our experience from the NICE / University of York study, we support the proposed priorities for future research investments. Alignment of regulatory and reimbursement pathways is very important and the learning from the EMA Adaptive Pathways pilots and IMI ADAPT-SMART project should help identify ATMP specific areas for further research. We particularly support the proposal to prioritise research investments in the area of innovative reimbursement and payment mechanisms as our work highlighted this as key to timely patient access to potentially transformational therapies.

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