Regenerative Medicine in the UK Health System

Final Conference of the ESRC REGenableMED project

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https://www.york.ac.uk/satsu/current-projects/regenablemed/

Summary report on the Conference

The Conference reported the results and insights of the ESRC-funded REGenableMED project. Complemented by two panels – on international developments and on future strategy - a series of strategic issues were identified. The contribution from the delegates was especially important in helping to define these issues more precisely. The summary below is of direct relevance to current government policy and the recent Select Committee report. The principal ideas that should shape future planning are:

A novel innovation infrastructure for RM: Providing the right sort of organisational and scientific infrastructure for RM is vital. There needs to be a paradigm-shift away from a traditional approach to product development to one that focuses on a more co-ordinated value-chain, on targeted (rather than broad) population groups, patient access and on a broader range of evidentiary sources to track and assess emergent therapies. 'Transformative' products are in fact transformative *processes* in the RM field, which then need a transformative framework to support them.

As part of this process, further thought should be given to establishing a formal RM network at the national level to help coordinate infrastructural development building on recent policy. Moreover, this should foster better professional networking/communities of practice in the wider NHS, especially in regard to encouraging developmental learning within clinical practice. The envisaged Advanced Therapy Treatment Centres (see below) would operate much more effectively in such an environment.

Advanced Therapy Treatment Centres – building effective institutional and business models: The proposed ATTCs to be supported by Innovate UK are in effect 'pilots' of central importance to the development of the field (in the language of the 'trial', they are organisationally at 'Phase 1' in the process). The centres need to reflect an *institutional readiness* for RM therapies, specific clinical competences, a role for relevant patient associations in providing patient input, and an alignment between the new ATTCs and

existing rare-disease treatment centres. There is a role for the RM Clinical Reference Group informed by the social science from the project in regard to defining the scope and specification for possible centres. Centres need to ensure that they create additional knowledge and skills and leverage existing infrastructure (such as the NHSBT/ SNBTS and the NIHR Biomedical Research Centres), while being geographically accessible within the UK.

Five paradigmatic pathways to innovation have been identified in the project, which in turn have distinctive clinical adoption parameters. These need to be linked to the six key and distinctive business models that have also been identified through the project's detailed analysis of SMEs in the field.

Integration of clinical databases: Relevant biobanks, clinical registers and databases need to be more strongly integrated, especially in embracing real world data systems (i.e. 'off-trial' data); standardising data across these different sources should become a priority, and are key to moves towards proposed risk-sharing schemes (or more formally outcomes-based managed entry agreements).

Reimbursement and regulatory models: There are a number of reimbursement and regulatory models that do not necessarily map on to the specific market niche for RM. Risk-sharing commissioning schemes will also need coordinated data-collection infrastructures to support their iterative approach, and to resolve the tension between a 'values' and a 'needs' driven reimbursement and regulatory perspective. An annuity cap model is especially worth exploring within this context to help manage problems of affordability in the health care system, and foster innovation, within the recently adopted net budget impact test (that innovative therapies do not exceed an overall budgetary impact of £20m over three years).

Understanding international developments: There are diverse regulatory models internationally. The conference discussed presentations related to recent developments in Europe, China and Japan. Developments relating to iPSCs in Europe have had a significant impact in the entry of major pharmaceutical companies to the area while in China it is clear that there are tensions between national regulation and regional regulatory 'bionetworks'. In Japan, the relatively recent (2014) regulatory changes to foster early access for patients reflects Japan's desire to play a leading role internationally in the RM field.

Further information can be found at the project website, especially in regard to the 4 Policy Briefings that are available:

https://www.york.ac.uk/satsu/current-projects/regenablemed/newsandbriefings/#tab-5

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