REGENERATIVE MEDICINE IN THE UK HEALTH SYSTEM

INTRODUCTION TO THE PROJECT

Andrew Webster
PI REGenableMED project
Director, Science & Technology Studies Unit (SATSU), University of York

Since start of project in January 2014 see an increasingly complex RM/health system

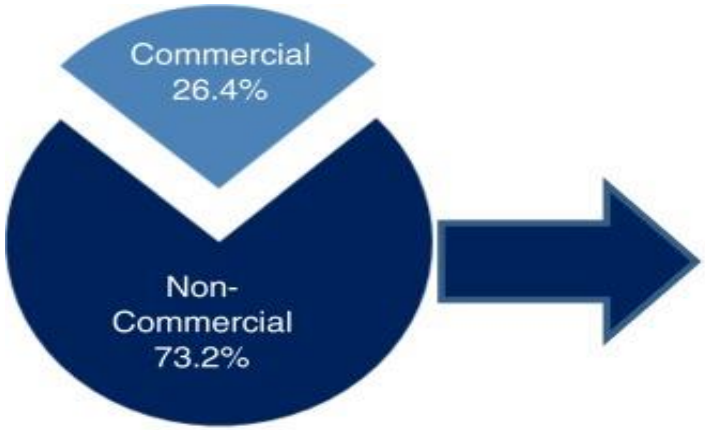
1. Some key S&T developments
CRISPR and CAR T-cells – rapid increase in scientific/commercial activity
Major increase in iPSC activity
Release of UKSCB clinical grade lines
8 ATMPs approved – but with limited success
Moves to merge health informatics and cell production – new bio-platforms
Recent CTs of pluripotent lines – 30 now globally
Responding to immunogenicity: towards an iPSC haplobank
Regenerative therapy to restore function paralleled by move towards preventing *de*generative structures in first place

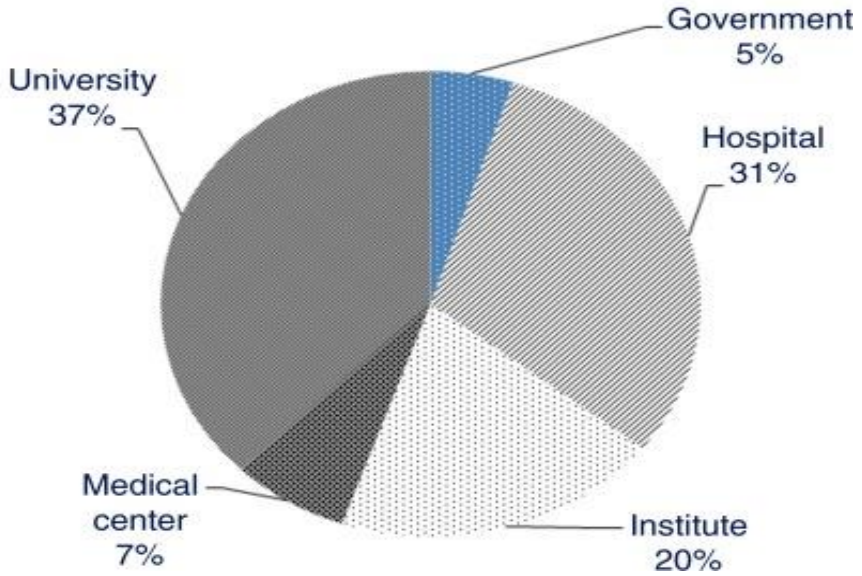
2. Some key organisational/policy developments
C & *Gene* Therapy Catapult
Regulatory change in Japan (Nov 2014) and 'REGROW' lobby in USA
Development of iPSC STEMBANCC (1500 lines)
RegMed Expert Group proposals
AT Manufacturing Task Force
House of Commons RM Inquiry 2017
Review by DH of its Innovation Landscape and the (separate) AAR
UKRI and the Industrial Strategy Challenge Fund (place for RM within 'leading edge and healthcare' priorities
Brexit – possibilities for re-localising some regulatory aspects (eg MHRA/HTA processes undertaken in parallel?)

So we see co-evolving technologies and organisational and policy networks – an emergent infrastructure for RM?

Regenerative Medicine worldwide*

- Identification of 939 clinical trials investigating ATMPs (85% ongoing, 15% completed).
- Majority in the early stages (Phase I, I/II: 64.3%, Phase II, II/III: 27.9%, Phase 3: 6.9%).
- Disease areas: cancer (24.8%), cardiovascular diseases (19.4%), musculoskeletal (10.5%), immune system and inflammation (11.5%), neurology (9.1%), and others.



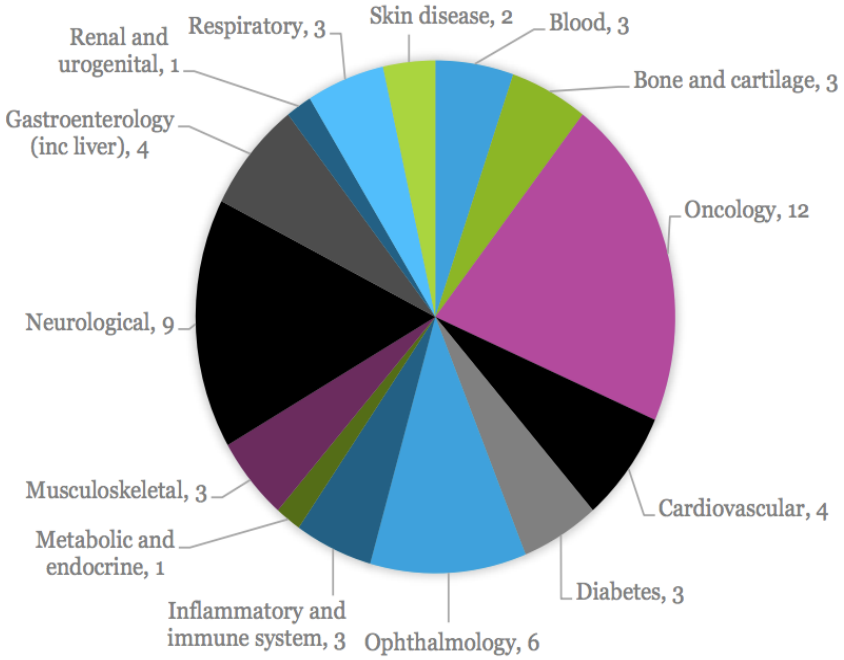


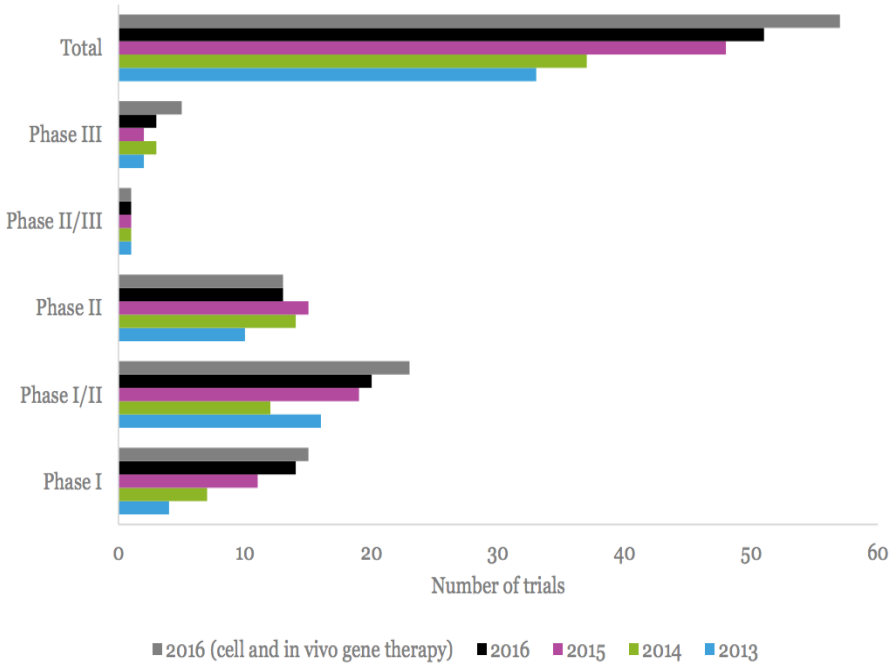
** E. Hanna, C. Rémuzat, P. Auquier, M. Toumi, Advanced therapy medicinal products: current and future perspectives, J Mark Access Health Policy. 2016; 4: 10.3402/jmahp.v4.31036. Published online 2016 Apr 25.*

REGenableMED

Regenerative Medicine in the UK

- 42 developers of ATMPs
- 60 clinical trials in the UK (2016)





REGenableMED

Broad theoretical approach of the project: understanding innovation as socio-technical process

Innovation and the Scrabble metaphor: ‘ A market is like a Scrabble board: there is no point in wanting to place an innovation that does not correspond to the possibilities it affords…no point in keeping aside the perfect word’ (Callon TCS, 2007)

Distributed, interactive (non-linear) model of innovation (see Hyssalo et al *The New Production Of Users,* 2016).

From working to *workable* – context as *process* not pre-given *condition* (see Carl May, *Implementation Science*, 2016)

REGenableMED

What the REGenableMED project has examined since May 2014:

Close engagement with developments in the science-base
Mapped wide range of stakeholders and their role using horizon-scanning techniques
Developed a detailed analysis of the regulatory and legal aspects of RM – including comparative work
Detailed exploration of SMEs in the field and their diverse business models and reimbursement using value-chain analysis
Manufacturing models (scale-up vs scale-out)
Patient associations and their perspective on RM
Pathways through to clinical adoption and how these can be differentiated
Detailed examination of the clinical trial process
Wider developments in the health system – especially innovation policy and relationship between different networks

Selected key findings relating to RM in the health system

- there are specific innovation challenges of a technical, regulatory and manufacturing nature with regard to deriving, stabilising, classifying and scaling-up live (cell) tissue for clinical purposes, not found in other areas of biomedical innovation
- through the detailed case studies and wider fieldwork we have identified five paradigmatic routes to the clinic being pursued by clinical and commercial actors and mapped these onto the leading firms in the field within the UK and elsewhere
- clinical therapies are and will be shaped by the interplay of 'technology identities, adoption and development spaces' that generate diverse contexts within which implementation is more, or less, likely: these can help model the actual and prospective take-up of therapies in the future
- RM will require a range of novel or redistributed manufacturing micro-factories close to clinics to be viable
- as a result, scenarios need to be developed for the likely size and profiles of clinical populations treatable through different manufacturing modalities and scales to support national planning in the NHS

…eg, second one →

Project has identified five innovation pathways

Five different pathways:

1. *Enabling, gateway innovation such as immunotherapy: e.g.* gene-modified CAR T-Cells for leukaemia [Oxford Biomedica]
2. *Automated cell processing 'point-of-care' device/technique*: e.g. the 'Celution System' [Cytori - Deeside]
3. *Surgeon-led innovation* – e.g. the bioengineered trachea [Videregen/UCL]
4. *Implantation/infusion therapy innovation:* e.g. wound/skin repair (which would not occur naturally) [Tissue Regenix - Leeds]
5. *Bioprocessing innovation* - e.g. expertise and services to other parties [Cellular Therapeutics - Manchester]

These are **paradigmatic of the innovation pathways** within regenerative medicine.

They have different *innovation* and so *adoption* profiles

Some key outputs

Policy Briefings – see composite in programme pack

Papers – c20 and rising (see list in programme pack)

Evidence to HoC Inquiry which has been drawn on directly

Responses to over 40 national and international formal Consultations (especially with the EMA)

Major database of 700+ organisations – open access to this in July 2017

Collaboration with C&GTC, OECD, MHRA, BIS, ATMTF, RMEG, EuroStemCell, UKSCB

Key ‘socio-technology’ messages to take away

1. Moves towards C&GT Treatment Centres:
- Need for an effective pilot – ‘implementation laboratories’
  Need to decide what outcomes can be expected
- How to build on existing system (eg NHSBT) learn from other areas (eg AHSNs’ Test beds) and build new data systems

2. Accelerate responsibly : avoid danger of moving rapidly to solutions without carefully knowing what the problem is and what success looks like, and what we need to do to adapt if things go wrong – move rapidly but outstrip our capacity to deliver innovation

3. Need to align ‘technological readiness’ with ‘institutional readiness’ – from working to workable innovation and link ‘innovation’ with ‘improvement’ strategies/policies

The REGenableMED Project

A social science analysis of Regenerative Medicine

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

York: Andrew Webster, Ruchi Higham, John Gardner (now at Monash)

Sussex: Alex Faulkner, Aurélie Mahalatchimy

Edinburgh: Joyce Tait, James Mittra, Geoff Banda

Birmingham: Sue Simpson, Sandhya Duggal

ESRC Grant Ref: ES/L002779/1

REGenableMED

E·S·R·C ECONOMIC & SOCIAL RESEARCH COUNCIL