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Mapping a route for the commercialisation of regenerative medicine;

The House of Lords Science and Technology Committee's
Regenerative Medicine Inquiry.

Lord Krebs, Chairman of the House of Lords Science and Technology Committee

Tuesday 21 May 2013

Purpose of the inquiry

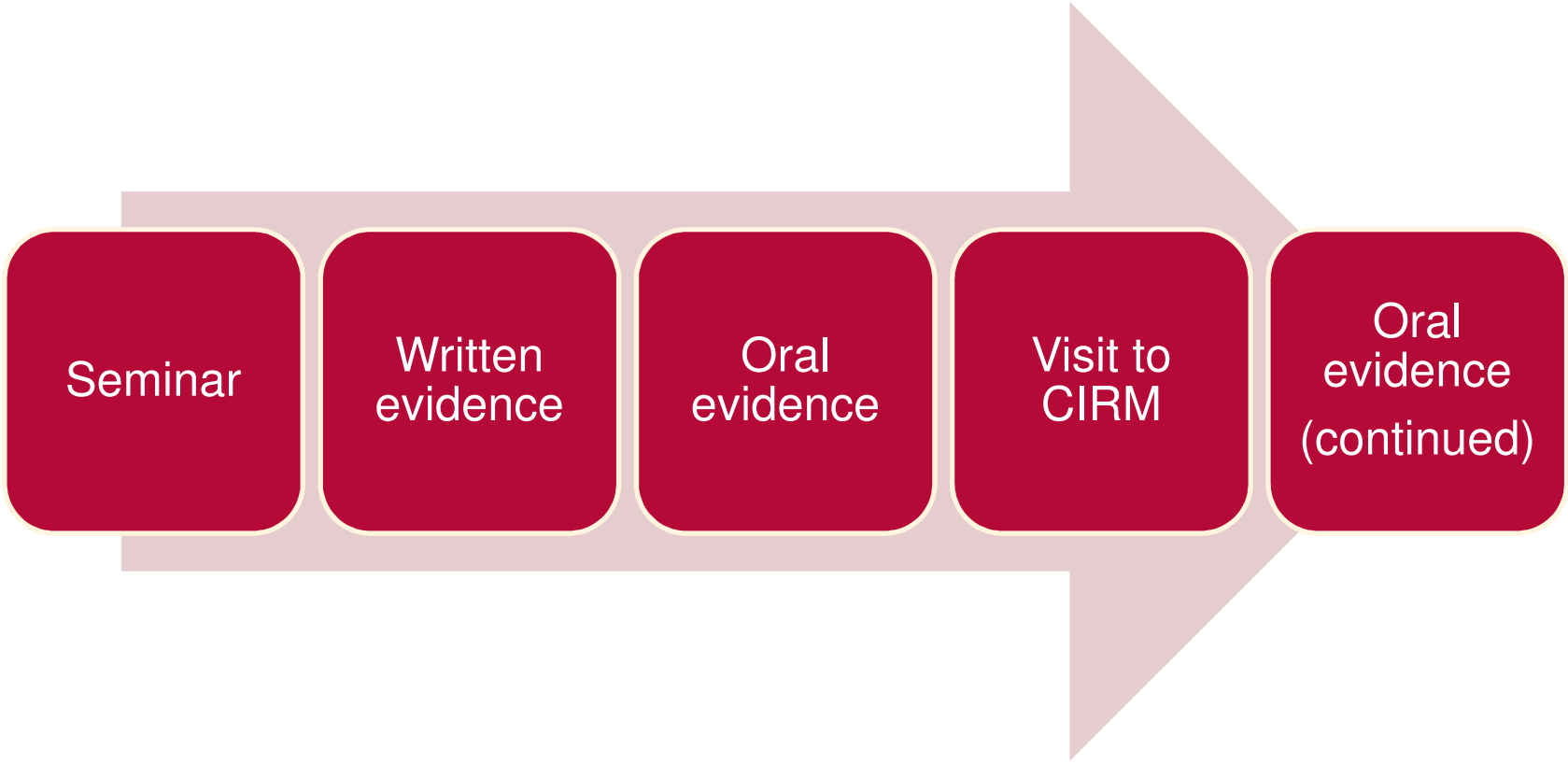
- UK strengths in regenerative medicine
- Translation to clinic likely in the next 5-10 years
- Identify and recommend solutions to barriers to translation and commercialisation (including regulation, finance, infrastructure); to make recommendations to Government
- Excluded ethical considerations from scope

Definitions

- Regenerative medicine: “methods to replace or regenerate human cells, tissues or organs in order to establish normal function”.

- This definition could include: cell therapies, tissue engineering, gene therapy, biomedical engineering, pharmaceuticals, devices.

Our process



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Examples

- What's available now?
  - Pancreatic islet transplantation; bone marrow transplant; skin grafts.

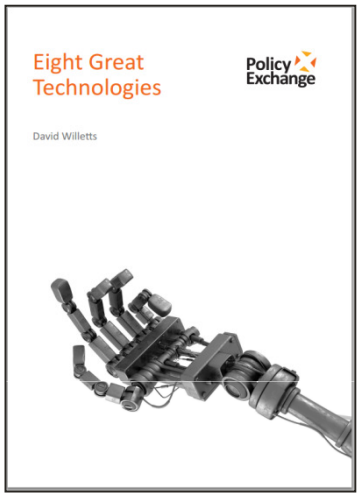
- What might be available in the next 5-7 years?
  - Treatments for: ARMD; MS; and stroke.

- What potential treatments could we see in the long term?
  - Parkinson's disease, cardiovascular disease and diabetes

Value of regenerative medicine

- 15 million people in the UK are affected by chronic disease
- Median 5 year increase 14%
- Quality of life considerations
- Economic benefits:
  - Return to work
  - Unsustainable costs to the NHS

Government initiatives



- Taking stock of Regenerative Medicine (2011)
- Life science strategy (2011)
- Strategy for Regenerative Medicine (2012)
- One of eight priority technologies (2013)

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Emerging findings 1: the landscape

- Areas of strength in UK science base include stem cells, tissue engineering, gene therapy
- UK has second highest number of ATMP trials in Europe (2004-10)
- Second highest number of SMEs in RM in Europe

Emerging findings 1: the landscape

- £72 m/year public sector investment
- £12m/yr for five years for the Catapult
- £13m/yr from AMRC charities (and growing)

Emerging findings 2: regulation

- Up to nine EU/UK regulators are involved; complex environment
- MHRA guidance is third most expensive in Europe
- HRA may help to streamline process

Emerging findings 3: clinical trials and scale-up

- NHS should make the UK a favourable place for clinical trials
- But causes for concern include: delays; lack of adequate support; design and scale
- The NIHR is providing some support

Emerging findings 3: clinical trials and scale-up

- Is there capacity to produce RM products at scale for clinical use?

- Is the infrastructure for delivery to the patient adequate?

Emerging findings 4: commercialisation

- Business model not well established
- High level of risk and uncertainty a disincentive to private investors
- The Catapult
- Know-how may be as important as patents
- ECJ ruling in 2011 is not a barrier to commercialisation
- An appropriate model for reimbursement and pricing

Emerging findings 5: international

- Harmonisation of regulatory requirements would be desirable
- Risks of regenerative medicine tourism
- Lessons from CIRM; innovative finance models such as French Citizens innovation bond

Next steps

- June 2013: Report due to be published
- Autumn 2013: Government response expected
- 2013-2014: Debate in House and possible follow-up by Committee

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