Translating complex surgical ATMP: an academic steeplechase

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Paris, 18th May, 2017
An advanced therapy medicinal product (ATMP) is a medicinal product which is either:

- a gene therapy medicinal product
- a somatic cell therapy medicinal product
- a tissue engineered product

The definition of ATMPs is found in Directive 2001/83/EC as amended by the ATMP Regulation 1394/2007 and includes combination ATMPs.
Regenerative medicine is a branch of translational research in tissue engineering and molecular biology which deals with the "process of replacing, engineering or regenerating human cells, tissues or organs to restore or establish normal function".

Mason, 2010
Translational pathway: From bench to bedside to population impact

Academic Health Science Centre

Discovery science
- World-class Life Science research base
- The Francis Crick Institute

Experimental medicine
- 3 BRCs and 2 BRUs
- Clinical Research Facilities
- Therapeutic Innovation Capabilities
- Industry engagement

Clinical Trials
- CLRN
- Clinical Trials Units
- Quintiles Prime site
- UCL Institute of Clinical Trials & Methodology

Implementation

Population health

Reverse translation

Wolfson Institute of Preventive Medicine

LIFE STUDY

NIHR CLAHRC
North Thames
Approaching the hurdles

• Great team
• Institutional (various) support
• Communication with stakeholders: especially funders and regulators
Gene Rx conceptualised

?first patient (unpublished)

One patient temp success

1st permanent genetic change

Successful I/II trials

1st US/EU license (Glybera)

$600m investment p.a.

>12 clinically approved products

Tissue Engineering Today

Failure

Tissue Engineering Tomorrow
ACCOMPAGNER L'INNOVATION AU SERVICE DE TOUS LES PATIENTS

LES JOURNÉES DE L'AGENCE DE LA BIOMÉDECINE

Centre universitaire des Saints-Pères
PARIS René Descartes

18 & 19 MAI 2017