Bringing gene and cell therapies from lab to patients: the EuroGCT research pathways

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Faculté de Droit et de Science Politique Aix*Marseille Université

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EURO GCT European Consortium for Communicating Gene & Cell Therapy Information

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INTRODUCTION & AIM

Bringing gene and cell therapies from lab to patients raises challenges from the development of these therapies to patient access. Therapy development must comply with many regulatory requirements in order to ensure patient safety. These requirements have been established throughout the life cycle of these therapies from fundamental research to post-administration vigilance, through manufacturing, clinical trials, marketing authorisation, patentability, or pricing & reimbursement.

The **EuroGCT** website aims to provide accessible and reliable information on therapy development and regulatory pathways.

METHODOLOGY

- Legal and scientific watch
- Collection and organisation of ATMPs legal acts and guidance
- Dissemination of information to EuroGCT partners
- Review of research pathways main entries drafts by partners & external collaborators

GENERAL CHALLENGES RAISED & RESEARCH PATHWAYS TREE

Temporality

Variety of Actors

Legal Complexity

- Binding texts vs. guidelines
- European Union law vs. national laws
- Legal classification leading to different pathways

1 *Research & Innovation*

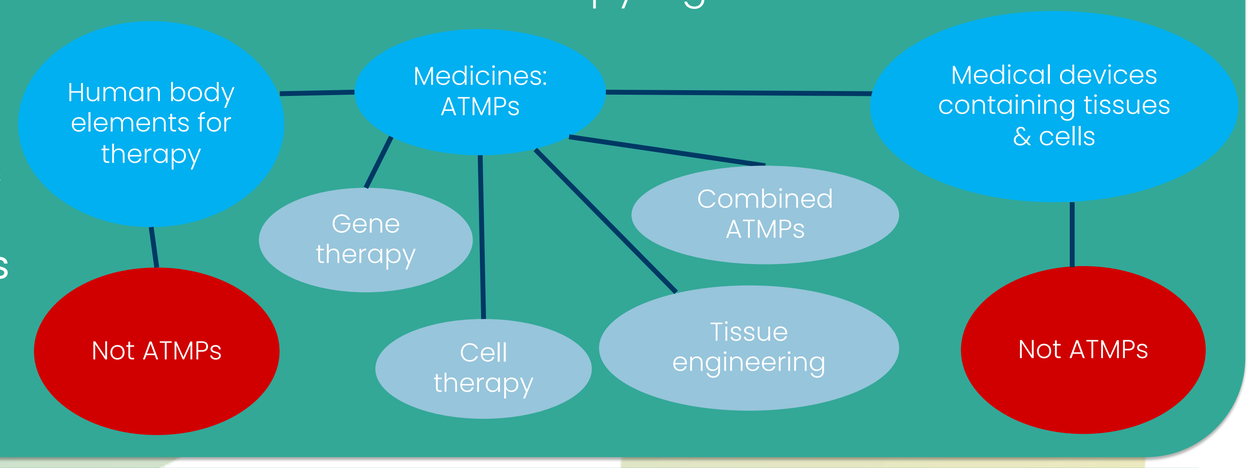
Translating discoveries from basic research

- *Fundamental research*
- *Clinical research*
- *Funding*
- *Incentives*
- *Early interactions with regulators*

2 *Therapy Classification*

Determining legal mechanisms for gene & cell therapies

Gene & cell therapy legal classification



3 *Manufacturing*

Overview of ATMPs manufacturing processes

- *Manufacturing Authorisation*
- *Good Manufacturing Practice*
- *Investigational ATMP Dossier*
- *Capability*
- *Scalability*
- *Packaging & Labelling*

4 *Commercialisation*

Enabling timely patient access to ATMPs

- *Market access for ATMPs*
  - *Standard & expediting Marketing Authorisation*
- *Good Distribution Practice*
- *Good Pharmacovigilance Practice*
- *Pricing & Reimbursement*

5 *Actors & Networks*

Stakeholders mapping in Europe and beyond

6 *Public Involvement & Data*

Best practices for public involvement & health data handling

CONCLUSION

Research Pathways content should be written in lay language and regularly updated to provide reliable and accessible information. Key content will be translated and available in seven European languages: English, French, German, Italian, Polish, Spanish, and Portuguese.

CONTRIBUTE

Contribution and participation is warmly welcome!

Join the EuroGCT ELSI Network of Expertise:

- To provide drafts content of entries
- To provide lay summaries of your research papers
- To translate existing entries
- To review draft entries
- To provide useful updated resources

All contributions will be credited on the EuroGCT website: www.eurogct.org

To get involved in this collaborative work. please contact the EuroGCT Information officers on ELSI

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