



HAL
open science

Finding out the regulatory requirements for the development of Gene and Cell therapy products with the EuroGCT research pathways

Auxane Delage, Luc-Sylvain Gilbert, Hsin-Yu Kuo, Jennifer Lorigan, Clare Blackburn, Anna Couturier, Aurélie Mahalatchimy

► To cite this version:

Auxane Delage, Luc-Sylvain Gilbert, Hsin-Yu Kuo, Jennifer Lorigan, Clare Blackburn, et al.. Finding out the regulatory requirements for the development of Gene and Cell therapy products with the EuroGCT research pathways. European Society for Blood and Marrow Transplant 49th annual meeting, Apr 2023, Paris, France. halshs-04290303

HAL Id: halshs-04290303

<https://shs.hal.science/halshs-04290303>

Submitted on 16 Nov 2023

HAL is a multi-disciplinary open access archive for the deposit and dissemination of scientific research documents, whether they are published or not. The documents may come from teaching and research institutions in France or abroad, or from public or private research centers.

L'archive ouverte pluridisciplinaire **HAL**, est destinée au dépôt et à la diffusion de documents scientifiques de niveau recherche, publiés ou non, émanant des établissements d'enseignement et de recherche français ou étrangers, des laboratoires publics ou privés.

Illustrating regulatory requirements for the development of Gene and Cell therapy products with the EuroGCT Research Pathways

Auxane Delage¹, Luc-Sylvain Gilbert², Dr. Hsin-Yu Kuo³, Jennifer Lorigan⁴, Dr. Clare Blackburn⁵, Dr. Anna Couturier⁶, Aurélie Mahalatchimy⁸

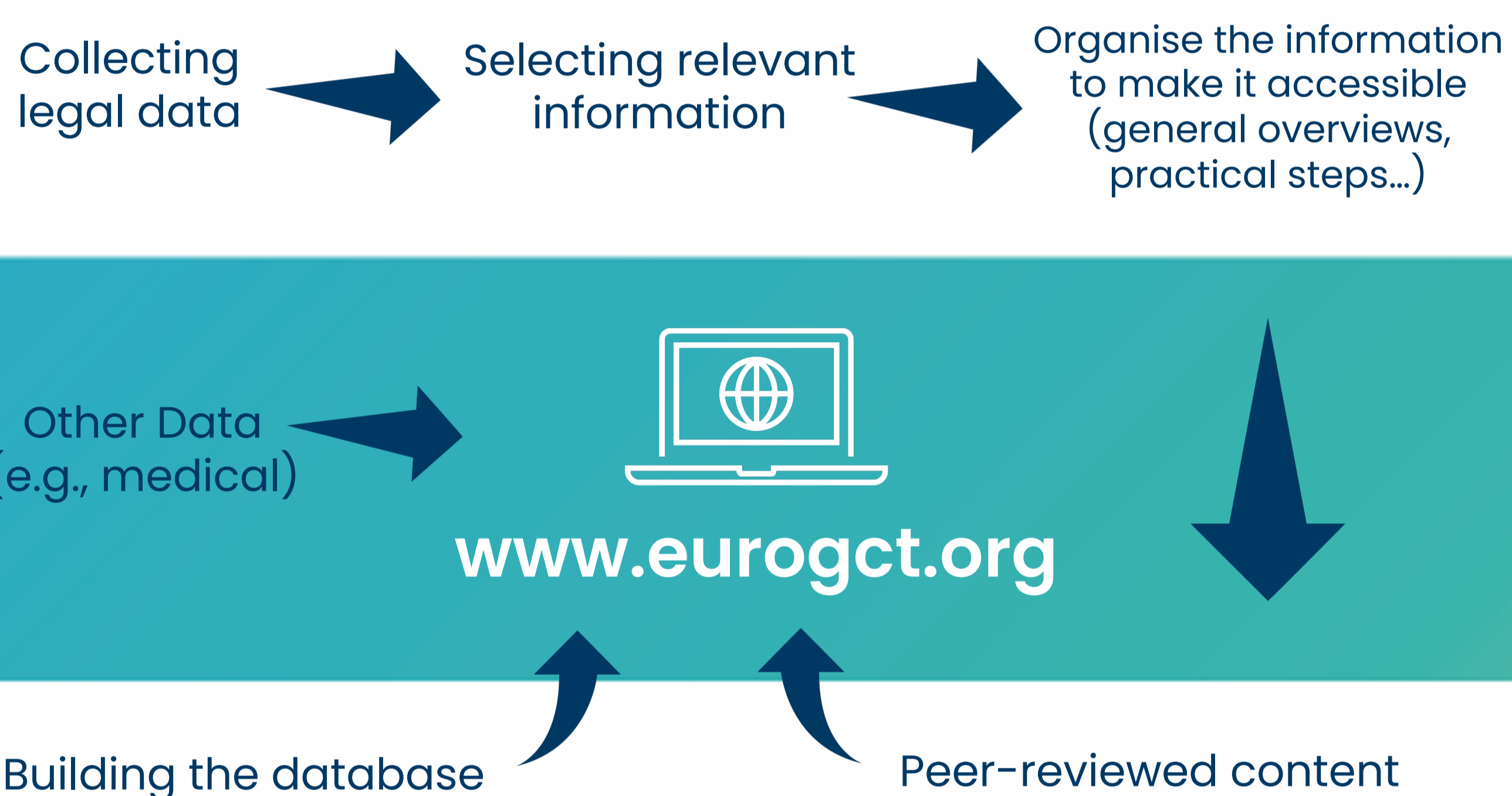
¹EuroGCT Information Officer on Ethical, Legal & Societal issues (ELSI), Research Engineer and PhD student in law, Law Faculty, UMR 7318 International, Comparative and European laws, UMR 7318 DICE CERIC, CNRS, Aix Marseille Univ., Toulon Univ., Aix-en-Provence, France; ²EuroGCT Information Officer on ELSI, Research Engineer in law, Law Faculty, UMR 7318 International, Comparative and European laws, UMR 7318 DICE CERIC, CNRS, Aix Marseille Univ., Toulon Univ., Aix-en-Provence, France; ³EuroGCT Information Officer on ATMP Product Development Pathway, Centre for Regenerative Medicine, Institute for Regeneration and Repair, University of Edinburgh, UK; ⁴EuroGCT Information Officer on Gene and Cell Therapy, Centre for Regenerative Medicine, Institute for Regeneration and Repair, University of Edinburgh, UK; ⁵EuroGCT Project Coordinator, Centre for Regenerative Medicine, Institute for Regeneration and Repair, University of Edinburgh, UK; ⁶EuroGCT Project Manager, Centre for Regenerative Medicine, Institute for Regeneration and Repair, University of Edinburgh, UK; ⁷EuroGCT Deputy coordinator and WP4 leader, Permanent Researcher in law at the French National Centre for Scientific Research (CNRS, CR), Law Faculty, UMR 7318 International, Comparative and European laws, UMR 7318 DICE CERIC, CNRS, Aix Marseille Univ., Toulon Univ., Aix-en-Provence, France. Acknowledgement: This work has been supported by Horizon 2020 funded EuroGCT project (GA N° 965241, PI: C. Blackburn).

BACKGROUND

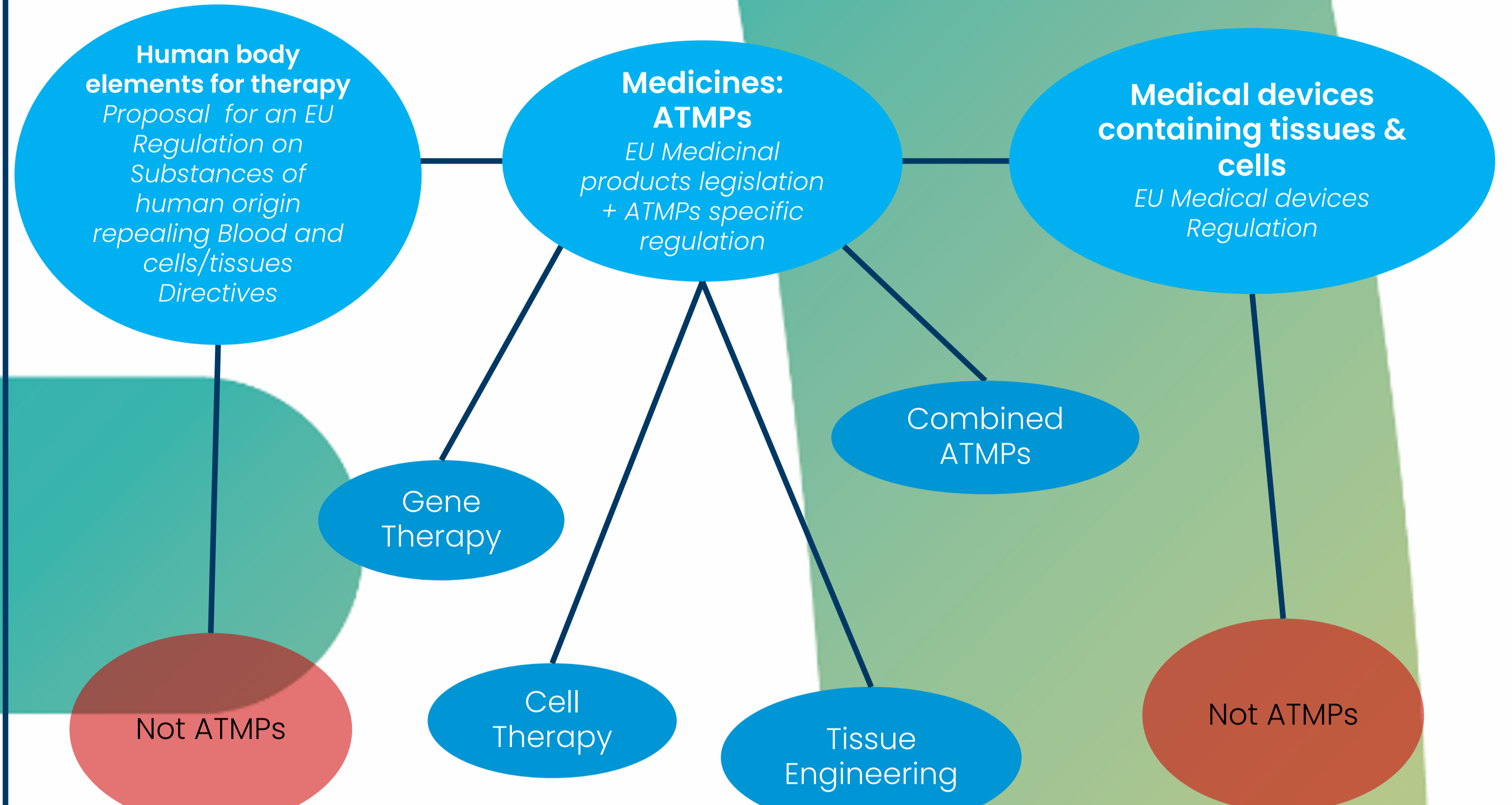
Communicating information on the regulatory framework applicable to gene and cell therapies, at every stage of development and towards every actor involved, is a challenge. Therapy development needs to comply with several regulatory requirements mostly to ensure patients' safety. The complexity comes from the diversity of the regulatory landscape. Some requirements are binding, others are guidance only. Most aspects are governed by European Union law, but some remain regulated on a national scale. The regulatory landscape is a combination of general and specific rules and guidance depending on competent institutions, legal classifications of the products, and steps of their developments.

Methodology

Communicating information on GCT with an online tool



Legal Classification of Gene & Cell Therapy



CHALLENGES

- TEMPORALITY**
- Changes in regulation or legislation
 - Scientific discoveries

- DIVERSITY OF ACTORS**
- European Medicines Agency
 - National Medicines Agencies
 - HTA Bodies
 - Ethics committees
 - GMO authorities

- LEGAL COMPLEXITY**
- European Union law vs. national laws
 - Binding texts vs. guidance
 - Legal classification leading to different pathways

Addressing the challenges in finding information with EuroGCT Research Pathways

Research & Innovation | Therapy Classification | Manufacturing | Commercialisation | Public Involvement & Data

- Fundamental Research
- Clinical Research
- Translational Science
- Incentives
- Early Interactions with Regulators
- See figure above
- Manufacturing Authorisation
- Good Manufacturing Practice
- Investigation Medicinal Product Dossier
- Capability
- Scalability
- Packaging and Labelling
- Market Access for ATMPs
- Distribution
- Advertising
- Pharmacovigilance
- Pricing and Reimbursement
- Data Protection
- Data storage
- Data collection, processing, controlling
- Data sharing and Open Data
- Mission creep and Data Misuse

CONCLUSION

The EuroGCT Research Pathway resources facilitates better decision-making at key points in development of new therapies and thus enable improved product development, by providing the research community, regulatory and healthcare authorities with an information source on the practical steps needed for cell and gene therapy development.

Creating and documenting this process adds value to the community as a whole as we navigate bringing novel treatments responsibly from the lab to the clinic.

CONTRIBUTE

Interested in contributing your insight? We welcome involvement in the further development of the EuroGCT Research Pathways Resource.

- 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
 Auxane Delage: auxane.delage@univ-amu.fr
 Luc-Sylvain Gilbert: luc.gilbert@univ-amu.fr