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EuroGCT D4-2: Resources on practical steps- a set of information resources on the practical steps needed for cell and gene therapy development

Hsin-Yu Kuo, Aurélie Mahalatchimy, Luc-Sylvain Gilbert, Auxane Delage,
Clare Blackburn

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***Project title: European Consortium for Communicating
Gene and Cell Therapy Information***

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***A set of information resources on the practical steps needed for
cell and gene therapy development***

Date: 31st July 2023

Project	
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D 4.2 – Resources on practical steps, a set of information resources on the practical steps needed for cell and gene therapy development

The European Consortium for Communicating Gene and Cell Therapy Information (EuroGCT) has two major objectives:

- To provide patients, people affected by conditions, healthcare professionals and citizens with accurate scientific, legal, ethical and societal information and with engagement opportunities, and thus to support better informed decision-making related to cell and gene-based therapies.
- To facilitate better decision-making at key points in development of new therapies and thus enable improved product development, by providing the research community and regulatory and healthcare authorities with an information source on the practical steps needed for cell and gene therapy development.

Work Package 4 (WP4) is developing an information hub for the research and regulatory communities that facilitates access of these communities to information on the practical steps needed for cell- and gene-based therapy development and promotes information exchange within and between these communities.

Deliverable 4.2 is a set of information resources on the practical steps needed for cell and gene therapy development, which can be viewed online at www.eurogct.org/research-pathways.

Research Pathways

Bringing therapies from the lab bench to patients is a complex process. We've compiled resources and opportunities to help guide through the development process. From target identification to final products.

Find new information and resources for researchers, regulators, and those interested in therapy development.

[Learn more about Research Pathways](#)

Additional navigation options
More ways to navigate Research Pathways

- Key Pathways**
Supporting key resources on CAR development
- Case Studies**
Learn from specific development cases and relevant resources
- Public and Public Engagement**
A CAR bridge between and Public Engagement Directory
- Advis & Networks**
Mapping the stakeholders in Europe and beyond

Search with keywords: List updated: Descending: (Showing 12 of 32)

Filter by:

- Theme:
- Target audience:
- Region:
- Resource type:
- Organization type:
- Disease of focus:

EATIS Transmed Academy
EATIS Transmed Academy is the EATIS online learning environment for translational scientists. Find here well-paced online courses, live courses, workshops, recorded webinars, guidelines documents, resources and more.
[Show all >](#)

EBDC Orphan Drug Development Guide
The Orphan Drug Development Handbook (ODDH) by the International Rare Diseases Research Consortium (IRDiC) is a patient-focused guideline that describes the marketable, incentives, resources and practice needs for developing orphan and innovative drug therapies for rare disease indications and how to best use them.
[Show all >](#)

Human body elements for therapeutic applications
Human body elements for therapeutic applications (HBE) human body elements, including tissues, cells, blood and stem cell preparations for use in regenerative medicine.
[Show all >](#)

Advertising
Advertising of medicinal products including ATMPs, is mainly regulated by national laws apart from minimum binding requirements established by EU law.
[Show all >](#)

Case Study: Holofer
Discover our Holofer project in one of the few ATMPs that have been granted marketing authorisation in Europe. An interesting case of collaboration about pathologic fibrosis in the liver. From early research to approval for the research community to registration in development programs.
[Show all >](#)

Specificities of the marketing authorisation dossier for biopharmaceuticals
This document provides information on specificities of the marketing authorisation dossier for biopharmaceutical products (ATMPs) at dossier level compared to the existing reference marketing products which cover the regulatory aspects. The document provides information on the specificities of the marketing authorisation dossier for biopharmaceutical products in terms of quality and clinical aspects.
[Show all >](#)

The EBMT/ISCT CAR-T Cell Handbook
This open access CAR-T cell handbook published by Europe and led by partners in the European Society for Blood and Marrow Transplantation (EBMT) and the European Hematology Association (EHA) provides an up-to-date overview of the CAR-T cell technology and covers some aspects of CAR-T cell treatments, including the underlying biology, indications, management of side effects, access and manufacturing issues.
[Show all >](#)

Conditional Marketing Authorisation
To facilitate early patient access to medicinal products, marketing authorisation (MA) can be granted through specific regulatory mechanisms offered by European states. When an advanced therapy medicinal product (ATMP) application or other medicinal product is conditional marketing authorisation (CMA) may be granted on the basis of less complete data than usually required.
[Show all >](#)

Data sharing / Open data
EU and UK 2023 4. Experts University of Toulouse (UCL) and GCT/ODD
Following the receipt of funding from the European Union, the data made available in this use for a defined period of time, subject to applicable national, European, legal or organisational requirements. Open data refers to making available research-related publications without restrictions regarding the access.
[Show all >](#)

Accelerated Assessment
EU and UK 2023 4. Experts
To facilitate early patient access to medicinal products, marketing authorisation (MA) can be granted through specific regulatory mechanisms offered by European states. The accelerated assessment is a procedure that allows the marketing authorisation holder to submit a marketing authorisation application. It is designed to support the development of innovative medicinal products for the treatment of serious conditions, in particular from the perspective of Therapeutics Innovation.
[Show all >](#)

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This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement 101019714 (EURO-GCT) and contributing partners.

Screenshot of Research Pathways information hub: www.eurogct.org/research-pathways 12:26, 26 July 2023

The Research Pathways page has been redeveloped and published online as a filterable and searchable database. (Details in **D4.1**). The indexed database contains information on practical steps needed for cell and gene therapy development developed by the EuroGCT consortium or signposting to external resources. These resources include guidance on manufacturing guidelines, regulatory and scientific guidelines, intellectual property rights, market acceptability and ethical matters. It builds on the initial information published (**D4.9**), and further enhanced by the new search/filter functions, links to grouped topical sections, as well as new content developed by the project and signposting to additional collated resources. Contents within Research Pathways will be continuously updated throughout the project duration.

The Research Pathways page also offers a gateway towards topical sections outlined as follows:

- **Key Research Pathways:** this section contains the collection of resources developed by EuroGCT WP4.
- **Key Pathway Resources:** this section contains collated key resources on ATMP development by medicines agencies, and existing complementary work produced by other organisations.
- **Case Studies:** this section contains our collection of new 'case study' content type, presenting collated resources on the development of specific ATMPs.
- **Patient and Public Involvement:** this links to the ATMP Engage Patient and Public Involvement (PPI) Directory, a searchable and filterable directory with PPI resources for developers of cell and gene therapies.
- **Actors and Network:** this section contains our initial mapping of stakeholders in ATMP development, covering the breadth of advanced therapies activities in Europe and beyond.

Screenshots of these sections along with example pages will be shown in the following pages of this report.

Home / Research Pathways / Key Research Pathways

Key Research Pathways

Development pathways for bringing gene and cell therapies from the lab to patients

Gene and cell therapies are evolving rapidly and show promise for treating many diseases and conditions. Hurdles remain in bringing safe, efficacious and high-quality medicines based on genes, cells or tissues - Advanced Therapy Medicinal Products (ATMPs) - to patients in a timely manner.

EuroGCT aims to provide the research and regulatory communities with reliable and accessible information on the practical steps needed for gene and cell therapy development while promoting information exchange within and between these communities.

As this initiative develops, we begin with aggregating existing resources for guidance at each stage of development and will go on to fill information gaps identified in the field with expert-reviewed material. We hope to promote the free flow of knowledge to facilitate informed decision-making for researchers and regulators developing new therapies.

Research and Innovation

The development of innovative therapies starts with translating discoveries from basic research. This section explores the steps from fundamental to pre-clinical and clinical research underpinning the development of innovative gene and cell therapies.

Research and Innovation includes information on:

- [Clinical research](#)
- [Translational Science](#)

[Read more on Research and Innovation](#)

Therapy Classification

Therapy classification determines the legal mechanisms that govern the regulation of gene and cell therapies to ensure the quality, safety, and efficacy of these therapies on the EU market.

Therapy Classification includes information on:

- [ATMPs](#)
- [Detailed look elements for therapeutic evaluation](#)

[Read more on Therapy Classification](#)

Manufacturing

Advanced therapy medicinal products (ATMP) manufacturing concerns the industry-scale production of these therapies for supporting clinical development and commercialisation. This section provides an overview of the ATMP manufacturing processes, key terms, and related resources.

Manufacturing includes information on:

- [Manufacturing Authorisation](#)
- [Good Manufacturing Practice](#)
- [Packaging and labelling](#)

[Read more on Manufacturing](#)

Commercialisation

Enabling timely patient access to Advanced Therapy Medicinal Products (ATMPs), commercialisation covers marketing authorisation, and post-authorisation activities such as pharmacovigilance, pricing and reimbursement.

Commercialisation includes information on:

- [Market Access for ATMPs](#)
- [Advertising](#)

[Read more on Commercialisation](#)

Actors and Networks

The thriving field of gene and cell therapy is supported by a large community of academic, clinical, translational, industrial and patient organisations. This section maps the stakeholders and their activities in Europe and beyond.

[Read more on Actors and Networks](#)

Public Involvement and Data

Developing gene and cell therapy involves people and their data. This section introduces best practices for public involvement and the handling of health data throughout the therapy development process.

Public Involvement and Data includes information on:

- [Data collection](#)
- [Data collection, accession, curation](#)
- [Data privacy & governance](#)
- [Minimum necessary data collection](#)

[Read more on Public Involvement and Data](#)

[Back to Research Pathways](#)

Screenshot of Key Research Pathways: www.eurogct.org/research-pathways/key-research-pathways 12:26, 26 July, 2023

The 'Key Research Pathways' section contains the collection of original resources produced by the EuroGCT WP4. These guides are categorised into six main groups: Research and Innovation, Therapy Classification, Manufacturing, Commercialisation, Actors and Networks, and Public Involvement and Data. Each category has short introduction text and links to published EuroGCT guides for the practical steps available at each stage of development.

The screenshot displays the EuroGCT website's content for 'Conditional Marketing Authorisation'. The main text is organized into several key sections:

- Introduction:** Discusses the regulatory framework and the role of EMA in Europe.
- Stakeholders:** Identifies key players including regulators, industry, and patients.
- Challenges:** Outlines the complexities and hurdles in the regulatory process.
- Opportunities and Incentives:** Explores the benefits and motivations for seeking conditional marketing authorisation.
- Interactions with regulators:** Details the communication and collaboration required with regulatory bodies.
- Practical steps:** Provides a structured approach to navigating the regulatory process.

A sidebar on the right side of the page features a 'Table of contents' with the following items:

- Introduction
- Stakeholders
- Challenges
- Opportunities and Incentives
- Interactions with regulators
- Practical steps

Screenshot of 'Conditional Marketing Authorisation' entry: www.eurogct.org/research-pathways/commercialisation/market-access-atmps/expediting-marketing-authorisation-0 12:41, 27 June, 2023

This is an example page of EuroGCT's original resource. More published resources can be directly accessed through the Key Research Pathways Section: www.eurogct.org/research-pathways/key-research-pathways or be found through searching in the main Research Pathways database: <https://www.eurogct.org/research-pathways>

Home / Key Pathway Resources

Key Pathway Resources

The section contains key resources on ATMP development by medicines agencies and working in partnership with other organisations.

Resources by European Medicines Agency

European Medicines Agency
 Key resources available on ATMP development by medicines agencies and working in partnership with other organisations.

[Key EMA Resources](#)

Existing complementary work on ATMP development

ATMP Sweden Regulatory Guide
 ATMP Sweden has compiled a starting point manual regarding ATMP development, which may assist ATMP developers in understanding the regulatory process.

[ATMP Sweden Resources](#)

EJP RD Innovation Management Toolbox
 EJP RD is a cross-border network of excellence in rare disease research and innovation. This toolbox will help you understand the toolbox and how EJP RD can help you.

[EJP RD Resources](#)

ARM Project A-Cells and Project A-Cell
 The Alliance for Regenerative Medicine (ARM) and its partners have established a Gene and Cell Therapy (GCT) project to support the development of regenerative medicine products. The project is led by the European Medicines Agency (EMA) and the European Commission (EC).

[ARM Resources](#)

ADVI ATMP Roadmap
 The roadmap for advanced therapy medicinal products (ATMP) has been developed as an ATMP roadmap, which will help you understand the regulatory process and the development of ATMP products.

[ADVI Resources](#)

EATRIS Translational Academy and ADVANCE Project
 The translational academy (TA) and the advanced therapy medicinal products (ATMP) development project (ADVANCE) are two key initiatives of the European Medicines Agency (EMA) and the European Commission (EC).

[EATRIS Resources](#)

IRDIRC Orphan Drug Development Guide
 A patient-focused guidebook by the International Rare Disease Research Consortium (IRDIRC) on developing drugs or therapies for rare disease indications.

[IRDIRC Orphan Drug Development Guide](#)

ATTC Manufacturing and Preparation Toolkit
 The Advanced Therapy Medicines Production (ATMP) development the manufacturing and preparation toolkit to provide you with the information you need to develop your ATMP product.

[ATTC Resources](#)

NCATS Platform Vector Gene Therapy (Pave-GT) pilot project
 The National Center for Advancing Translational Research (NCATS) is leading a pilot project to develop a platform for the development of gene therapy products.

[NCATS Resources](#)

Restora Project
 The Restora project develops technologies that will enable the development of advanced therapy medicinal products (ATMP) for the treatment of rare diseases.

[Restora Project](#)

Resources for the UK context

Key UK Resources
 A collection of key resources for gene and cell therapy development in the UK context.

[Key UK Resources](#)

Table of contents
 Resources by European Medicines Agency
 Existing complementary work on ATMP development
 Resources for the UK context

[Back to Home/UP Pathways](#)

Screenshot of 'Key Pathway Resources': www.eurogct.org/key-pathway-resources 12:41, 27 June, 2023

The 'Key Pathway Resources' section contains a collection of key resources on ATMP development published by European Medicines Agency (EMA), other projects, and/or organisations active in the field of gene and cell therapy.

[Home](#) / [Case Studies](#)

Case Studies

This section contains case studies on ATMP development. Follow the links below to learn about the development processes, the keys to success, the challenges and how developers overcame them:

Case study: Holoclar

Learn about the development of
Holoclar

[Go to case study](#)

Case Study: Strimvelis

A successful public-private
partnership bringing ATMP to market

[Go to case study](#)

[Back to Research Pathways](#)

Did you find the content on this page useful? If not, you can leave us a message so we can improve

[Send us your thoughts](#)

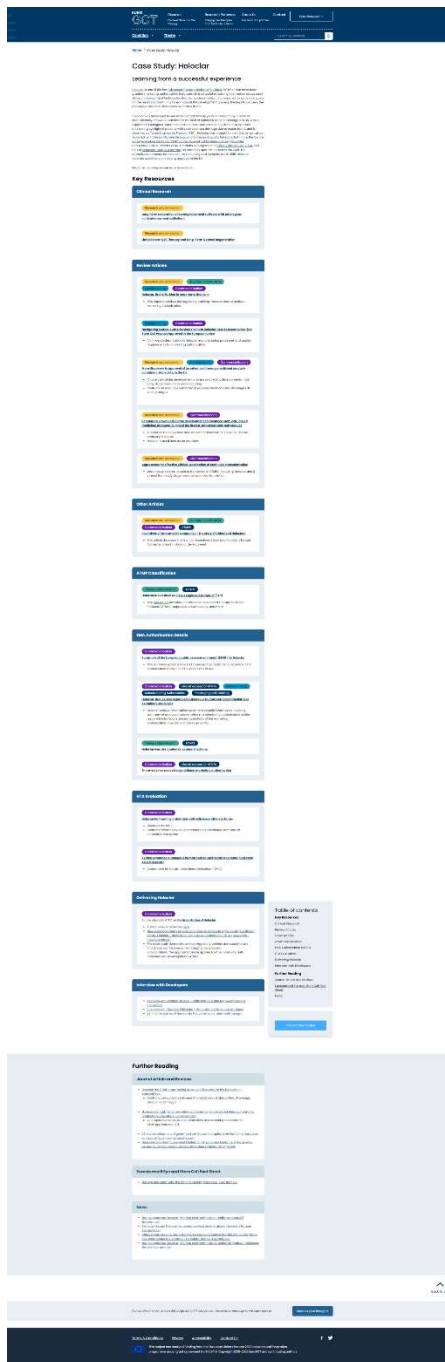
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Screenshot of 'Case study' section: www.eurogct.org/case-studies 12:41, 26 July, 2023

The 'Case Studies' section contains case studies on ATMP development. Currently there are two case studies on approved ATMPs (Holoclar and Strimvelis). Future work will involve co-developing case studies with partners for them to share their experiences and learnings in ATMP development. More details can be found in **D4.1-** Landscape mapping.



Screenshot of 'Case Study: Holoclar': www.eurogct.org/case-study-holoclar 12:41, 27 June, 2023

Here is an example of case study pages. In this case study for Holoclar, we collated existing information about each stage of its development, from early-stage research to approval and beyond, to serve as a guide for the research community to learn about the development process, the keys to success, the challenges and how developers overcame them. These case studies provide researchers and developers with an opportunity to engage with and to create supporting resources for the researcher and developer community.

ATMP Engage PPI Directory
 The PPI Directory contains patient and public involvement (PPI) resources for developers of cell and gene therapies, including toolkits, guidelines, advice, templates and case studies.

Looking for a specific resource? Begin typing to find it

Search Directory

Who and What is PPI?
 These resources set the context for patient and public involvement - what is it, what are the benefits, and who is involved?
[Learn more about PPI](#)

Planning and Management
 These resources include practical steps to plan and manage PPI, from strategy and budget to training and communication.
[Learn more about Planning and Management](#)

Case Studies
 These case studies offer examples of good PPI practice.
[Go to case studies](#)

Feedback and Evaluation
 These resources include support for recording and evaluating the impact of PPI and incorporating feedback into the process.
[Learn more about feedback and evaluation](#)

Interested in PPI, but not sure what you are looking for? Browse all the PPI directory resources or find out more about the PPI directory here

[Browse the PPI Directory](#)

[About the PPI Directory](#)

Who are we?
 The Advanced Therapy Medicinal Products Patient and Public Involvement and Engagement Working Group (ATMP Engage) brings together UK-based stakeholders with an interest in ATMPs to discuss and collaborate on PPI activity.
[Learn more about ATMP Engage](#)

ATMP Engage | Public & Patient Involvement Working Group

ATTC | CATAPULT | GENETIC ALLIANCE

EURO GCT | NHS | ACCELERATED ACCESS COLLABORATIVE

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Screenshot of 'Patient and Public Involvement (landing page)': www.eurogct.org/ppi-directory 13:05, 27 June, 2023

The 'Patient and Public Involvement' (PPI) section is linked to the ATMP Engage PPI Directory. This directory contains more than 150 PPI resources for developers of cell and gene therapies, including toolkits, guidelines, advice, templates and case studies.

The screenshot displays the ATMP Engage PPI Directory website. At the top, there is a navigation bar with the EURO GCT logo and menu items: Home, Research Pathways, About Us, Contact, and User Settings. Below the navigation bar is a search bar with a dropdown menu for 'Condition' and a 'Search by keyword' input field.

The main content area is titled 'ATMP Engage PPI Directory' and includes a brief description: 'The Directory contains patient and public involvement (PPI) resources for developers of cell and gene therapies, including books, guidelines, advice, templates and case studies.' Logos for ATMP, CATAPULT, and NHS are visible.

Below the header, the page shows search results for 'PPI Themes'. The left sidebar contains a list of filters: PPI Themes (with sub-items like PPI Basics and Manuals, Stakeholder for PPI, Roles and Responsibilities, PPI Tools/State of Organisations, Knowledge Building, Strategies/Challenges/Issues on PPI), Research Field (Genetics, Biomedical Lab, Pharmaceutical, ATMP/Gene Therapy Development, Other), Organisation Type (Funder, Academia, Healthcare, Regulatory Body, NHS, Industry, Third Sector/Charity, Other Organisation), Development Stage (Identifying/Understanding Patient Need, Research and Clinical Development, Regulatory Approval, Health Technology Assessment, Service Delivery), Audience (Research/Developers, Public/Patients, Clinical Sector, Industry, Other groups), Country (UK, Europe, North America, Other countries), and Content Type (Resource, Journal, Repository, Article).

The main results area shows 'Showing 5 of 157 results' and 'Page 1 of 34'. Five search results are displayed as cards:

- ATMP PPI Articles**: Content Type: Article. Includes a 'View this Resource' button.
- Patient Engagement Training and Resources Repository**: Organisation Type: Fundraiser/Charity. Content Type: Repository. Includes a 'Visit this Resource' button.
- Patient Engagement Resources**: Organisation Type: Fundraiser/Charity. Content Type: Repository. Includes a 'Visit this Resource' button.
- PCORI Events**: Organisation Type: Funder. Content Type: Repository. Includes a 'Visit this Resource' button.
- Engagement in health research literature explorer**: Organisation Type: Funder. Content Type: Repository. Includes a 'Visit this Resource' button.

At the bottom of the results area, there is a pagination control showing '1 2 3 4 5 - - total'.

Below the search results, there is a 'Who are we?' section with the ATMP Engage logo and a list of partner logos: ATMP, CATAPULT, GENETIC ALLIANCE, EURO GCT, and NHS ACCELERATED ACCESS COLLABORATIVE.

The footer contains 'Terms & Conditions', 'Privacy', 'Accessibility', and 'Contact Us' links, along with social media icons for Facebook and Twitter. A small text block states: 'This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 101016744. Copyright 2022. All rights reserved.' and 'EURO GCT' logo.

Screenshot of 'Patient and Public Involvement (search page)': www.eurogct.org/atmp-ppi-database 13:05, 27 June, 2023

The collated PPI resources in this directory are fully indexed, making it searchable and filterable.

Screenshot of 'Actors and Network': www.eurogct.org/research-pathways/actors-and-networks 13:05, 26 July, 2023

The 'Actors and Network' section contains 'our initial information' on the mapping of gene and cell therapy stakeholders in Europe and beyond. It will be further developed and reported in **D4.3** due month 48.