



**HAL**  
open science

## **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

### **► To cite this version:**

Hsin-Yu Kuo, Aurélie Mahalatchimy, Luc-Sylvain Gilbert, Auxane Delage, Clare Blackburn. EuroGCT D4-2: Resources on practical steps- a set of information resources on the practical steps needed for cell and gene therapy development. European Commission - CORDIS. 2023, <https://cordis.europa.eu/project/id/965241/results>. halshs-04290639

**HAL Id: halshs-04290639**

**<https://shs.hal.science/halshs-04290639v1>**

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.

## ***Deliverable (Record of Website)***

***Grant Agreement Number: 965241***

***Project acronym: EuroGCT***

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

***Deliverable: D4.2 Resources on practical steps***

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

***Date: 31st July 2023***

<b>Project</b>	
Project acronym:	EuroGCT
Project full title:	European Consortium for Communicating Gene and Cell Therapy Information
Grant agreement no.:	965241
Funding scheme:	Co-ordination and Support Action
Project start date:	1 February 2021
Project duration:	60 months
Call topic:	SC1-HCO-19-2020 - Reliable and accessible information on cell and gene-based therapies
Project web-site:	<a href="http://www.eurogct.org">www.eurogct.org</a>
<b>Document</b>	
Deliverable number:	D4.2
Deliverable title:	Resources on practical steps, a set of information resources on the practical steps needed for cell and gene therapy development
Due date of deliverable:	Month 30
Associated Milestones	NA
Actual submission date:	31 July 2023
Last Editor:	Hsin-Yu Kuo
Contributors:	Hsin-Yu Kuo, Aurélie Mahalatchimy, Luc-Sylvain Gilbert, Auxane Delage, Clare Blackburn
Work Package no.:	4
Work Package title:	An Information and Engagement Hub for the Research and Regulatory Communities
Work Package lead beneficiary:	2-AMU
Work Package participants:	All partners
Dissemination level:	Public
Nature:	Website
Draft/Final:	Final
No of pages (including cover):	13

## **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](http://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:
- Organisation type:
- Disease 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 >](#)

**EBIC 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 medicines, resources and practice models for developing traditional 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 cellular therapies programs.  
[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 >](#)

**Gene Study Helpline**  
Gene Study Helpline is one of the few ATMPs that have been granted marketing authorisation in Europe. We are pleased to offer information about our gene study helpline for clinicians from gene therapy research to support for the research community to support clinical development programs.  
[Show all >](#)

**Specificities of the marketing authorisation dossier for biopharmaceuticals**  
This document provides information on the specificities of the marketing authorisation dossier for biopharmaceutical products compared to the existing reference marketing products which are not for medicinal purposes. The application for a marketing authorisation dossier to demonstrate a significant disease high similarity of the biopharmaceutical to an existing medicinal product in terms of quality and clinical efficacy.  
[Show all >](#)

**The EBMT Jena CAR-T Cell Handbook**  
This open access CAR-T cell handbook published by Springer and co-edited by 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, storage 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 to European states when an advanced therapy medicinal product (ATMP) demonstrates an unmet medical need of patients a conditional marketing authorisation (CMA) may be granted on the basis of less comprehensive data than usually required.  
[Show all >](#)

**Data sharing / Open data**  
To promote the reuse of research data, access to data from data holders is data used for a defined purpose and subject to applicable national, European, legal or organisational requirements. Open data refers to making available research-related publications without restrictions regarding the reuse.  
[Show all >](#)

**Accelerated Assessment**  
To facilitate early patient access to medicinal products, marketing authorisation (MA) can be granted through specific regulatory mechanisms offered to European states. The accelerated assessment is a procedure to reduce the time needed to obtain marketing authorisation for medicinal products. It is designed to support the development of innovative medicines for the treatment of patients with unmet medical needs, in particular from the perspective of Therapeutics Innovation.  
[Show all >](#)

[Terms & Conditions](#) [Privacy](#) [Accessibility](#) [Contact Us](#)

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement/101019720/2020-2023/ERC-101019720/2020-2023/ERC-101019720

Screenshot of Research Pathways information hub: [www.eurogct.org/research-pathways](http://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 indications](#)

[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](http://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 shows a webpage with the following structure:

- Introduction**: A paragraph of text.
- Stakeholders**: A list of stakeholders involved in the process.
- Challenges**: A list of challenges faced in the process.
- Opportunities and Incentives**: A list of opportunities and incentives.
- Interactions with regulators**: A list of interactions with regulators.
- Practical steps**: A list of practical steps.
- Table of contents**: A list of contents.

Screenshot of 'Conditional Marketing Authorisation' entry: [www.eurogct.org/research-pathways/commercialisation/market-access-atmps/expediting-marketing-authorisation-0](http://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](http://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 reporting and assessment guide 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 collaborative network of medicines agencies across Europe, which have developed this toolbox to assist EJP RD members.

[EJP RD Resources](#)

**ARM Project A-Cells and Project A-Cell**  
 The Alliance for Regenerative Medicine (ARM) and partners have published a Gene and Cell Gene therapy roadmap for Regenerative Quality by design's (RQD) project to support the development of Cell therapy manufacturing. Contact ARM for more information.

[ARM Resources](#)

**ADVI ATMP Roadmap**  
 The Association of European Regenerative Medicine (AERM) has developed an ATMP roadmap, which will assist the industry in understanding the regulatory process. The roadmap is based on the current regulatory framework for patient treatment, including gene therapy, cell therapy, and other related resources developed by AERM.

[ADVI Resources](#)

**EATRIS Transmed Academy and ADVANCE Project**  
 EATRIS is a transnational project, which aims to develop a common framework for the development of ATMPs. The project is supported by the European Union and the industry.

[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 Association of Regenerative Therapies (ART) has developed the Manufacturing and Preparation Toolkit to provide a practical guide for manufacturing ATMPs. The toolkit covers all aspects of ATMP development.

[ATTC Resources](#)

**NCATS Platform Vector Gene Therapy (PoVE-GT) pilot project**  
 The National Center for Advancing Translational Research (NCATS) is supporting a pilot project to develop a platform for the development of gene therapy. The project is supported by the National Institutes of Health (NIH).

[NCATS Resources](#)

**Restora Project**  
 The Restora project develops technologies that will enable the development of regenerative medicine. The project is supported by the European Union and the industry.

[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 Roadmap Pathways](#)

Screenshot of 'Key Pathway Resources': [www.eurogct.org/key-pathway-resources](http://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](#)

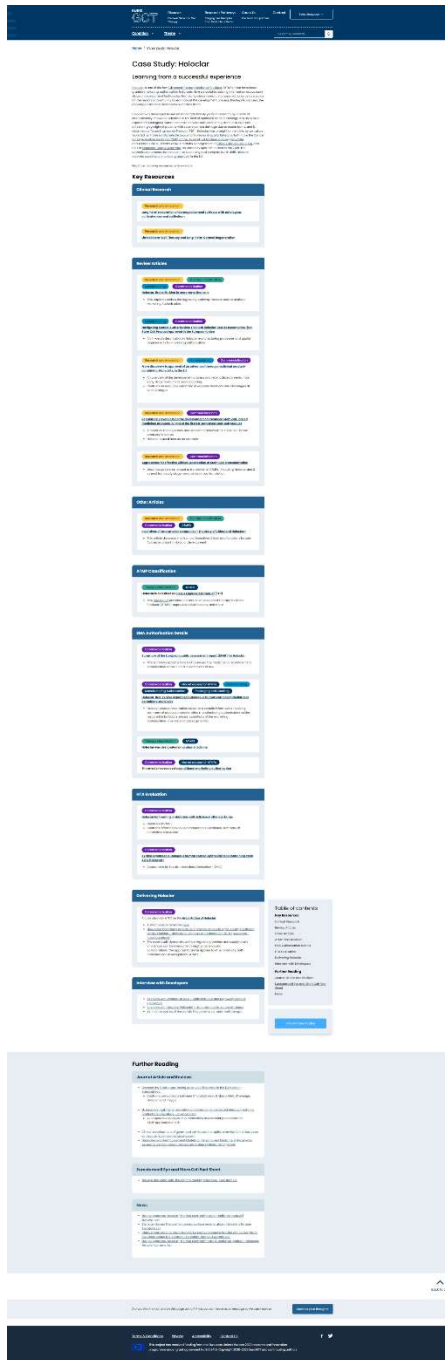
[Terms & Conditions](#) [Privacy](#) [Accessibility](#) [Contact Us](#)



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 985241.0 Copyright 2020-2022 EuroGCT and contributing authors

Screenshot of 'Case study' section: [www.eurogct.org/case-studies](http://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](http://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.

Screenshot of 'Patient and Public Involvement (landing page)': [www.eurogct.org/ppi-directory](http://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 links for Home, Research Pathways, Engage with Patients, About Us, Contact, and User Settings. Below the navigation bar is a search bar and a dropdown menu for 'Condition' and 'Theme'. The main header area features the 'ATMP Engage PPI Directory' title and a description: 'The ATMP Engage PPI Directory contains patient and public involvement (PPI) resources for developers of cell and gene therapies, including booklets, guidelines, advice, templates and case studies.' Logos for ATMP, CATAPULT, and NHS are also visible.

The main content area shows search results for 'ATMP PPI Articles'. On the left, there are several filter categories: PPI Themes, Research Field, Organisation Type, Development Stage, Audience, Country, Content Type, and Who are we?. The search results list several articles, each with a title, a brief description, and a 'Visit this Resource' button. The articles include:

- ATMP PPI Articles** (Article)
- Patient Engagement Training and Resources Repository** (Repository)
- Patient Engagement Resources** (Repository)
- PCORI Events** (Repository)
- Engagement in health research literature explorer** (Repository)

At the bottom of the page, there is a footer with 'Terms & Conditions', 'Privacy', 'Accessibility', and 'Contact Us' links. 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 101016144. Copyright 2022 EuroGCT and contributing authors.'

Screenshot of 'Patient and Public Involvement (search page)': [www.eurogct.org/atmp-ppi-database](http://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.

The screenshot displays the 'Actors and Networks' section of the EURO GCT website. The page is structured as follows:

- Actors and Networks:** A brief introduction followed by a search bar.
- Research Pathways:** A vertical list of links including:
  - Research and innovation
  - Therapy Classification
  - Regulatory
  - Commercialisation
  - Access and delivery
  - Public involvement and Care
- Actors Activity Map:** A section with a search bar and a map area.
- Table of contents:** A vertical list of links including:
  - Actors activity map
  - Networks
  - National medicines agencies
  - Other National Competent Authorities (NCAs)
  - National Health Technology Assessment (NHTA) Bodies
  - Research Ethics Committees
  - Training providers
- Networks:** A section with a search bar and a list of links for various networks.
- National and International Societies:** A list of links to various societies.
- Regional Networks:** A list of links to regional networks.
- EU Projects:** A list of links to EU projects.
- National Medicines Agencies:** A list of links to national medicines agencies.
- Other National Competent Authorities in EU:** A list of links to other national competent authorities.
- National Health Technology Assessment (HTA) Bodies:** A list of links to national HTA bodies.
- Research Ethics Committees:** A list of links to research ethics committees.
- Training Providers:** A list of links to training providers.

At the bottom of the page, there are three icons representing different areas: 'EURO GCT', 'Actors and Networks', and 'Training Providers'. Below these icons are two buttons: 'Back to Home' and 'Back to Research Pathways'.

Screenshot of 'Actors and Network': [www.eurogct.org/research-pathways/actors-and-networks](http://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.