



**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.



HAL Authorization

## ***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. Don't forget to register to our platform.

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.  
Theme: Research and Innovation, Translational Science. Show all >

**EBDC Orphan Drug Development Guide**  
EBDC EBDC  
The Orphan Drug Development Handbook (ODDH) by the International Rare Diseases Research Consortium (IRDiC) is a patient-focused guideline that describes the worldwide regulatory, research and practice needs for developing orphan and innovative drug therapies for rare disease indications and how to best use them.  
Theme: Research and Innovation, Orphan-drug Research. Show all >

**Human body elements for therapeutic applications**  
EBDC EBDC EBDC  
Human body elements for therapeutic applications (HBE) is a patient-focused guideline that describes the worldwide regulatory, research and practice needs for developing orphan and innovative drug therapies for rare disease indications and how to best use them.  
Theme: Research and Innovation, Orphan-drug Research. Show all >

**Advertising**  
EBDC EBDC EBDC  
Advertising of medicinal products including ATMPs, is mainly regulated by national laws apart from minimum binding requirements established by EU law.  
Theme: Communication, Advertising. Show all >

**Case Study: Holofer**  
EBDC EBDC EBDC  
Discover our Holofer project in one of the few ATMPs that have been granted marketing authorisation in Europe. An interesting case study about a patient's journey from diagnosis to therapy. Register to our platform for the research community to be updated on development progress.  
Theme: Research and Innovation, Fundamental Research. Show all >

**Specificities of the marketing authorisation dossier for biopharmaceuticals**  
EBDC EBDC EBDC  
This document provides information on the specificities of the marketing authorisation dossier for biopharmaceuticals. It covers the regulatory requirements for the marketing authorisation dossier for biopharmaceuticals, including the marketing authorisation dossier for biopharmaceuticals, including the marketing authorisation dossier for biopharmaceuticals.  
Theme: Communication, Market access for ATMPs. Show all >

**The EBMT/ISCT CAR-T Cell Handbook**  
EBDC EBDC EBDC  
This handbook provides a comprehensive overview of the current state of CAR-T cell technology and covers various aspects of CAR-T cell treatments, including the underlying biology, indications, management of side effects, access and manufacturing issues.  
Theme: Fundamental Research, Manufacturing. Show all >

**Conditional Marketing Authorisation**  
EBDC EBDC EBDC  
To facilitate early patient access to medicinal products, marketing authorisation (MA) can be granted through specific regulatory mechanisms offered to European states. When an MA is granted through specific regulatory mechanisms offered to European states, the MA is granted through specific regulatory mechanisms offered to European states.  
Theme: Communication, Market access for ATMPs. Show all >

**Data sharing / Open data**  
EBDC EBDC EBDC  
Data sharing is a key element of open science. It allows researchers to share their data and to use it for a defined purpose. It is a key element of open science. It allows researchers to share their data and to use it for a defined purpose. It is a key element of open science.  
Theme: Data sharing / Open Data, Ethics. Show all >

**Accelerated Assessment**  
EBDC EBDC EBDC  
To facilitate early patient access to medicinal products, marketing authorisation (MA) can be granted through specific regulatory mechanisms offered to European states. When an MA is granted through specific regulatory mechanisms offered to European states, the MA is granted through specific regulatory mechanisms offered to European states.  
Theme: Communication, Market access for ATMPs. Show all >

1 2 3 4 5 6 Next >>>

**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/ID number: 101019718

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 classification](#)

[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:

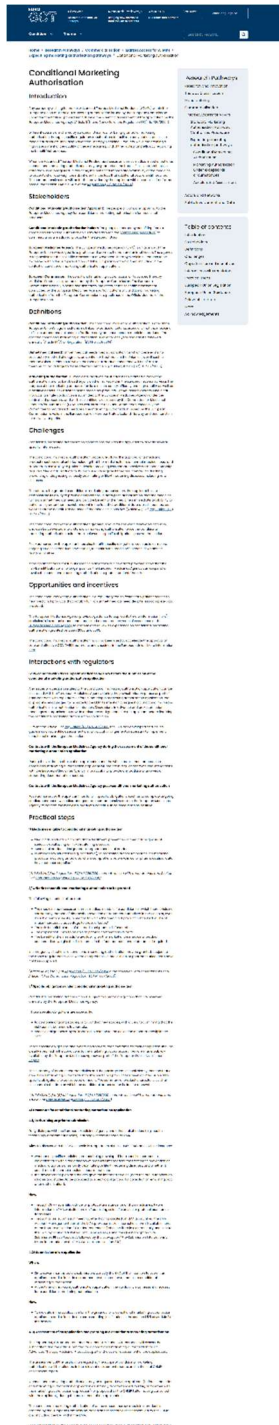
- [Data collection](#)
- [Data collection, accession, curation](#)
- [Data privacy & governance](#)
- [Minimum necessary data collection](#)

[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.



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 cross-border network of excellence in rare disease research. The toolbox aims to help researchers and clinicians EJP RD researchers.

[EJP RD Resources](#)

**ARM Project A-Cells and Project A-Cell**  
 The Alliance for Regenerative Medicine (ARM) and partners have established a Gene and Cell line safety-based pathway for Regenerative Quality or Regenerative Quality for Regenerative Quality (RQR) process. The pathway is based on the Common Data Element (CDE) and the Common Data Element (CDE) and the Common Data Element (CDE) and the Common Data Element (CDE).

[ARM Resources](#)

**ADVI ATMP Roadmap**  
 The roadmap for advanced therapy medicinal products (ATMP) has been developed as an ATMP roadmap, which will help the industry to understand the regulatory process and the challenges of ATMP development. The roadmap is based on the current regulatory framework and the challenges of ATMP development.

[ADVI Resources](#)

**EATRIS Translational Academy and ADVANCE Project**  
 The translational academy and the ADVANCE project are designed to support the development of ATMPs. The translational academy is a platform for the exchange of knowledge and experience between researchers and clinicians. The ADVANCE project is a platform for the exchange of knowledge and experience between researchers and clinicians.

[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 Manufacturers of Therapeutic Cell and Gene Products (ATTC) has developed the Manufacturing and Preparation Toolkit to provide a practical guide for manufacturing ATMPs. The toolkit covers all aspects of ATMP manufacturing and preparation.

[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 therapies. The project aims to develop a platform for the development of gene therapies that can be used to develop a wide range of gene therapies.

[NCATS Resources](#)

**Restora Project**  
 The Restora project develops technologies that will enable the development of gene therapies. The project aims to develop a platform for the development of gene therapies that can be used to develop a wide range of gene therapies.

[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/ATMP 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](#)

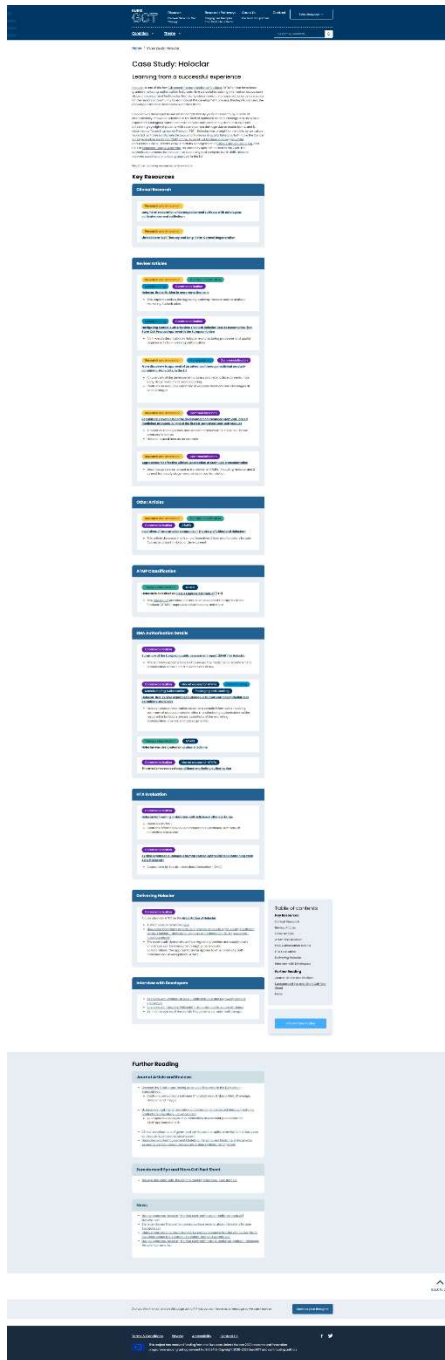
[Terms & Conditions](#) [Privacy](#) [Accessibility](#) [Contact Us](#)



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, 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 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 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** (Event)
- 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.

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.