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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

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

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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.
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 marketable, incentives, resources 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
Human body elements for therapeutic applications (HBE) human body elements, including tissues, cells, blood and stem cell preparations for use in cellular therapies programs.
Theme: Therapeutic Classification. Show all >

Advertising
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: Holozeron
EBDC EBDC
Discover our Holozeron case study for one of the first ATMPs that have been granted marketing authorisation in Europe. Discover more information about each stage of development for Holozeron from drug target selection to approval for the research community to begin clinical development programs.
Theme: Research and Innovation, Fundamental Research. Show all >

Specificities of the marketing authorisation dossier for biopharmaceuticals
EBDC EBDC
This document provides information on specificities of marketing authorisation (MA) dossiers for biopharmaceutical products (ATMPs) at dossier level compared to the existing reference marketing products which are not for medicinal uses. The document provides information on specificities of the MA dossier for biopharmaceutical products in terms of quality and clinical aspects.
Theme: Communication, Market access for ATMPs. Show all >

The EBMT/ISCT CAR-T Cell Handbook
EBDC EBDC
This open access CAR-T cell handbook published by EBMT and ISCT is jointly owned 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, access and manufacturing issues.
Theme: Fundamental Research, Manufacturing. Show all >

Conditional Marketing Authorisation
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 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.
Theme: Communication, Market access for ATMPs. Show all >

Data sharing / Open data
EBDC EBDC
Data sharing is a means of providing data access to data from other studies in data use for a defined purpose and/or to applicable national, European, legal or organisational requirements. Open data refers to making available research-related publications without restrictions regarding the access.
Theme: Data sharing / Open Data, Ethics. Show all >

Accelerated Assessment
EBDC EBDC
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 to market for medicinal products and to facilitate their availability. It is applicable to medicinal products that are innovative and have a high potential for medical progress. The use of accelerated assessment, in particular from the viewpoint of Therapeutics, Innovation.
Theme: Communication, Market access for ATMPs. 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/101019719/2020-2023/EBDC 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 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 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 web browser displaying the EuroGCT website. The page title is "Conditional Marketing Authorisation". The content is organized into several sections:

- Introduction:** Discusses the importance of conditional marketing authorisation (CMA) in accelerating the availability of innovative gene and cell therapies to patients.
- Stakeholders:** Identifies key stakeholders involved in the CMA process, including regulators, sponsors, and patients.
- Challenges:** Outlines the challenges associated with CMA, such as data requirements and regulatory uncertainty.
- Opportunities and Incentives:** Explores the opportunities and incentives provided by CMA to sponsors.
- Interactions with regulators:** Details the interactions between sponsors and regulators throughout the CMA process.
- Practical steps:** Provides a step-by-step guide to the CMA process.
- Table of contents:** A sidebar on the right lists the sections of the document for easy navigation.

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 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 Medicine, which is a 2025 project to support the development of Gene and Cell Therapy (GCT) products.

[ARM Resources](#)

ADVI ATMP Roadmap
 The Association of European Regenerative Medicine (AERM) has developed an ATMP roadmap, which will assist the regulatory process for ATMPs. The roadmap is based on the current regulatory framework for patient treatment, including Gene and Cell Therapy (GCT) products and other related resources developed by AERM.

[ADVI Resources](#)

EATRIS Transmed Academy and ADVANCE Project
 EATRIS is a network of medicines agencies and other stakeholders, which is focused on the development of ATMPs. The ADVANCE project is a collaborative effort to support the development of ATMPs.

[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 expert guidance on manufacturing ATMPs. The toolkit covers all aspects of ATMP manufacturing and preparation.

[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 therapies. The project will focus on the development of gene therapies for the treatment of rare diseases.

[NCATS Resources](#)

Restora Project
 The Restora project develops technologies that will enable the development of gene and cell therapies. The project is focused on the development of gene therapies 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/ATMP 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](#)

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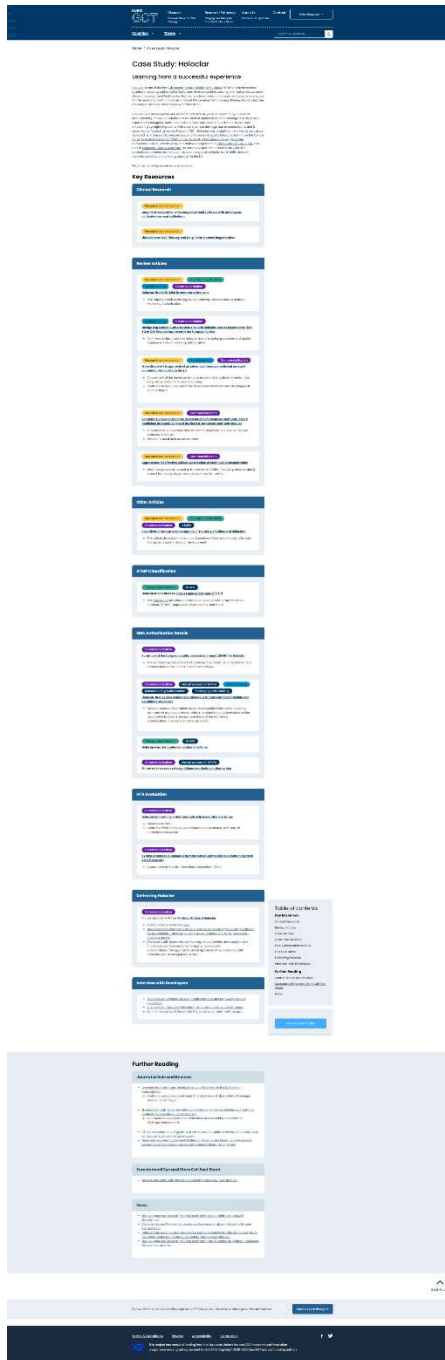
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Screenshot of 'Case study' section: www.eurogct.org/case-studies 12:41, 26 July, 2023

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.

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 shows the ATMP Engage PPI Directory website. The header includes the EURO GCT logo and navigation links. The main content area features a search bar and a list of search filters on the left, including PPI Themes, Research Field, Organisation Type, Development Stage, Audience, Country, and Content Type. The search results are displayed in a grid format, showing several articles such as 'ATMP PPI Articles', 'Patient Engagement Training and Resources Repository', 'Patient Engagement Resources', 'PCORI Events', and 'Engagement in health research literature explorer'. Each result includes a 'View this Resource' button and a brief description. The footer contains terms and conditions, a funding acknowledgment from the European Union, and social media icons.

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.