ADVANCE

Next Generation of Advanced Therapies' Specialists

Launch of ADVANCE Online course

December 1, 2020

JOIN THE COURSE

Welcome to the ADVANCE ATMPs Development Online Course! Through this course, you will gain scientific knowledge and understand the current challenges facing ATMPs development

This online course is divided into 4 Units representing the key challenges of the ATMPs development cycle

Scientific unit

Gain better understanding of the different classes of Advanced Therapies (gene therapy, cell therapy, and tissue engineering)

Manufacturing unit

Understand what sets the manufacturing of ATMPs apart from all other medicines

Regulatory unit

Discover which EU regulations to pay attention to and know the regulatory timeline for ATMP development

Pricing & Reimbursement unit

Learn about the importance of Health Technology Assessment (HTA) and the role of Patient Organisations

Self-paced **Free of charge**

eatris.eu/projects/advance/ **@ADVANCE_atmp**

Co-funded by the Erasmus+ Programme of the European Union

ADVANCE

Next Generation of Advanced Therapies' Specialists

ADVANCE Online course

The course consists of 4 Units corresponding to the 4 key challenges in ATMP Development

Unit 1: Scientific

The Scientific unit will explain the **different classes of Advanced Therapies**, through examples of gene therapy, cell therapy, and tissue engineering. The scientific unit is divided into 5 expert presentations that discuss cell therapies such as DCs and MSCs, and gene modified cell therapies such as CAR-Ts. It will also tackles the areas of **gene therapy approaches, vector biology and the cutting-edge technology behind gene editing** such as CRISPR-CAS which won the most recent Nobel prize for chemistry. The development of cell and gene therapy products will also be addressed together with the importance of **innovative preclinical models, multimodal imaging techniques, organoids and 3-D cell models** all which are important enabling technologies and tools to ascertain the potential therapeutic benefits of an ATMP in development and **ensuring the safety** of these products. Finally, this unit will tell the story of the **first market approved stem cell product in Europe** for a regenerative medicine for vision loss. This section will describe the development steps of an advanced therapy from research to commercialization. This will be an opportunity to better understand the core scientific approaches of advanced therapies and how they can be used to treat diseases or injuries, such as skin in burns victims, Alzheimer's, and cancer or muscular dystrophy, and can have **huge potential for the future of medicine and our patients**.

Co-funded by the Erasmus+ Programme of the European Union

ADVANCE
Next Generation of Advanced Therapies' Specialists

ADVANCE Online course

The course consists of 4 Units corresponding to the 4 key challenges in ATMP Development

Unit 2: Manufacturing

The Cutting-edge science described in the previous unit is only the start of the journey of an ATMP to the patients that really need them. For a therapy to be **available at the clinic** it must be manufactured to scale ensuring **safety** and **potency** are maintained. Innovative manufacturing requirements are necessary to **overcome the many technical difficulties** (e.g. product characterisation, biological safety, quality assurance, process scale) related to Good Manufacturing Practice or simply termed, GMP.The Manufacturing and Quality Control unit, will gives an overview of what goes into **manufacturing autologous** and **allogenic cell therapies**, what types of environments are needed, how scale-up works, and really understand what sets the manufacturing of ATMPs apart from all other medicines – the **challenges are unique**! This unit will also discuss the important field of IPSCs and how **quality** and **standards** in their manufacturing is key to their realization as an **effective therapeutic**. As most ATMPs are first manufactured in culture dishes at the start of their development journey, this unit aims to provide a greater understanding of the many challenges on the road to making a **new ATMP available** to all that need it through continuing advances in manufacturing, while following the **strict guidelines** set in place to ensure the safety of the patients that use these products

Co-funded by the Erasmus+ Programme of the European Union

ADVANCE

Next Generation of Advanced Therapies' Specialists

ADVANCE Online course

The course consists of 4 Units corresponding to the 4 key challenges in ATMP Development

Unit 3: Regulatory

Typically, academics are unaware of the need to develop a **regulatory strategy** from the start of the development pathway. The **European Medicines Agency** offers it expertise and guidance in the regulatory unit to explain all the legislation that applies to ATMPs. Did you know that some ATMPs are GMOs and that an ERA is an integral part of the MAA? Want to know what all of these acronyms stand for? Dive into the regulatory unit and get the confidence of knowing which **EU regulations** to pay attention to and know the **regulatory timeline** for ATMP development. The EMA will walk you through the EU's Regulation on advanced therapies which is designed to ensure the **free movement** of advanced therapy products within Europe, to **facilitate access** to the EU market, and to **foster the competitiveness** of European companies in the field, while guaranteeing the **highest level of health protection for patients.**

Co-funded by the Erasmus+ Programme of the European Union

ADVANCE
Next Generation of Advanced Therapies' Specialists

ADVANCE Online course

The course consists of 4 Units corresponding to the 4 key challenges in ATMP Development

Unit 4: Pricing & Reimbursement

The previous 3 units highlighted the **great promise of ATMPs and their great complexity**. Scientific, manufacturing and regulatory challenges and hurdles that must be overcome to get a product to the clinic for patient use have been expained. With this complexity however comes obvious expense and a **high price tag** that today remains a major obstacle in getting these often-curative therapies to the patients where standard available therapies have failed. How do we put a price tag on the **benefits of a novel therapy**? How do you price something where there is **no comparator?** How can a pharmaceutical company charge for a **therapy that's only used once**? We still are far from answering these questions but in this unit we will look at some of the tools used to **assess the future benefit** of an ATMP including **Health technology Assessment** (HTA) which is a is a key process to ascertain the potential value of an ATMP to the patient and society. Get a perspective of the patient on this issue through a **patient organization** where cell and gene therapies hold the **best promise** to treat an untreatable disease to date and where ensuring availability of novel ATMP products to the patient is **not hindered by issues of reimbursement**.

Co-funded by the Erasmus+ Programme of the European Union