



ADVANCED THERAPIES  
**TRANSFORMING  
MEDICINE**

# What are Advanced Therapy Medicinal Products?

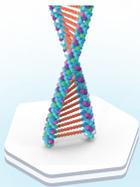


Advanced therapies offer patients new hope against a range of devastating illnesses, such as inherited diseases, leukaemia, blindness, Parkinson's disease, epilepsy and many others.

Advanced Therapy Medicinal Products are a new generation of innovative medicines based on genes, cells or tissues. Advanced therapies have ground-breaking therapeutic potential, particularly in disease areas where treatment options are absent or inadequate. Excitingly, these therapies are

starting to allow us to cure challenging conditions with a one-off treatment. As a result, they also have transformative implications for families, society and healthcare systems.

There are three types of advanced therapies:



Gene therapies



Cell therapies



Tissue-based therapies

**Gene therapy** alleviates the disease root cause or symptoms by replacing a malfunctioning gene or introducing a novel gene-based approach to

help the patient return to good health. Gene therapies hold great potential for treating, preventing or curing a wide range of inherited conditions.

**Cell therapy** involves transforming cells in order to fight disease. Cells are adapted before being introduced into the patient's body where

they target and treat diseased cells. The cells can be sourced from the patient's own body or from a healthy donor.

**Tissue-based therapies** seek to restore or replace damaged parts of the body through the combination of cells and active molecules. This aims to normalise the

damaged cells' structure as much as possible. Such therapies may allow a tissue or organ to develop and grow inside the patient.



Where standard medical and surgical practice have not proved effective in curing or treating genetic diseases, advanced therapies emerge as a promising option for a potentially lifelong cure.

## What advanced therapies are currently helping patients in the EU?

Here are some examples:

- Patients with skin cancer and acute forms of blood cancer, such as leukaemia and lymphoma, are being treated by therapies which detect cancer cells and trigger the body's immune system to attack them.
- Children with a rare inherited condition, causing their immune system to fail, are being treated by a therapy made from their own bone marrow.
- Patients who have been blinded through injury are having their sight restored by an innovative treatment using their own stem cells.



Since 2007 when the EU began to regulate advanced therapies, 14 advanced therapies have received marketing authorisation.

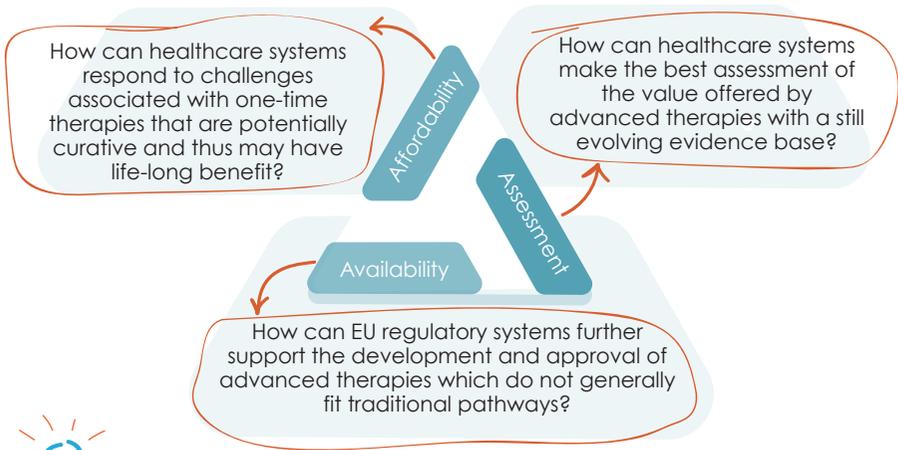


Advanced therapies that address the root cause of the disease with a one-time treatment can result in lower long-term costs for healthcare systems, compared to conventional treatments used for weeks, months, or even for life.

## What are the challenges for the use of advanced therapies?

Advanced therapies represent a novel category of treatments and are often highly complex to develop and deliver. To fully maximise their potential,

healthcare systems will need to adapt to ensure that patients across the EU can fully benefit. Advanced therapies pose new questions in three areas:



EuropaBio invites EU decision-makers to engage with us and our healthcare biotech members who are leading the field in advanced therapies.

We offer unparalleled knowledge and first-hand insight into the full range of issues relating to the pathway of innovative treatments from bench to bedside.