

What are cell and gene therapies?

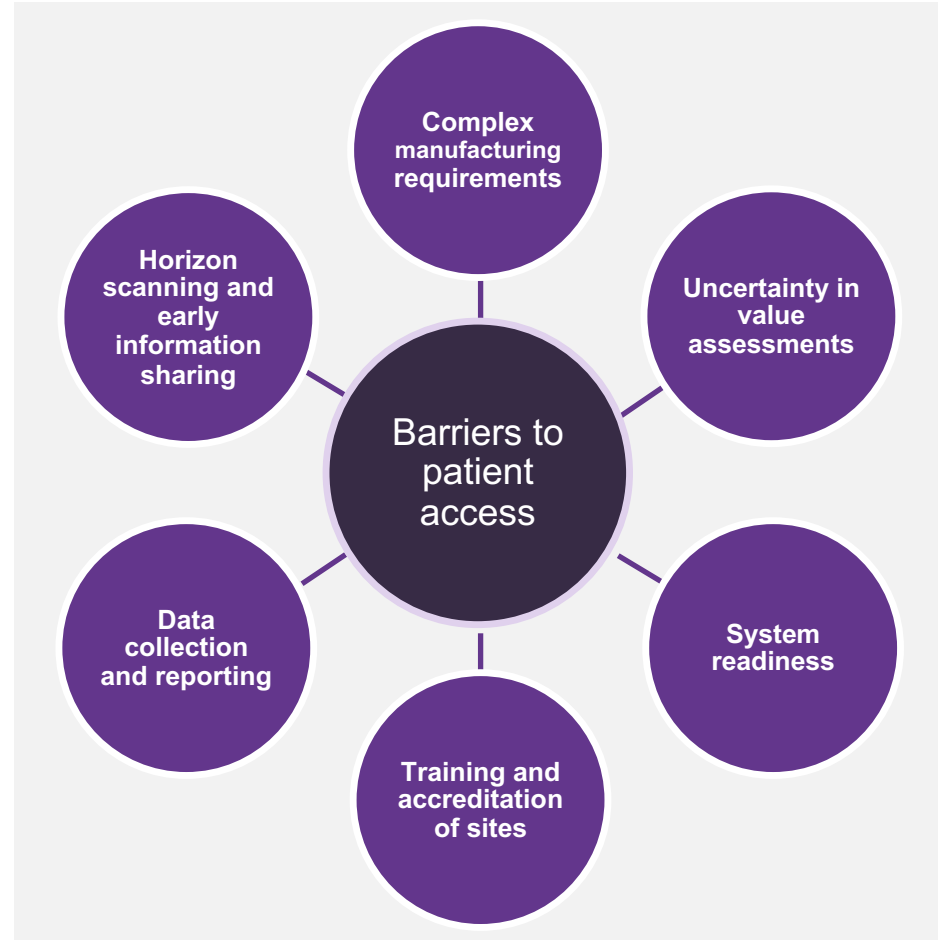
Cell and gene therapies, also known as advanced therapy medicinal products (ATMPs), are a class of potentially transformative products with the ability to provide long-term benefits for certain patients with debilitating or life-shortening diseases.¹ They are different from other therapies because they use genes, cells or tissues to fight the underlying cause of disease.²

The number of ATMPs coming to market in the coming years is expected to rise significantly.¹ However, as a result, they are likely to pose a range of challenges to the health system.

The UK is currently a world leader in the provision of these therapies having made a number available to patients to date.^{3,4}

The case for a national vision to support clinical adoption

To retain its early leadership and position as a world leader in the delivery of these treatments, the UK will need to adapt to ensure it is ready to accommodate the increase in cell and gene therapies being brought to market and to overcome the known barriers to expand patient access.



Recommended actions for a National Vision to deliver

Horizon scanning

Enhance horizon scanning processes to inform commercial discussions and support service planning

Manufacturing

Outline how the UK will further develop its domestic ATMP manufacturing capability

Clinical trials

Invest in the UK clinical trials environment so that trials can be approved and set up more quickly, including an increased focus on patient enrolment

Health technology assessment (HTA)

Undertake a review of the challenges that ATMPs pose to HTA bodies and make recommendations for reform

NHS workforce

Set out the steps that are required to improve awareness of ATMPs across the workforce, as well as the training and educational requirements to deliver them

Reimbursement

Explore alternative approaches to paying for ATMPs to ensure payer sustainability and value for money

Ongoing patient care and support

Outline how centres will be supported to provide ongoing patient care, support and monitoring

Health service capacity

Commit to increase capacity within existing centres and support the creation of new centres

Data collection

Provide the NHS a mandate to develop its data infrastructure to inform continued improvements in care

Collaboration

The success of any vision will be dependent on every stakeholder playing their part and working collaboratively. This must include Government, HTA bodies, payers, regulators, treatment centres, industry and patient groups. We welcome the commitment from the Rare Diseases Action Plan to publish a 'strategic approach' to ATMPs and we call on all parties to contribute to its development. This will be vital to understand the initiatives already underway and to prioritise action in areas of remaining unmet need.

- 1 [Medicine Manufacturing Industry Partnership \(2016\) Advanced Therapies Manufacturing Action Plan](#)
- 2 [European Medicines Agency \(2016\) Advanced therapy medicinal products: Overview](#)
- 3 [NHS England \(2018\) NHS England strikes deal for cancer treatment in a European first](#)
- 4 [NICE \(2021\) NHS England strikes deal on gene-therapy drug that can help babies with rare genetic disease move and walk](#)