CERSI-AT
Centre of Excellence in Regulatory Science and Innovation for Advanced Therapies

Updates from CERSI-AT – Edition one, July 2025

Announced on 10 February 2025, Cell and Gene Therapy Catapult and Birmingham Health Partners Centre for Regulatory Science and Innovation (CRSI) established the Centre of Excellence in Regulatory Science and Innovation for Advanced Therapies (CERSI-AT), a virtual network which drives innovation and scientific research to enhance ATMP regulatory decision-making. Through comprehensive stakeholder mapping, mobilised working groups, and detailed delivery plans, the centre implements pilot projects, analyses data, and disseminates findings via industry meetings, regulatory briefings, and white papers. By fostering collaboration between UK regulatory bodies, industry, researchers, and other stakeholders, the centre aims to streamline and improve ATMP regulatory processes, ensuring the UK has a world-leading regulatory environment that best supports ATMP innovation.

To effectively advance the UK's regulatory landscape for ATMPs, a multi-faceted approach is essential. This includes actively collating industry challenges and feedback to inform regulatory agency decision-making, while simultaneously strengthening relationships with these agencies to provide dependable and long-term support. The formation of relevant consortia and strategic partnerships further bolsters collaborative efforts. Creating a safe clinical "sandbox" setting facilitates the testing and development of innovative policy. To ensure widespread impact, we enable knowledge dissemination through training materials, workshops, exemplars, white papers and FAQs for educating stakeholders. Furthermore, supporting the identification and implementation of new UK regulatory standards, combined with the review and validation of international standards through pilot projects, will ensure the UK remains at the forefront of ATMP regulation.

CERSI-AT's Clusters

CERSI-AT has identified numerous regulatory science and innovation projects which it pools in clusters. Clusters and their projects have been prioritised based on their potential impact, feasibility, sustainability, cost-effectiveness and ambition to maximise resource utilisation for the centre. Each cluster is led by a qualified leader from the Cell and Gene Therapy Catapult or the University of Birmingham who ensures that the project teams can work through their delivery plans at pace and with all possible support. It is intended for the centre to add additional projects to existing clusters and form new cluster should it be required. The current clusters are:

Cluster 1 Streamlined Regulatory Processes	Work with UK regulators how to **optimise current regulatory processes** for ATMPs with the aim of timely adoption of safe and effective therapies
Cluster 2 Real World Evidence (RWE)	Create opportunities for the credible **collection and use of RWE** which is central to addressing ATMP data uncertainty at launch
Cluster 3 Patient-Reported Outcomes (PROs)	Assess the potential **use of PROs** to evaluate ATMP's impact on disease symptoms, adverse events, physical function, and patient's quality of life
Cluster 4 Clinical Development Acceleration and Optimisation	Help **optimise the UK clinical development landscape** and processes and enhance innovation and patient access to novel treatments with clear benefits
Cluster 5 Patient Engagement and Involvement (PPIE)	Identify and communicate areas of regulatory processes that would benefit from optimisation of how **patient voice** is accounted for
Cluster 6 Reference Standards	Work with MHRA's Soth Mimms science campus to develop standards to **support analytical development and validation** for ATMPs

CERSI-AT Cluster Overview

Quarter 1 Readout

Setup Phase

We have been focussed on the overall programme set-up and the building of the project teams for all clusters. In addition we have built our advisory board which draws on relevant stakeholders.

A CERSI-AT webpage has been created to serve as a central platform to house future centre outputs, e.g. newsletters, whitepaper and recordings.

Decentralised Manufacturing

Representatives from the Centre attended and contributed to the MHRA's Decentralised Manufacturing (DM) Workshop on 12 March 2025. The event focused on refining draft MHRA guidance for Manufacturing Authorisation, GxP inspections, clinical trials, Marketing Authorisation, and pharmacovigilance, specifically for DM models.

Key takeaways included:

- QPs will have expanded responsibility for batch release across multiple decentralised sites in hub-and-spoke models.
- Ongoing monitoring is crucial to ensure consistent product quality across sites with varying batch frequencies.
- The inherent variability of biological starting materials and limited batch sizes in ATMPs pose manufacturing consistency challenges.
- Ensuring adequately trained personnel at all remote sites with central hub oversight is essential.
- Robust communication between hub and spoke sites is vital for timely reporting of key information.

The workshop also addressed QC considerations, noting potential shifts towards Real-Time Release Testing for point-of-care products. The guidance documents are now being finalised by MHRA and will be published on their Decentralised manufacture hub.

GTMP Classification Workshop

CGT Catapult and the MHRA recently co-hosted a workshop to discuss potential updates to the UK's regulatory definitions of Advanced Therapy Medicinal Products (ATMPs). The discussions centred on how these definitions impact patients and stakeholders across the industry and clinical practice.

Currently, ATMP classification is closely tied to a product being biological, with specific considerations for gene therapies based on their mechanism of action (regulating, repairing, replacing, adding, or deleting genetic sequences, excluding vaccines). However, novel medicines are increasingly blurring the lines between traditional biological and chemical categories. These synthetically produced substances, functionally similar to existing ATMPs, may not currently qualify as such under the biological product criteria. This could lead to inconsistencies in classification based solely on manufacturing technology. Furthermore, ambiguities remain in the current definition of gene therapy, potentially excluding therapies like genome-editing proteins.

A position paper summarising the workshop's outcomes is now in development.

Highlighting the UK's Competitive Advantage

In support of CERSI-AT's mission to advance the UK's regulatory competitiveness in ATMPs. As part of the network, Cell and Gene Therapy Catapult have issued a white paper titled "Review of UK competitive advantage from a regulatory and market access perspective" 23 April 2025. Within the paper, CGT Catapult's experts offer a detailed analysis of the UK's regulatory and market access environment for ATMP development, emphasising key areas where the UK differentiates favourably from other regions and highlighting to developers that the UK can serve as an attractive location for developing and commercialising ATMPs.

Upcoming Events

Join us 02 October 2025 at the Royal Society, 6-9 Carlton House Terrace, London SW1Y 5AG, for symposium investigating the feasibility and appetite for a non-clinical data repository. The panel will be joined by the MHRA and the UK Centre of Excellence on In-Silico Regulatory Science and Innovation (UK CEiRSI).

For More

For more updates, including the EU and US, please visit the Cell and Gene Therapy Catapult's monthly Regulatory Round Up.