Regulatory Round-up

September 2025

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UNITED KINGDOM

Medicines and Healthcare products Regulatory Agency (MHRA)

Decentralised manufacturing: emerging considerations

The MHRA has created a blog post on emerging considerations regarding Decentralised Manufacturing, which covers the two elements of the new manufacturing and supply framework: point of care and modular manufacturing. The aim of the MHRA blog is to provide an outline of some of the new requirements and key thoughts now that this new framework is at the start of the implementation phase. The blog can be found here.

MHRA launches Route B notification pilot as part of clinical trials regulations rollout

On 1st October 2025, the MHRA launched the Route B pilot to help sponsors prepare for a new substantial modifications process under upcoming regulations, with responses delivered within 14 days. The pilot expands the MHRA's risk-proportionate approach to include the review of modifications to approved clinical trial applications. Under the new regulations, substantial modifications can be granted automatic approval through the Route B substantial modification process, providing they meet eligibility criteria.

Please find further information here. Additionally, more information is provided on the MHRA inspectorate blog here.

EUROPE

European Commission (EC)

New targets for clinical trials in Europe

The EC, the Heads of Medicines Agencies (HMA) and European Medicines Agency (EMA) have jointly developed two new targets for clinical trials, to monitor progress against the ambition to make the European Union (EU) a more attractive destination for clinical research and improve timely access to innovative medicines for patients. In five years, the aim is that:

- An additional 500 multinational clinical trials are added to the current average of 900 that are already authorised each year (i.e. an estimated 100 per year).
- Two thirds (66%) of clinical trials should begin recruiting patients within 200 calendar days or less from the date of application submission. This is in comparison to only 50% of clinical trials today.

These ambitious goals build on ongoing efforts to create a more supportive environment for clinical research. A key part of this is the Accelerating Clinical Trials in the EU (ACT EU) initiative, a collaboration between EC, HMA and EMA, which seeks to optimise how clinical trials are designed and run. Please find further information here.

USA

Food and Drug Administration (FDA)

Draft guidance: Safety labeling changes – Implementation of Section 505(0)(4) of the Federal Food, Drug, and Cosmetic (FD&C) Act

The FDA published a draft guidance for industry entitled "Safety Labeling Changes—Implementation of Section 505(o)(4) of the Federal Food, Drug, and Cosmetic Act." This draft guidance provides information on the implementation of the statutory provision that authorises the FDA to require application holders for certain drug and biological products to make labeling changes based on new safety information that becomes available after approval of the drug, as determined by the FDA, should be included in the labelling of the drug. Please find further information here.

Deadline for comments is 18th November 2025.

Three new draft guidance documents for cell and gene therapy products

The FDA published three new draft guidance documents aimed at cell and gene therapy products:

1. Expedited Programs for Regenerative Medicine Therapies for Serious Conditions. This guidance provides sponsors engaged in the development of regenerative medicine therapies for serious or life-threatening diseases or conditions with our recommendations on the expedited development and review of these therapies, including as provided under section 506(g) of the FD&C Act, as added by section 3033 of the 21st Century Cures Act (Cures Act). This guidance provides:

- Description of the expedited programs available to sponsors of regenerative medicine therapies for serious conditions, including those products designated as regenerative medicine advanced therapies (RMATs).
- Information about the provisions in the Cures Act regarding the use of the accelerated approval pathway for regenerative medicine therapies that have been granted designation as an RMAT.
- Considerations in the clinical development of regenerative medicine therapies and opportunities for sponsors of such products to interact with the Center for Biologics Evaluation and Research (CBER) review staff.

2. Post approval methods to capture safety and efficacy data for cell and gene therapy products.

This guidance discusses methods and approaches for capturing post-approval safety and efficacy data for cell and gene therapy products. Note that this guidance does not address data collected for the purpose of expanding clinical indications.

3. Innovative designs for clinical trials of cellular and gene therapy products in small populations.

This guidance provides recommendations to sponsors who are planning clinical trials of cell and gene therapy products intended for use in a disease or condition that affects a small population—generally one that meets the definition of a rare disease or condition under section 526(a)(2) of the FD&C Act (21 U.S.C. 360bb(a)(2)). It describes FDA requirements and provides considerations for the use of various clinical trial designs and endpoints to generate clinical evidence to support product licensure. This guidance expands on principles described in FDA's existing guidance documents related to this topic, by providing additional recommendations for the planning, design, conduct, and analysis of cell and gene therapy trials to facilitate FDA's assessment of product effectiveness.

FDA CBER Office of Therapeutic Products (OTP) public listening meeting: Leveraging knowledge for facilitating the development and review of cell and gene therapies

The FDA CBER OTP hosted a virtual public listening meeting to solicit perspectives from cell and gene therapy manufacturers and other stakeholders on leveraging prior knowledge and experience to facilitate product development and application review. During the meeting, speakers shared how internal prior and public knowledge can be leveraged to help advance development and regulation of CGT products. Recording of the meeting can be found here.

Public consultations

Medicines and Healthcare Products Regulatory Agency (MHRA)

	Title	Consultation Period	Category
1.	*ICH E20 guideline on adaptive designs for clinical trials*	*End date: 30 November 2025*	*Public Consultation*

European Commission (EC)

	Title	Consultation Period	Category
1.	*EudraLex Volume 4 - Good Manufacturing Practice Guidelines: Chapter 4, Annex 11 and New Annex 22*	*End Date: 07 October 2025*	*Stakeholder Consultation*

European Medicines Agency (EMA)

	Title	Consultation Period	Category
1.	*ICH Q3E Guideline for extractables and leachables*	*End date: 18 December 2025*	*Public consultation*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Approaches to Assessment of Overall Survival in Oncology Clinical Trials*	*End date: 20 October 2025*	*Draft guidance*
2.	*Safety Labeling Changes – Implementation of Section 505(0)(4) of the FD&C Act.*	*End date: 18 November 2025*	*Draft guidance*
3	*Expedited Programs for Regenerative Medicine Therapies for Serious Conditions*	*End date: 24 November 2025*	*Draft guidance*
4	*Postapproval Methods to Capture Safety and Efficacy Data for Cell and Gene Therapy Products*	*End date: 24 November 2025*	*Draft guidance*
5.	*Innovative Designs for Clinical Trials of Cellular and Gene Therapy Products in Small Populations*	*End date: 24 November 2025*	*Draft guidance*