Regulatory Round-up

October 2025

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UNITED KINGDOM

Medicines and Healthcare products Regulatory Agency (MHRA)

The new Innovative Licensing and Access Pathway welcomes first investigational products

The MHRA has announced that the UK's updated Innovative Licensing and Access Pathway (ILAP) has welcomed its first three investigational therapies, including treatments for a fatal infant metabolic disorder, Duchenne muscular dystrophy, and a rare neurodegenerative disease. These therapies received 'Innovation Passports', granting early, coordinated support from the NHS, MHRA, and health technology bodies to accelerate development and access. ILAP uniquely unites regulators, healthcare providers, and assessors from the start, focusing on transformative treatments for unmet clinical needs—especially rare diseases. Future availability depends on proven safety, effectiveness, and value.

Developers interested in ILAP can now apply for the next round. Full guidance is available on the MHRA website.

MHRA and NICE invite early adopters to trial accelerated aligned pathway – six months ahead of schedule

The MHRA and NICE have launched early access to the Aligned Pathway, allowing pharmaceutical companies to apply six months ahead of schedule. This streamlined process combines licensing and value assessments, enabling simultaneous decisions and reducing delays in patient access to new medicines. A joint scientific advice service, set to launch by April 2026, will further support companies by offering coordinated guidance. The initiative supports UK health and life sciences strategies, aiming to boost efficiency, innovation, and faster NHS access to promising treatments. Pharmaceutical companies are encouraged to register products on UK PharmaScan at least three years before marketing authorisation and to engage early with both organisations. Please find further information here.

New British Pharmacopoeia (BP) Guidance: Best Practice for Replication Competent Virus (RCV) Testing

The BP has published best practice guidance for RCV testing, an essential safety step when using viral vectors in ATMPs. Developed with input from MHRA, NHS, industry, and academic experts, this guidance provides practical, non-mandatory advice tailored to the unique risks posed by different viral vectors.

What's included:

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- Guidance on testing for replication competency in AAV and Lentivirus vectors
- A harmonised framework aligned with existing standards
- Practical insights from across the sector to help you build robust, fit-for-purpose assays

The guidance is available to download here.

100 years of protecting patients through biological standards for medicines

On World Standards Day (14 October), the MHRA celebrated 100 years of biological standards, which ensure the safety, effectiveness, and consistency of medicines and vaccines globally. MHRA experts develop and distribute these standards—supporting scientific progress and healthcare worldwide. Based at its science campus, the MHRA supplies over 95% of WHO's biological standards, delivering more than 110,000 units last year to 1,500 organisations across 81 countries. The MHRA's biological standards play a vital role in ensuring the safe development, testing, and use of medicines, vaccines, and diagnostic tools as well as enable scientific findings to be reliably compared, replicated, and expanded upon.

Please find further information here.

Patients to benefit as UK and US regulators forge new collaboration on medical technologies and AI

The UK's MHRA is deepening collaboration with the US FDA to accelerate medical technology innovation, improve patient safety, and reduce barriers to market access. Key initiatives include:

- Launch of the MHRA National AI Commission with UK and US experts to guide safe, transparent use of AI in healthcare.
- New international reliance routes allowing faster UK access to FDA-approved medical devices.
- Joint efforts to align regulatory processes and support earlier patient access to innovative technologies.
- Planned medtech reforms in Great Britain, entering legislation in 2026, aim to enhance global competitiveness and streamline approvals from 2027.

Please find further information here.

EUROPE

European Medicines Agency (EMA)

Reflection paper on patient experience data

The EMA has released a draft reflection paper for public consultation on patient experience data—information directly from patients about their treatment experiences and preferences. This data offers valuable insights beyond clinical outcomes and can

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complement traditional evidence in medicine development and regulation. The paper encourages developers to integrate patient perspectives throughout a medicine's lifecycle and outlines general principles for collecting and analysing such data. It also highlights sources like clinical trials, real-world data, mobile health tech, and social media. Stakeholder feedback will help shape future EU strategies to better align treatments with what matters most to patients.

This draft reflection paper is open for public consultation until 31 January 2026. Please find further information here.

Guideline on quality aspects of phage therapy medicinal products

EMA has issued a draft guideline which outlines regulatory expectations for the development, manufacturing, and quality control of PTMPs. These products are classified as biological medicinal products under EU law but require special considerations due to their unique properties (e.g. specificity, self-replication, evolutionary potential).

Key points:

- Applies to strictly lytic bacteriophages, including modified and synthetic types.
- Excludes cell-free production systems, gene therapy-classified phages, and patient-specific or magistral preparations.
- Follows the eCTD framework for regulatory submissions.
- Encourages early consultation with the Committee for Advanced Therapies (CAT) for classification issues.

The guideline supports safe and effective development of phage-based treatments for bacterial infections in humans.

The draft guideline is open for consultation until 30 April 2026.

USA

Food and Drug Administration (FDA)

Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments

The FDA has released its final guidance in the Patient-Focused Drug Development (PFDD) series, titled *"Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments."* This document provides recommendations for stakeholders—including patients, researchers, and developers—on how to incorporate patient experience and caregiver input into clinical outcome assessments for drug development and regulatory decisions. It finalizes the draft version issued on June 30, 2022, and is the third of four planned PFDD methodological guidance documents.

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Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers

The FDA issued a guidance document to clarify regulations on expanded access to investigational drugs for treatment use under an investigational new drug application (21 CFR part 312, subpart I), effective since October 13, 2009. In response to frequent questions from stakeholders—including industry, researchers, and patients—the FDA released a Q&A-style guidance in June 2016 (updated in October 2017) to address common concerns. Since then, additional questions have emerged, especially regarding new provisions introduced by the 21st Century Cures Act and the FDA Reauthorization Act of 2017. The guidance document is available to download here.

FDA approves labeling changes that include a Boxed Warning for Immune Effector Cell-associated Enterocolitis following treatment with Ciltacabtagene Autoleucel (CARVYKTI)

The FDA has received reports of immune effector cell-associated enterocolitis (IEC-EC) in patients treated with CARVYKTI, based on clinical trials and postmarketing data.

As a result, the FDA has updated CARVYKTI's Boxed Warning, Warnings and Precautions, and Adverse Reactions sections to reflect the risk of IEC-EC. Patients and clinical trial participants with IEC-EC should be managed according to the institutional guidelines including referral to gastroenterology and infectious disease specialists. In patients with treatment refractory IEC-EC, additional work up should be considered to rule out T cell lymphoma of the gastrointestinal tract which has been reported in patients with treatment refractory IEC-EC in the postmarketing setting.

Additionally, the FDA approved updates to the Clinical Studies section to include overall survival (OS) data from the CARTITUDE-4 trial. This study showed a statistically significant OS improvement in CARVYKTI-treated patients with relapsed and lenalidomide-refractory multiple myeloma.

Despite the risks, the FDA concludes that CARVYKTI's overall benefit—including improved survival—continues to outweigh its potential risks. Please find further information 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*

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European Medicines Agency (EMA)

	Title	Consultation Period	Category
1.	*ICH Q3E Guideline for extractables and leachables*	*End date: 18 December 2025*	*Public consultation*
2.	*Guideline on quality aspects of phage therapy medicinal products*	*End date: 30 Apr 2026*	*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*

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