Regulatory Round-up

April 2026

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United Kingdom

Medicines and Healthcare Products Regulatory Agency (MHRA)

Launch of clinical trial reforms

The new UK clinical trial regulations, which came into force on 28 April 2026, represent the most significant reform of the framework in over 20 years. Introduced by the MHRA and Health Research Authority (HRA), the changes establish faster, risk-proportionate approval routes, including notifiable trials and the legally mandated Route B modification pathway, while maintaining high safety standards. The reforms are expected to further reduce trial set-up times and strengthen the UK's position as a leading environment for clinical research. Please find further information here.

UK and US deepen regulatory cooperation on medical devices, building on wider pharmaceutical partnership

The UK and the US have announced steps to deepen regulatory cooperation on medical devices, strengthening collaboration between the MHRA and the Food and Drug Administration (FDA) to support faster access to safe and innovative technologies for patients in both countries. The initiative builds on the wider UK–US pharmaceutical partnership, aiming to reduce regulatory duplication for manufacturers while maintaining high safety and performance standards.

The two regulators will work more closely to explore greater alignment of medical device regulation, including potential future mutual recognition mechanisms that could streamline approval processes and support earlier patient access to new medical technologies. This cooperation reinforces the UK's position as a global leader in life sciences innovation while ensuring regulatory independence and robust patient safety protections are maintained. Please find further information here.

MHRA expands Artificial Intelligence (AI) Airlock programme with a £3.6 million funding boost over three years

The MHRA has announced a £3.6 million, three-year expansion of its AI Airlock programme, the UK's regulatory sandbox for AI as a medical device (AIaMD). As the UK's first regulatory sandbox for AIaMD, AI Airlock brings together regulators, the NHS and approved bodies to identify and address regulatory challenges early, helping high-quality AI technologies reach patients more quickly while maintaining robust safety standards. The funding will enable longer-term, real-world testing of innovative AI technologies, support faster patient access while maintain robust regulatory oversight and safety standards. Please find further information here.

Health Research Authority (HRA)

Protocol guidance and template for use in a Clinical Trial of an Investigational Medicinal Product. (CTIMP) from 28 April 2026

The protocol guidance and template for use in a clinical trial of investigational medicinal products (CTIMP) have been updated to reflect the Medicines for Human Use (Clinical Trials) (Amendment) Regulations 2025, which come into force in the UK on 28 April 2026.

Further updates have been made to align with the Data Protection Act 2018 and the UK General Data Protection Regulation (UK GDPR).

- protocol guidance and template for use in a CTIMP

The tool will undergo wider review and improvement over summer 2026, with a new version planned for release later this year. Please find further information here.

Commercial model clinical trial agreements update

The HRA has published commercial and non-commercial model agreements that are in use since 28th April 2026.These updates align the agreements with recent amendments to the UK clinical trials regulations and associated policy changes applying to both CTIMP and non-CTIMP studies. Please find further information including how to give feedback here.

Clinical trials regulations: Modification Tool has launched

The HRA has launched a new Modification Tool within the Integrated Research Application System to support implementation of the updated UK clinical trials regulations effective from 28 April 2026, replacing the former Amendment Tool and aligning with revised regulatory terminology and modification categories. The tool provides sponsors with clear guidance on modification classification and review pathways for both CTIMP and non-CTIMP studies and will become fully operational on the date the new regulations take effect. Please find further information here.

EUROPE

European Directorate for the Quality of Medicines (EDQM)

EDQM publishes guidelines to promote traceability of medicines in hospital settings

EDQM has published new guidelines on best practices for the traceability of medicines in hospital settings, aimed at strengthening patient safety and reducing medication errors. The guidance promotes improved traceability at the point of administration, including the use of digital and barcode-based systems, and supports harmonised approaches across Council of Europe member states to enhance safe and effective medicines use in hospitals. Please find further information here.

European Medicines Agency (EMA)

EMA recommendation for approval: Itvisma

The Committee for Medicinal Products for Human Use has recommended granting a marketing authorisation for Itvisma (onasemnogene abeparvovec), a gene therapy indicated for the treatment of 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the *SMN1* gene in patients 2 years of age and older. Onasemnogene abeparvovec is a non-replicating recombinant viral vector designed to introduce a functional copy of the survival motor neuron gene (*SMN1*) in the transduced cells to address the monogenic root cause of SMA. By providing an alternative source of SMN protein expression in motor neurons, it is expected to promote the survival and function of transduced motor neurons Please find further information here.

EMA consults on virtual control groups to help reduce animal use in medicines development

EMA has issued a draft qualification opinion on the use of virtual control groups as a new approach methodology to help reduce animal use in medicines development. The draft Committee for Medicinal Products for Human Use qualification opinion represents an important step in advancing the 3Rs principles, while ensuring that non--clinical safety assessments remain scientifically robust and protective of patient safety. By recognising the regulatory acceptability of virtual control groups within a defined context of use, EMA provides a clear framework to support innovation in preclinical research and sets a foundation for future adoption of alternative methodologies through evidence-based, internationally aligned implementation. The deadline for comments is 12 May 2026. Access to the draft qualification opinion and information on how to submit comments can be found here.

New pilot to support development of 'breakthrough' medical devices

EMA has announced a pilot programme launching in Q2 2026 to support the development of breakthrough medical devices and in vitro diagnostics. The initiative will provide manufacturers with enhanced regulatory support and priority scientific advice through EMA-administered medical device expert panels, with the aim of accelerating patient access to highly innovative technologies while maintaining robust safety and performance standards.

USA

Food and Drug Administration (FDA)

FDA approval: Otarmeni

On 23 April 2026, the FDA approved Otarmeni (lunsotogene parvec-cwha). This represents a significant regulatory milestone as it is the first *in vivo* gene therapy approved for genetic hearing loss, authorised under the Commissioner's National Priority Voucher pilot program. The therapy is indicated for paediatric and adult patients with severe-to-profound sensorineural hearing loss due to biallelic otoferlin (OTOF) variants, with approval based on Phase 1/2 CHORD study data and granted

under an accelerated approval pathway contingent on confirmatory evidence of durable clinical benefit, setting an important regulatory precedent for genotype-directed, disease-modifying approaches in rare neurosensory disorders.

FDA Issues Draft Guidance on Genome Editing Safety Standards to Advance Gene Therapy Development

The FDA's draft guidance issued on 14 April 2026 strengthens the regulatory framework for genome-editing gene therapies by outlining standardized, science-based expectations for non-clinical safety assessment, with a strong focus on the use of next-generation sequencing (NGS) to evaluate off-target editing and genomic integrity. The guidance supports Investigational New Drug (IND) and Biologics License Application (BLA) submissions for both ex vivo and in vivo products and aligns with the FDA's broader strategy to accelerate development of individualized and ultra-rare disease therapies while maintaining rigorous safety standards. The deadline for comments is 14th July 2026.

FDA Announces Major Steps to Implement Real-Time Clinical Trials

The FDA has announced major steps to advance the implementation of real-time clinical trials, including the successful initiation of proof-of-concept studies and the launch of a forthcoming pilot program. This initiative represents a significant modernization of the clinical trial framework, enabling the secure, real-time transmission of key safety and efficacy signals to regulators while studies are ongoing.

By leveraging advances in data science and artificial intelligence, real-time clinical trials have the potential to enhance patient safety oversight, reduce administrative lag, and support more timely regulatory decision-making, particularly in early-phase development. The FDA's collaboration with sponsors on these proof-of-concept trials demonstrates the technical feasibility of this approach and underscores a continued commitment to improving efficiency without compromising scientific rigor or ethical standards. Please find further information here.

INTERNATIONAL

International Conference on Harmonisation (ICH)

Training Module Issued for ICH Guideline M15 on Model-informed Drug Development

The ICH has issued a training module for Guideline *M15: General Principles for Model-Informed Drug Development (MIDD)*. The module provides timely and practical support to facilitate consistent understanding and implementation of the newly finalised M15 guideline across ICH regions. By outlining the key principles, scope and objectives of MIDD, and by explaining the harmonised framework for the assessment of MIDD evidence, model evaluation, and reporting expectations, the training module reinforces transparent, risk-based and scientifically robust use of modelling and simulation in regulatory decision-making. The availability of this training material represents an important step toward embedding MIDD as a routine and well-understood component of global drug development and regulatory review. Please find further information here.

Public consultations

Medicines and Healthcare products Regulatory Agency (MHRA)

	Title	*Consultation Period*	Category
1.	*Substances of human origin (SoHO): review of UK legislation*	*End date: 17 June 2026*	*Open call for evidence*

European Medicines Agency (EMA)

	Title	Consultation Period	Category
1.	*Guideline on non-inferiority and equivalence comparisons in clinical trials*	*End date: 31 May 2026*	*Draft guidance*
2.	*Concept paper on the development of a reflection paper on proof-of-concept data to support the development of anti-cancer medicinal products in paediatric patients*	*End date: 30 June 2026*	*Public consultation*
3.	*Draft Qualification opinion for Virtual Control Groups (VCG) to replace Concurrent Control Groups (CCG) in rat non-GLP Dose-Range Finding (DRF) studies*	*End date:12 May 2026*	*Draft document*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Responding to FDA Form 483 Observations at the Conclusion of a Drug CGMP Inspection*	*End date: 08 May 2026*	*Draft guidance*
2.	*Safety Assessment of Genome Editing in Human Gene Therapy Products Using Next-Generation Sequencing*	*End date: 14 July 2026*	*Draft guidance*