Regulatory Round-up

March 2026

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United Kingdom

Medicines and Healthcare products Regulatory Agency (MHRA)

Patients to get new medicines up to six months sooner under new joint MHRA-NICE approval process

The MHRA and NICE have launched a new aligned approval pathway designed to get patients in England access to some new medicines three to six months sooner by synchronizing decision-making on product licencing and value assessments.

Alongside the pathway, the MHRA and NICE are also launching an improved Integrated Scientific Advice service. It will offer a single-entry point, meeting and report, and one payment, while aligning data and scientific expectations where possible.

The pathway launches on 1 April 2026 and is part of commitments in the government's '10 Year Health Plan for England' and 'Life Sciences Sector Plan' for the MHRA and NICE to work together more closely to get medicines to patients sooner. Please find further information here.

Global impact of UK health data resource highlighted in newly published paper

A newly published analysis highlights the global impact of the UK's Clinical Practice Research Datalink (CPRD), which is run by the MHRA. It shows that the CPRD has supported nearly 3,800 peer-reviewed studies across 29 countries – with the UK, United States and Canada as leading contributors – and continues to play a major role in advancing medical research, patient safety, and health policy. Please find further information here.

UK sets out world-leading pathway for space-manufactured drugs

The UK has outlined a world-leading pathway for bringing space-manufactured drugs to patients. The UK Space Agency, supported by the MHRA, Regulatory Innovation Office within the Department for Science and other bodies, suggests that the unique environment of microgravity, impossible to replicate on Earth, can improve how biologic drugs form, behave and work within the human body and have the potential to improve outcomes for people with cancer, rare diseases and other conditions by enhancing medicine quality, stability and performance.

To support innovators directly, the UK Space Agency and Innovation Acclerator team at the MHRA are preparing a coordinated package of regulatory guidance, principles-based case studies and a regulatory sandbox that will outline clear regulatory routes

for space, biotech and pharmaceutical companies from in-orbit research to safe, effective use on Earth.

Building on the MHRA's experience in developing innovative and proportionate regulatory pathways, the Agency works closely with developers and partners to ensure that existing and future regulations remain fit for purpose for medicines manufactured using advanced and novel manufacturing approaches. This includes manufacturing that may take place in microgravity or other unique environments, where modular manufacturing and atypical distribution practices may occur. The MHRA encourages developers of space-manufactured drugs to engage with the Agency early so they can receive support in navigating regulatory expectations. Please find further information here.

MHRA approach to medicines using non-animal methods

The MHRA has published guidance that describes its approach to assessing applications for medicines that use alternative methods to replace the use of animal models in science.

The MHRA is committed to the 3Rs principles to replace, reduce and refine animal use in medicine development as part of the UK government policy to move towards phasing out animal testing. Advancements in non-animal-based testing methods make it possible to reduce reliance on animals in the development of medicines, however each application reviewed by the MHRA is considered individually.

Where it can be shown that deviating from expectations for *in vivo* studies in current ICH guidelines seems to pose no risk to human health of the specific clinical use proposed, the MHRA is open to considering proposals that omit animal studies, if alternative methods address safety. However, the MHRA will continue to accept applications that include animal studies in line with international guidelines in place at the time the studies in animals were done.

For certain advanced therapy medicinal products, animal models may not be scientifically relevant or predictive of human safety. In these cases, the MHRA accepts that *in vivo* animal studies may not contribute meaningfully to the assessment of risk.

This guidance applies to Clinical Trial Authodisation applications and Marketing Authorisation applications for medicinal products, and does not relate to animal use in quality control testing for batch release of products. Please find further information here.

DHSC is seeking evidence and stakeholder views on the UK's legislation for substances of human origin (SoHO)

The Department of Health and Social Care (DHSC) has announced an open call for evidence, seeking views on the UK's legislative framework for substances of human origin (SoHO) that are collected from humans and used in medical treatments. This includes views on current UK legislation and on the potential implications of the new EU SoHO Regulation (Regulation (EU) 2024/1938), which will also apply in Northern Ireland from 7 August 2027 under the terms of the Windsor Framework.

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Historically, UK law has implemented EU directives to set minimum standards for the collection, testing, processing and distribution of blood, tissues and cells. These standards are designed to protect public health and ensure traceability and safety throughout the supply chain.

The introduction of the EU SoHO Regulation presents an opportunity for the UK to consider whether changes are needed to ensure patient safety, and the quality and continuity of supply, including redefining additional substances as SoHO.

This call for evidence seeks stakeholder views on whether, and how, evidence generated through the EU's regulation review process, including the approaches set out in the EU SoHO Regulation, could inform potential changes to the UK legislative framework. It will also consider how changes to UK laws could further facilitate the movement of SoHO between the UK and EU, and within the UK itself.

The aim of this call for evidence is to ensure that the UK legislative framework for SoHO keeps pace with scientific and technological advances, while maintaining high standards of safety, quality and accessibility for patients. Responses are welcome from individuals working in the SoHO sector and from others with relevant expertise or with an interest in the regulation and its use, and respondents are encouraged to provide examples where possible. The call for evidence is open until 17 June 2026.

EUROPE

European Medicines Agency (EMA)

New PRIME tools to accelerate development of medicines in the EU

The EMA has launched three new major features of PRIME with the goal of supporting continued scientific dialogue, giving developers faster answers, and better supporting preparation for the submission of marketing authorisation applications.

The first tool is a regulatory roadmap and product development tracker, helping to chart a medicine's progress and highlighting potential issues early with continuous alignment with the EMA throughout development. The second tool is expedited scientific advice, a fast-track route for developers to receive regulatory input on questions critical to their development. The third tool is a submission readiness meeting with the EMA, allowing for discussion of the progress of the product against the development plan and helping identify any gaps in the CHMP data package.

These tools will be integrated into PRIME as permanent features as part of the revised EU pharmaceutical legislation that is expected to come into force. The EMA is also exploring a new concept of an EMA Product Development Coordination as primary point of contact for developers to facilitate better support through development. Please find further information here.

EMA scientific guideline on development of anti-cancer medicinal products in paediatric patients

The EMA has released a concept paper outlining high level recommendations for generating and assessing meaningful proof-of-concept data to support the

development of novel anti-cancer medicinal products in paediatric patients, highlighting key evidence domains, challenges in evaluating paediatric oncology data, and a proposed process for engaging academic and expert stakeholders to inform the assessment of Paediatric Investigation Plans.

The deadline for this consultation is 30 June 2026.

Network Data Steering Group (NDSG) workplan 2026-2028 on data and AI in medicines regulation

The EMA has published the NDSG Workplan 2026–2028 which sets out how the EU medicines regulatory network will optimise the use of data and artificial intelligence to support innovation, improve evidence generation, and strengthen regulatory decision-making. The plan focuses on enhancing data analytics and standardisation frameworks, supporting the European Health Data Space through DARWIN EU, and ensuring AI is used responsibly within an ethical and compliant framework.

USA

Food and Drug Administration (FDA)

FDA approves first gene therapy for severe leukocyte adhesion deficiency type I

The FDA has approved Kresladi (marnetegragene autotemcel), the first gene therapy for the treatment of severe leukocyte adhesion deficiency type I (LAD-I).

Severe LAD-I is a rare, inherited immune deficiency caused by mutations in the *ITGB2* gene, which prevent white blood cells from effectively fighting infections. Patients with severe LAD-I experience recurrent, life-threatening bacterial and fungal infections with substantial morbidity and mortality in the first decade of life.

Kresladi is indicated for the treatment of paediatric patients with severe LAD-I caused by biallelic variants in *ITGB2* who do not have an available HLA-matched sibling donor for allogeneic hematopoietic stem cell transplant. It consists of the patient's autologous (own) hematopoietic (blood) stem cells which are genetically modified to introduce functional copies of the *ITGB2* gene. After conditioning, a single dose of Kresladi is infused intravenously to restore CD18 and CD11a cell surface expression in white blood cells.

The FDA granted accelerated approval of Kresladi, as well as a Rare Pediatric Disease Priority Review Voucher to Rocket Pharmaceuticals, Inc. Please find further information here.

FDA launches new adverse event look-up tool

The FDA has launched its new Adverse Event Monitoring System (AEMS) platform, a unified, modernised platform designed to significantly improve the accessibility, transparency, and efficiency of adverse event reporting across all FDA-regulated products.

This new tool replaces the Agency's previously outdated and fragmented systems, which spanned seven separate databases and created gaps in postmarket safety surveillance. By consolidating reports related to drugs, biologics, vaccines, cosmetics, and animal food into a single, streamlined dashboard, AEMS enhances FDA's ability to detect safety signals and monitor emerging risks more effectively.

The Agency will also migrate historical adverse event data to AEMS and decommission certain legacy systems. By the end of May 2026, AEMS will contain real-time adverse event reports for all FDA-regulated products.

The FDA emphasises that this modernisation effort will not only improve public and researcher access to real-time safety data but is also projected to reduce reliance on costly legacy platforms. Additionally, the move from quarterly to real-time publication of adverse event reports is expected to reduce Freedom of Information Act requests and strengthen the FDA's post market surveillance capabilities. Please find further information here.

FDA Q&A on pyrogen and endotoxins testing

The FDA has issued the guidance document titled *"Pyrogen and Endotoxins Testing: Questions and Answers"*, providing industry stakeholders with consolidated recommendations on the appropriate methodologies, sampling plans, acceptance criteria, and regulatory expectations for pyrogen and bacterial endotoxin testing across biological product, drugs and devices.

This guidance reflects FDA's current thinking on the application of United States Pharmacopeia (USP) bacterial endotoxin testing standards and related industry methodologies, including Chapter <85> "Bacterial Endotoxins Test" and Chapter <161> "Medical Devices – Bacterial Endotoxin and Pyrogen Tests".

The document outlines FDA's recommendations on key topics such as establishing scientifically sound sampling plans for in-process and finished product testing, determining when retesting is appropriate, addressing sample storage and handling to preserve endotoxin detectability, and implementing alternative testing methods where justified. These questions and answers are intended to support consistent, compliant testing practices and promote product safety by ensuring reliable detection of pyrogens and endotoxins in regulated products.

FDA draft guidance on responding to FDA Form 483 (observations at the conclusion of a drug CGMP inspection)

The FDA has released a draft guidance for industry titled *"Responding to FDA Form 483 Observations at the Conclusion of a Drug CGMP Inspection,"* intended to support foreign and domestic human and animal drug manufacturing establishments inspected by FDA, including biologics and combination product facilities, in preparing thorough, well structured responses to inspectional observations issued on FDA Form 483. This guidance outlines the FDA's current expectations for the organisation, content, and timing of written responses, emphasising that a clear, evidence based submission is essential for demonstrating commitment to Current Good Manufacturing Practice (CGMP) compliance.

The draft guidance stresses the importance of robust investigations, root cause analyses, and Corrective and Preventive Action plans, supported by appropriate documentation. FDA recommends submitting a single, consolidated response within 15 business days, highlighting that timely, comprehensive submissions enable the Agency to better evaluate proposed corrective actions and determine whether additional regulatory action is necessary. The guidance also underscores the role of executive management oversight, reinforcing that leadership accountability and resource allocation are critical to ensuring sustained compliance.

The deadline to submit comments on the guidance is 8 May 2026.

Patient and consumer warning about potential serious risks of harm following use of unapproved products from human cells or tissues

The FDA has issued a patient and consumer warning highlighting the serious potential risks of harm associated with the use of unapproved products derived from human cells or tissues. The Agency reports that it continues to receive complaints and adverse event reports, including patient deaths, linked to such unapproved products. In one recent case, a patient in the US died after self-injecting Laennec, an imported human placenta tissue derived product that is not FDA approved. FDA has stated it is actively investigating this incident.

The FDA warns that many of these unapproved products are promoted online for treating a wide range of diseases or medical conditions, despite lacking any FDA review for quality, safety, purity, or potency. These products are typically regulated as drugs or biological products, but without FDA approval, their manufacturing conditions, sterility, and clinical safety remain unverified. Patients and consumers are strongly urged to avoid using any unapproved human cell or tissue derived products, as they may pose significant health risks, including infection, adverse reactions, or death. Please find further information here.

Japan

Pharmaceuticals and Medical Devices Agency (PMDA)

Japan becomes first to approve stem cell therapies for Parkinson's and heart failure

Japan has become the first country in the world to approve two stem cell–based regenerative therapies for clinical use, including one for Parkinson's disease and one for severe heart failure. These landmark approvals involve induced pluripotent stem cell (iPSC)-derived treatments, marking a major milestone in regenerative medicine. The Parkinson's therapy, AMCHEPRY, delivers iPSC derived dopamine producing neurons directly into the brain, aiming to replace cells lost due to disease. The heart failure therapy, RiHEART, uses sheets of stem cell derived cardiac muscle cells applied to the heart to support tissue repair and improve heart function. Both therapies received conditional approval, allowing clinical use while additional safety and efficacy data continue to be collected.

Public consultations

Medicines and Healthcare products Regulatory Agency (MHRA)

	Title	*Consultation Period*	Category
1.	*Medical devices regulations: targeted consultation on the indefinite recognition of CE marked devices*	*End date: 10 April 2026*	*Public consultation*
2.	*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 quality aspects of phage therapy medicinal products*	*End date: 30 April 2026*	*Draft guidance*
2.	*Guideline on non-inferiority and equivalence comparisons in clinical trials*	*End date: 31 May 2026*	*Draft guidance*
3.	*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*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Considerations for the use of the Plausible Mechanism Framework to Develop Individualized Therapies that Target Specific Genetic Conditions with Known Biological Cause*	*End date: 27 April 2026*	*Draft guidance*
2.	*Responding to FDA Form 483 Observations at the Conclusion of a Drug CGMP Inspection*	*End date: 08 May 2026*	*Draft guidance*