Regulatory Round-up

December 2025

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United Kingdom

Medicines and Healthcare products Regulatory Agency (MHRA)

Strengthening collaboration between the MHRA and the Department of Health Northern Ireland

The MHRA, together with the Department of Health in Northern Ireland and wider regional partners, has announced a strengthened programme of collaboration aimed at supporting innovation, enhancing patient safety, and ensuring that people in Northern Ireland continue to benefit from world-class regulation of medicines and medical technologies.

This enhanced partnership includes key commitments such as:

- expanding the visibility and use of the Yellow Card scheme across Northern Ireland to improve surveillance of medicines and medical devices,
- increasing participation in cutting edge research to accelerate the development of new therapies,
- establishing an MHRA presence in Northern Ireland to support closer engagement with local industry, academia, and the health and care system.

In addition, both organisations will explore new opportunities in regulatory science and innovation, recognising the potential of Northern Ireland's life sciences ecosystem. This collaboration builds on discussions from the first MHRA Board seminar held in Belfast in November 2025 and reflects a strong commitment to deepening cross UK regulatory partnerships for the benefit of patients and healthcare providers alike. Please find further information here.

UK and Singapore launch a Regulatory Innovation Corridor to speed up access to breakthrough health technologies

The UK and Singapore have launched a new Regulatory Innovation Corridor to accelerate patient access to breakthrough health technologies. Through this first of its kind partnership between the MHRA and Singapore's Health Sciences Authority, companies will be able to engage with both regulators simultaneously, enabling faster, smarter clinical development and reducing delays. The initiative focuses on high impact areas such as cancer, neurodegenerative diseases, obesity, rare diseases, and advanced diagnostics, supporting both countries' ambitions to strengthen global life sciences innovation. Please find further information here.

British Pharmacopoeia (BP)

Characterisation of the capsid particle population in rAAV products: determination of vector genome identity, integrity and encapsidated DNA impurities & capsid protein characterisation

BP has released two draft guidance documents concerning the characterisation of the capsid particle population in recombinant adeno associated virus (rAAV) products. These drafts outline approaches for assessing vector genome identity, integrity, and encapsidated DNA impurities, as well as capsid protein characterisation, with the aim of supporting consistent, robust analytical practices across the gene therapy sector.

The BP is inviting feedback from stakeholders across GMP manufacturing, research and development, academia, and clinical environments to help refine and improve these documents ahead of formal publication.

The deadline for submitting consultation responses is 27 March 2026. Stakeholders should return completed response forms to the BP as instructed on the consultation page.

The draft guidance documents and response forms can be found here.

EUROPE

European Commission (EC)

New measures to make EU health sector more innovative, competitive and resilient

The EC has announced a comprehensive package of new measures designed to strengthen innovation, competitiveness, and resilience across the EU health sector. The initiative introduces a proposed Biotech Act, revised medical device rules, and the Safe Hearts Plan, collectively aiming to accelerate development of advanced health technologies, support EU biotech growth, streamline regulatory pathways, and address cardiovascular disease – the leading cause of death in Europe.

The Biotech Act proposes to amend the Clinical Trials Regulation, ATMP Regulation, Substance of Human Origin (SoHO) Regulation and the Directive on deliberate release of genetically modified organisms, and includes several proposals that are relevant to ATMP developers, including:

- Low-risk GMO ATMPs to be exempt from full environmental risk assessment.
- Future-proof ATMP definitions by empowering the EC to update definitions without expanding scope.
- Accelerated clinical trial review timelines, including eliminating the additional 50 days for ATMPs.
- Allowing use of Active Substance Master Files (ASMF) and Certificates of Suitability (CEP) for ATMPs.

Please find further information here.

European Directorate for the Quality of Medicines (EDQM)

EDQM releases revised draft "Guideline on requirements for revision/renewal of certificates of suitability to the European Pharmacopoeia monographs" for public consultation

EDQM has released a revised draft guideline for public consultation on the requirements for the revision and renewal of Certificates of Suitability to European Pharmacopoeia monographs, aiming to clarify procedures and ensure continued compliance with quality standards. It applies to manufacturers and stakeholders involved in demonstrating that pharmaceutical substances comply with European Pharmacopoeia monographs. The aim of the revision is to streamline processes, improve transparency, and maintain high standards of quality and safety in medicines. The consultation deadline for this document is 16 January 2026. Please find further information including access to the guideline here.

European Medicines Agency (EMA)

EMA welcomes political agreement on new EU pharmaceutical legislation

The recent political agreement on the comprehensive reform of EU pharmaceutical legislation is expected to have a significant impact on the ATMP sector, including:

- Amending the existing ATMP Regulation (EC) No 1394/2007 and streamlining EMA's committee structure, with specialist remits currently covered by CAT, COMP and PDCO expected to be integrated into the CHMP.
- Introducing faster centralised marketing authorisation assessments (reduced from 210 to 180 days), strengthened PRIME support, expanded possibilities for extended scientific advice involving HTA bodies and medical device expert panels, and marketing authorisations that will be valid by default for an unlimited period, unless a time limit is required on safety grounds.
- Enabling the European Commission to establish regulatory sandboxes allowing innovative medicines that cannot be developed under existing rules to be tested under adapted regulatory requirements.

In addition, the legislation foresees tailored regulatory frameworks for non-standard products such as personalised therapies, improvements to paediatric development through a more efficient process and the formalisation of iterative Paediatric Investigation Plans, and a requirement for applicants to make approved product information available in electronic form. The formal adoption and exact timelines for implementation are still to be confirmed. Please find further information here.

Technology Capability Investment Plan

The EMA's *Technology Capability Investment Plan 2028* outlines how the Agency will modernise and transform its digital infrastructure to become the central digital hub of the European Medicines Regulatory Network. It focuses on meeting new legislative demands, improving efficiency through advanced technologies such as AI, modernising ageing IT systems, and enabling a more agile, data driven regulatory environment to better protect public and animal health.

Emer Cooke, EMA's Executive Director: 2025 achievements in medicine regulation

In her 2025 end of year message, EMA's Executive Director Emer Cooke highlighted a landmark agreement to overhaul EU pharmaceutical legislation, the recommendation of 104 new medicines and major advances in innovation support, digitalisation, and workforce investment, marking a pivotal year for strengthening Europe's medicines regulatory system. Please find further information here.

USA

Food and Drug Administration (FDA)

FDA approves first CAR T-Cell therapy for marginal zone lymphoma

The FDA has approved Breyanzi (lisocabtagene maraleucel), a chimeric antigen receptor (CAR) T-cell therapy for adults with marginal zone lymphoma (MZL) who have relapsed or failed at least two prior treatments.

MZL is a rare, slow-growing cancer of the lymphatic system, a type of B-cell non-Hodgkin lymphoma. This approval marks the first time that patients with MZL will have access to a personalised, genetically engineered T-cell therapy specifically tailored to their cancer. Please find further information here.

FDA approves first cellular therapy to treat patients with severe aplastic anaemia

The FDA has approved Omisirge (omidubicel-onlv), the first cellular therapy for patients with severe aplastic anaemia (SAA). SAA is a rare, life-threatening blood disorder where the bone marrow fails to produce enough red blood cells, white blood cells, and platelets. Treatment for SAA depends on age and usually consists of either immunosuppressive therapy and/or haematopoietic stem cell transplant preferably from a matched sibling or matched related donor. If a donor is not available, providers may seek the use of umbilical cord transplant to treat SAA.

Omisirge is a stem cell therapy in which donated cord blood stem cells are chemically enhanced with nicotinamide (a form of vitamin B3) and then given to a patient to help restore their blood and immune system.

Please find further information here.

FDA approves first gene therapy treatment for Wiskott-Aldrich Syndrome

The FDA has approved Waskyra (etuvetidigene autotemcel), the first gene therapy for Wiskott-Aldrich Syndrome (WAS), a rare inherited immune disorder. WAS is a rare, life-threatening genetic disease caused by mutations in the WAS gene. The condition is characterised by bleeding, eczema, recurrent infections, and increased susceptibility to autoimmunity and lymphoreticular malignancies.

Waskyra consists of the patient's own hematopoietic (blood) stem cells, which have been genetically modified to include functional copies of the WAS gene. Following reduced-intensity conditioning, the gene-corrected cells are infused intravenously to restore blood cell production. Waskyra restores functional WAS protein expression in affected cells, addressing the underlying cause of the disease.

Please find further information here.

Guidance for industry: Enhancing participation in clinical trials – eligibility criteria, enrolment practices, and trial designs

The FDA has issued a guidance titled *Enhancing Participation in Clinical Trials — Eligibility Criteria, Enrollment Practices, and Trial Designs* which recommends strategies for sponsors to broaden eligibility criteria and improve enrolment practices to achieve more diverse and representative clinical trial populations. It emphasises including participants across varied demographic and clinical characteristics to ensure trial results better reflect real-world patients and support more accurate assessments of safety and effectiveness.

Draft guidance for industry: Study of sex differences in the clinical evaluation of medical products

The FDA has issued a draft guidance titled *Study of Sex Differences in the Clinical Evaluation of Medical Products* which emphasises the importance of designing clinical studies that enrol adequate numbers of both females and males, encourages analysis and interpretation of sex specific data, and recommends including sex specific information in regulatory submissions to improve the generalisability of results and identify potential differences in safety and effectiveness between sexes.

INTERNATIONAL

International Conference on Harmonisation (ICH)

Recommendations for future guidelines related to advanced therapy medicinal products

The ICH Cell and Gene Therapy Discussion Group (CGTDG) has published its paper *Recommendations with Regard to Future Advanced Therapy Medicinal Products-related Guidelines*, outlining strategic priorities and harmonisation needs for future global ATMP regulations, including a roadmap for new and revised guidelines to address the unique complexities of cell and gene therapy products.

Public consultations

European Medicines Agency (EMA)

	Title	Consultation Period	Category
2.	*Guideline on quality aspects of phage therapy medicinal products*	*End date: 30 Apr 2026*	*Draft guidance*
3.	*Guideline on non-inferiority and equivalence comparisons in clinical trials*	*End date: 31 May 2026*	*Draft guidance*

British Pharmacopoeia (BP)

	Title	Consultation Period	Category
1.	*Determination of Vector Genome Identity, Integrity and Encapsidated DNA Impurities*	*End date: 27 March 2026*	*Draft guidance*
2.	*Capsid Protein Characterisation*	*End date: 27 March 2026*	*Draft guidance*

European Directorate for the Quality of Medicines & HealthCare (EDQM)

	Title	Consultation Period	Category
1.	*Guideline on Requirements for revision/renewal of certificates of suitability to the European Pharmacopoeia Monograph*	*End date: 16 January 2026*	*Draft guidance*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Study of Sex Differences in the Clinical Evaluation of Medical Products*	*End date: open*	Draft guidance