Regulatory Round-up

February 2026

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United Kingdom

Medicines and Healthcare Products Regulatory Agency (MHRA)

Conclusion of MHRA Real-World Evidence Scientific Dialogue Pilot Programme

The pilot phase of the MHRA Real-World Evidence (RWE) Scientific Dialogue Programme has been concluded.

The aim of the programme was to advance data driven innovation, strengthen evidence generation methodologies, and support regulatory and health technology assessment (HTA) decision making across the product lifecycle.

During the pilot, four applicants were selected for confidential meetings with the MHRA and one for the pre-competitive workshop jointly convened by the MHRA and the National Institute for Health and Care Excellence (NICE).

The confidential meetings encompassed a broad spectrum of RWE issues across multiple disease areas and stages of the product lifecycle, while the workshop concentrated specifically on the distinct challenges and opportunities associated with leveraging real-world data for rare diseases in both regulatory evaluations and HTAs.

The findings of the pilot are now being assessed and options for continuation being explored. Further updates will follow once the evaluation is complete and recommendations for the next phase are agreed. Please find further information here.

UK and Japan Strengthen Science and Technology Ties

The UK and Japan are deepening their science and technology collaboration with major new initiatives in advanced therapeutics, including a multi-million-pound investment into the UK's life sciences' gene therapy industry and a partnership to deliver a new national rare disease genomics pilot in Japan.

In a boost for UK life sciences manufacturing, Orchard Therapeutics, the UK subsidiary of Japanese company Kyowa Kirin, is set to invest around £11 million in the UK. Subject to final agreement of terms and conditions of the UK government's Life Sciences Innovative Manufacturing Fund, the funding will go towards the discovery of new drugs which could treat and beat diseases, transforming outcomes for patients.

The investment by Kyowa Kirin into Orchard Therapeutics paves the way for gene therapies for devastating illnesses to be researched and developed in the UK, supporting high-skilled jobs and helping ensure patients can access the most

innovative treatments, with further potential investment in R&D to follow. Please find further information here.

Medical Devices Regulations: Targeted Consultation on the Indefinite Recognition of CE Marked Devices

The MHRA has launched a targeted consultation on proposals for the approach to recognising CE marked medical devices in Great Britain.

Published on 16 February 2026, the consultation seeks feedback on measures intended to safeguard patient access, support continuity of supply, and align with broader reforms to modernise the UK's medical device regulatory framework.

Key proposals include:

- Extending the current transitional arrangements for devices that comply with the Medical Device Directive (MDD) to align with the EU timelines for devices to transition from MDD to EU Medical Devices Regulation (EU MDR)
- Indefinitely recognising devices that comply with the EU MDR and EU in vitro Diagnostic Medical Devices Regulation (EU IVDR)
- Introduction of an international reliance route for devices classified higher in Great Britan than in the EU

The consultation deadline is on 10 April 2026. Please find further information here.

Medicines: Get Integrated Scientific Advice from the MHRA and NICE

The MHRA and NICE have published new guidance on 20 February 2026 on the Integrated Scientific Advice (ISA) service.

ISA replaces the previous MHRA–NICE joint scientific advice service and provides pharmaceutical companies with coordinated scientific advice on evidence requirements for market authorisation and HTA, through a single, streamlined process. The service is most suitable for products in clinical development and aiming to follow MHRA and NICE aligned pathway timelines.

The service enables companies to clarify regulatory and HTA evidence requirements early, reduce the risk of conflicting advice, and discuss evidence generation plans directly with both bodies before receiving final written advice. Where relevant, MHRA and NICE will also indicate how UK advice may differ from other regulators, such as the Food and Drug Administration (FDA) or the European Medicines Agency (EMA).

Further details on the MHRA and NICE ISA service, including good questions for the ISA service, documents you need to prepare, fees, presenting at an ISA meeting and ISA service reports, is available HYPERLINK "https://www.gov.uk/government/publications/get-more-help-to-apply-for-medicines-integrated-scientific-advice-isa".

EUROPE

European Commission (EC)

HTA Coordination Group Publishes its 2025 Annual Report

The Member State Coordination Group on Health Technology Assessment (HTACG) has released its 2025 Annual Report, outlining the outcomes from the first year of implementing the EU Health Technology Assessment Regulation.

The report highlights the main accomplishments of the HTACG and its four subgroups throughout 2025, including progress on joint clinical assessments, joint scientific consultations, and the engagement of experts in these activities. It also details work carried out on identifying emerging health technologies, developing methodological and procedural guidance, and advancing communication efforts with key stakeholders.

In 2025, the HTACG started thirteen joint clinical assessments on new oncology products and advanced therapy medicinal products (ATMPs). Please find further information including access to the report here.

European Medicines Agency (EMA)

Committee for Advanced Therapies Work Plan 2026

EMA's Committee for Advanced Therapies (CAT) has adopted its Work Plan for 2026, outlining key priorities to advance the development, evaluation, and regulatory oversight of ATMPs. In 2026, the CAT will focus on:

- strengthening scientific and regulatory guidance
- enhancing training for assessors and stakeholders
- providing specialised ATMP expertise to support other EMA committees and decision-makers

The Work Plan highlights continued efforts in areas such as international regulatory collaboration, identifying emerging regulatory challenges, and monitoring new legislation affecting the ATMP landscape. Planned activities include training on the recently finalised guideline for investigational ATMPs in clinical trials, organising webinars for developers, and revising the existing Q&A document on gene therapies to reflect current regulatory positions.

Through these initiatives, the CAT aims to enable efficient ATMP development, bolster regulatory harmonisation, and support the integration of ATMP expertise into the evolving European regulatory framework. Please find more details here.

Review of the Stepwise Paediatric Investigation Plan (sPIP) Pilot: Outcomes and Future Perspectives

EMA has concluded its review of the stepwise Paediatric Investigation Plan (sPIP) pilot, confirming that the model is both feasible and effective in expediting paediatric drug development while maintaining scientific rigour.

The sPIP pilot was launched to manage exceptional paediatric development programmes in which critical data are unavailable at the time of initial PIP submission, thereby constraining evidence-based planning of key study elements.

According to the report published on 27 January 2026, the pilot demonstrated clear advantages in regulatory efficiency, flexibility, and suitability for products developed under significant scientific uncertainty.

The EMA reported that the stepwise approach supported timely paediatric development by allowing sponsors to initiate paediatric programmes with incomplete data, provided that outstanding elements would be addressed at predefined milestones. Between 2023 and 2025, the pilot received twenty-seven eligibility requests, of which fifteen sPIPs were submitted and eight received adopted opinions. These covered areas such as neurologic and metabolic genetic disorders, immunology, hepatology, and oncology, reflecting the pilot's emphasis on complex and rare paediatric conditions. Notably, 12.5% of included products were classified as ATMPs, demonstrating applicability across diverse therapeutic modalities.

Overall, the pilot affirmed the value and practicality of the stepwise PIP framework, with EMA stating that future efforts will focus on refining milestone management, integrating the procedure as a permanent regulatory pathway, and ensuring strong long-term monitoring to maintain high standards of paediatric oversight and public health protection.

USA

Food and Drug Administration (FDA)

FDA Launches Plausible Mechanism Framework for Accelerating Development of Individualized Therapies for Ultra-Rare Diseases

The FDA has issued draft guidance establishing a new regulatory framework designed to accelerate the development and approval of targeted, individualised therapies for ultra-rare diseases.

Announced on 23 February 2026, the framework is designed for sponsors seeking to generate substantial evidence of effectiveness and safety in circumstances where randomised controlled trials are not feasible due to extremely small patient populations. It focuses on genome editing and RNA-based therapies (e.g., antisense oligonucleotides), while leaving open the possibility that other tailored therapeutics could quality where they directly address the specific underlying cause of disease.

The guidance focuses on therapies that target a specific genetic, cellular, or molecular abnormality and are designed to correct or modify the underlying cause of disease. Key criteria include:

- Identifying the disease-causing abnormality

- Demonstrating the therapy targets the root cause or proximate biological pathway
- Leveraging well-characterised natural history data in untreated patients
- Confirming successful target engagement or editing
- For traditional approval, therapies should demonstrate improvement in clinical outcomes, disease course, or biomarkers if they are established to predict clinical benefit

The guidance also contemplates that genome editing products targeting different mutations within a single gene may be included in one product application and potentially evaluated under a master protocol. A highly supported "plausible" mechanism of action could then support addition of further mutation-specific variants beyond those initially studied.

FDA leadership emphasised that this initiative aims to remove regulatory barriers, support scientific innovation, and deliver meaningful treatments more rapidly to patients with few or no existing therapeutic options.

The draft guidance is available for public comment until 27 April 2026

INTERNATIONAL

International Conference on Harmonisation (ICH)

Mock Example Developed to Illustrate Quality Modules of ICH M4Q(R2) Common Technical Document

The ICH M4Q(R2) Expert Working Group has developed a mock example, "Sakura Bloom R2," to illustrate the potential structure and presentation of information proposed in the revised Modules 2.3 (Quality Overall Summary) and 3 (Quality) of the Common Technical Document – Quality.

This mock-up demonstrates how the updated concepts and organisation proposed in the draft M4Q(R2) Guideline may be applied in practice, supporting industry and regulatory stakeholders in understanding the intended dossier format and content.

The mock example is provided solely for educational and illustrative purposes. It does not represent an actual regulatory submission and may not fully align with all ICH Quality guidelines or regional requirements, and its purpose is solely to facilitate understanding of the draft M4Q(R2) concepts.

The draft ICH M4Q(R2) Guideline is currently open for public consultation, with stakeholders encouraged to comment specifically on the guideline itself, rather than on the mock example. Please find further information on the mock example and public consultation here.

Final Training Module Published for ICH Guideline E2B(R3)

The ICH has issued the final training module, *Implementation Guide for Electronic Transmission of Individual Case Safety Reports, Module III*, for ICH Guideline E2B(R3). This module completes the three-part training series designed to support the

technical and practical implementation of electronic Individual Case Safety Report submissions. It provides detailed guidance to facilitate harmonised, efficient, and consistent electronic safety reporting between regulatory authorities and the pharmaceutical industry.

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*

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*

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*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Minimal Residual Disease and Complete Response in Multiple Myeloma: Use as Endpoints to Support Accelerated Approval*	*End date: 23 March 2026*	*Draft guidance*

2.	*Use of Bayesian Methodology in Clinical Trials of Drug and Biological Products*	*End date: 13 March 2026*	*Draft guidance*
3.	*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*