

# Regulatory Round-up

May 2026

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## United Kingdom

### Medicines and Healthcare products Regulatory Agency (MHRA)

#### MHRA consults on new regulatory framework to make UK a global leader in rare disease therapy development

The MHRA has launched a public consultation on a proposed new Rare Disease Therapies Regulatory Framework that would introduce significant regulatory innovation to the UK's rare disease landscape, aiming to address the significant unmet medical need affecting over 3.5 million people in the UK who currently have limited or no treatment options.

The draft framework sets out a technology-agnostic regulatory framework which recognises the unique challenges associated with rare diseases, such as small patient populations, limited clinical data, and difficulties in conducting traditional randomised trials. It is designed for therapies targeting rare diseases with a prevalence of typically one in approximately 50,000 or fewer in the UK.

Central to the proposal is the introduction of a new regulatory pathway, the Investigational Marketing Authorisation, which would allow earlier, controlled patient access to promising therapies while additional clinical and real-world evidence continues to be generated.

The framework sets out expectations across the full product lifecycle, including eligibility criteria, evidence generation using innovative methods (e.g. adaptive trials, real-world data and patient video assessments), enhanced patient engagement, and a structured transition to full marketing authorisation where appropriate.

The new guidance is designed to position the UK as the destination of choice for rare disease therapy development and clinical trials, while safeguarding patients and maintaining confidence in regulatory decision-making.

MHRA is calling on the pharmaceutical and life sciences industries to play a central role in shaping the final framework. The deadline for responding to the consultation is 30 July 2026. Access to the draft framework and information on how to respond to the survey questions can be found [here](#).

#### MHRA opens UK-wide consultation on redefining gene therapies

MHRA, acting jointly with the Department of Health in Northern Ireland, has launched a consultation to update the legal definition of gene therapy medicinal products (GTMPs) in the UK, ensuring regulation keeps pace with rapid advances in gene editing, synthetic biology, and manufacturing technologies.

#### 29 April– 28<sup>th</sup> May 2026

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Advances in science and manufacturing have prompted consideration on whether the current definitions remain appropriate for use. The proposed changes aim to modernise the framework by defining gene therapies based on how they work, rather than their biological origin, providing greater clarity and consistency across both biological and synthetic products.

The consultation seeks views on five key proposals:

- Removing the requirement for gene therapies to be biological in origin.
- Clarifying when synthetic or recombinant nucleic acids bring a product into scope.
- Ensuring products involving sequence specific genome editing are clearly regulated as GTMPs, regardless of the composition of the active substance.
- Maintaining the exclusion of vaccines against infectious diseases from GTMP definitions.
- Proposing updates to the Human Medicines Regulations 2012 to support these revised definitions.

The updated definition will not alter the regulatory classification of products already licensed.

The consultation will run until 22 June 2026. Please find further information [here](#).

### **MHRA approval: Vyjuvek**

On 15 May 2026, the MHRA approved beremagene geperpavec (Vyjuvek) for the treatment of wounds in patients with dystrophic epidermolysis bullosa (DEB), a rare genetic disorder characterised by fragile, blister-prone skin. The treatment is approved for use from birth.

Vyjuvek is the first topical, redosable gene therapy which is administered as a gel directly to wounds. It works by delivering functional copies of the COL7A1 gene into skin cells, enabling the production of type VII collagen, and supporting improved wound healing without altering the patient's underlying DNA.

This decision represents a significant advancement in the management of DEB, providing a new therapeutic option for patients with a condition that currently has limited treatment choices. Please find further information [here](#).

### **MHRA invites views on proposed changes to medical device regulation**

The MHRA has launched a consultation to gather stakeholder views on proposed changes to the regulation of medical devices and in vitro diagnostic devices in Great Britain. The [draft Medical Devices \(Amendment\) Regulations 2026](#) introduces updated premarket requirements aimed at strengthening patient safety while supporting faster access to innovative technologies. Key proposals include enabling streamlined access for devices already approved in comparable jurisdictions, improving traceability through mandatory unique device identifiers, and aligning UK requirements with international standards and best practice.

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Additional measures seek to enhance transparency and patient information, including the introduction of implant cards, as well as strengthened requirements for technical documentation and clearer alignment between product claims and intended use.

Overall, the proposed changes aim to create a proportionate, patient-centred regulatory framework that promotes innovation while maintaining high standards of safety, quality, and oversight within the UK MedTech industry, with the aim for the UK to become one of the top three fastest countries in Europe to access MedTech by 2030.

The MHRA seeks stakeholder views on proposed GB pre-market medical device and in vitro diagnostic device regulation changes to inform impact assessment. The deadline for submitting responses to the MHRA survey is 19 June 2026. Please find further information including how to respond to the open call for evidence [here](#).

### **MHRA supports Northern Ireland life sciences sector with new hub**

The MHRA's new hub in central Belfast is a positive step in strengthening support for Northern Ireland's growing life sciences sector. By providing direct access to regulatory expertise and fostering closer collaboration across industry, academia, and healthcare, the hub will help accelerate innovation and bring new medicines and medical technologies to patients more efficiently and ensure the MHRA's work is even more strongly linked to the whole of the UK. Please find further information [here](#).

### **MHRA–NICE real-world evidence scientific dialogue programme**

The MHRA Real-World Evidence (RWE) Scientific Dialogue Programme webpage has been updated to reflect the relaunch of the service, incorporating improvements based on insights from the pilot phase.

Key updates include:

- Enhanced service description: Providing clearer information on the scope, purpose, and eligibility criteria of the refreshed MHRA–NICE RWE Scientific Dialogue (RWE SD).
- Revised expression of interest process: Outlining how applicants can submit requests to the RWE SD and what to expect at each stage of the process.
- Strengthened applicant guidance: Offering clearer direction on the types of questions that are appropriate for discussion.
- Content refresh: Updating language, structure, and accessibility to improve usability and user experience.

Together, these updates mark the transition from a pilot initiative to an established service, aiming to deliver greater clarity, consistency, and accessibility for stakeholders engaging with the programme. Please find further information [here](#).

### **MHRA guidance on environmental risk assessments (ERAs) in medicines**

The MHRA has published [guidance](#) which sets out the expectations for ERAs for medicinal products in the UK. The MHRA seeks to align ERA requirements with those

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of international regulatory authorities, while ensuring their appropriate application within the UK.

ERAs are a mandatory component of all marketing authorisation applications for medicinal products in the UK, as required under [Schedule 8 of the Human Medicines Regulations](#). An ERA evaluates the potential environmental impact of a product based on the physicochemical, ecotoxicological, and environmental fate properties of the active substance, and includes consideration of exposure arising from its use.

## Europe

### European Directorate for the Quality of Medicines (EDQM)

#### EDQM opens digital stakeholder consultation on 23<sup>rd</sup> edition of the Blood Guide

EDQM has initiated a digital stakeholder consultation for the fully revised 23<sup>rd</sup> edition of the [Guide to the preparation, use and quality assurance of blood components](#). The “Blood Guide” is intended for all professionals working in the donation, collection, testing, processing, storage, distribution and transfusion of blood and blood components, for blood establishments, hospital blood banks and healthcare and regulatory professionals in the blood sector.

The consultation, conducted via a dedicated online platform, aims to enhance transparency, accessibility, and stakeholder engagement by enabling interested parties to review and comment on the updated content. Following the consultation period, all comments will be evaluated prior to final adoption of the guide by the European Committee on Blood Transfusion (CD-P-TS), with publication of the 23<sup>rd</sup> edition planned in a fully digital format.

The deadline for the consultation is 3 July 2026. Please find further information [here](#).

### European Medicines Agency (EMA)

#### EMA revamp of marketing authorisation assessment templates

EMA’s Committee for Medicinal Products for Human Use (CHMP) is undertaking a revamp of its marketing authorisation assessment report templates to improve the efficiency, consistency, and quality of the evaluation process for new medicines. This initiative focuses on reducing duplication across multiple interrelated assessment reports, streamlining workflows, and optimising the use of resources. Please find further information [here](#).

#### EU tracks progress towards 2030 clinical trial targets

EMA, together with the European Commission and the Heads of Medicines Agencies, have published their first [progress report](#) monitoring delivery against the EU’s 2030 clinical trial targets. The report, based on data from January to March 2026, marks an important step toward improving transparency and accountability in the EU clinical research landscape.

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Early findings indicate gradual progress toward key objectives, including increasing the number of multinational clinical trials and accelerating patient recruitment timelines. The EU aims to add 500 additional multinational trials by 2030 and ensure that 66% of trials recruit patients within 200 days of application submission; current figures show 40.5% meeting this timeline. The new clinical trial targets are aligned with the EU Biotech Act.

The report highlights that while activity levels are approaching targets, continued effort is required to strengthen the EU's attractiveness as a destination for clinical research and to improve timely patient access to innovative treatments.

Progress will be tracked through regular quarterly reporting and annual review under the Accelerating Clinical Trials in the EU (ACT EU) initiative, ensuring alignment with the EU's broader ambition to create a more efficient, competitive, and patient-focused clinical trials environment.

## USA

### Food and Drug Administration (FDA)

#### **FDA guidance on chemistry, manufacturing, and controls (CMC) flexibilities for developing human cellular and gene therapy products for a Biologics License Application (BLA)**

The FDA has issued [final guidance](#) outlining a flexible, risk-based approach to CMC requirements for human cellular and gene therapy (CGT) products being developed for BLAs. The guidance recognises the unique complexity of CGT products, including small patient populations, individualised manufacturing processes, and limited production runs. In response, the FDA is applying regulatory flexibility within existing statutory frameworks to support efficient product development while maintaining standards for safety, quality, and efficacy.

Key elements of the guidance include:

- the use of phase-appropriate manufacturing controls
- risk-based approaches to comparability and validation
- the utilisation of prior knowledge and evolving datasets during development

These flexibilities are intended to facilitate earlier clinical development and streamline regulatory review without compromising product integrity.

Overall, the FDA's approach aims to accelerate patient access to innovative therapies for serious or life-threatening conditions with unmet medical needs, while ensuring that CGT products continue to meet applicable regulatory requirements throughout their lifecycle.

#### **FDA advances drug repurposing to address unmet medical needs**

The FDA has announced an initiative to advance drug repurposing to address unmet medical needs. The agency is seeking input from stakeholders to identify new uses for already approved drugs, including new indications or patient populations, with the

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aim of accelerating access to treatments by leveraging existing safety and scientific data.

The FDA is focused on areas with significant unmet need, including rare diseases, neurodegenerative conditions, metabolic diseases, women's and men's health conditions, and substance use disorders, and is requesting information on candidates for drug repurposing with promising preliminary clinical and preclinical data, particularly where commercial incentives are limited.

The deadline for comments is 11 June 2026. Please find further information, including how to submit comments [here](#).

### **FDA guidance on postapproval pregnancy safety studies**

The FDA has issued a [final guidance](#) on *postapproval pregnancy safety studies* which provides recommendations for sponsors on how to design and conduct studies to evaluate the safety of drugs and biologics when used during pregnancy after marketing approval.

The guidance emphasises the need to collect human safety data in the postmarketing setting, since pregnant individuals are typically excluded from pre-approval clinical trials and aims to support improved product labelling and informed clinical decision-making. The FDA recommends that sponsors use a multi-method, observational, postmarketing strategy including registries, real-world data, and case reports, to generate robust evidence on drug safety during pregnancy which translates into meaningful labelling updates.

## **INTERNATIONAL**

### **International Conference on Harmonisation (ICH)**

#### **ICH M11 Expert Working Group issues final overview presentation**

The ICH M11 Expert Working Group has released a final overview presentation (Step 4 of the ICH harmonisation process) for the M11 guideline on the Clinical electronic Structured Harmonised Protocol (CeSHarP). ICH M11 establishes a globally harmonised framework for clinical trial protocols, consisting of three integrated components: a guideline, a standardised protocol template, and a technical specification to support electronic data exchange.

This initiative addresses longstanding variability in protocol format and content across regions by introducing a common structure and interoperable standards applicable to interventional clinical trials of medicinal products across all phases and therapeutic areas, including pharmaceuticals, biologics, vaccines, drug-device combinations and cell and gene therapy products. Please find further information [here](#).

## **Public consultations**

### **Medicines and Healthcare products Regulatory Agency (MHRA)**

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|    | <b>Title</b>   | <b>Consultation Period</b> | <b>Category</b>        |
|----|--|----------------------------|------------------------|
| 1. | <u>Substances of human origin (SoHO): review of UK legislation</u>       | End date: 17 June 2026     | Open call for evidence |
| 2. | <u>Pre market medical devices regulation – stakeholder impact survey</u> | End date: 19 June 2026     | Open call for evidence |
| 3. | <u>Consultation on the regulation of gene therapy medicinal products</u> | End date: 22 June 2026     | Open consultation      |
| 4. | <u>Draft rare disease therapies regulatory framework</u>                 | End date: 30 July 2026     | Open consultation      |

### European Medicines Agency (EMA)

|    | <b>Title</b>   | <b>Consultation Period</b> | <b>Category</b>     |
|----|--|----------------------------|---------------------|
| 1. | <u>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</u> | End date: 30 June 2026     | Public consultation |

### Food and Drug Administration (FDA)

|    | <b>Title</b>   | <b>Consultation Period</b> | <b>Category</b>     |
|----|--|----------------------------|---------------------|
| 1. | <u>Request for Information: Drug Repurposing for Unmet Medical Needs</u>   | End date: 11 June 2026     | Public consultation |
| 2. | <u>Draft Guidance: Safety Assessment of Genome Editing in Human Gene Therapy Products Using Next-Generation Sequencing</u> | End date: 14 July 2026     | Public consultation |

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