

Regulatory Round-up

September 2026

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UNITED KINGDOM

Medicines and Healthcare Products Regulatory Agency (MHRA)

MHRA regulatory reform amendments tabled in Government's Health Bill

The UK Government has tabled three amendments to the Health Bill aimed at modernising the legislative framework for medicines and medical devices regulation. The proposals would strengthen the MHRA's information-sharing powers with trusted UK and international partners, make medicines and medical devices legislation easier to update in response to scientific and technological advances, and provide enabling powers for the future development of a medical device licensing regime. The amendments are subject to parliamentary scrutiny and would require further development and consultation before implementation.

Key proposed measures include:

- Enhanced information-sharing powers, enabling the MHRA to share specific medicines and medical devices information with trusted UK government bodies and international regulatory partners to support public health, patient safety and innovation, while maintaining safeguards for commercially sensitive and patient data.
- More agile regulatory processes, including provisions that would allow legislation to more readily reflect updates to technical standards and international guidelines. The reforms would also introduce a more proportionate consultation approach, allowing targeted engagement for minor regulatory changes while retaining full public consultation for significant policy reforms.
- Powers to support a future medical device licensing regime, providing the legislative foundation for the development of a streamlined framework for innovative medical devices.

Please find further information [here](#).

Funding opportunity launched to strengthen UK regulatory science and support healthcare innovation

28 August – 27th September 2026

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The UK has [launched](#) a £20 million funding programme to establish four new Centres of Excellence for Regulatory Science and Innovation (CERSIs), aimed at strengthening the UK's regulatory science capability and ensuring regulation keeps pace with advances in healthcare and life sciences. The initiative is a collaboration between the MHRA, the Medical Research Council (MRC), and the Office for Life Sciences (OLS), with centres expected to be funded for up to five years.

The new centres will bring together expertise from academia, industry, healthcare and regulators to address emerging regulatory challenges and support the safe adoption of innovative technologies. Four priority areas have been identified: data-driven approaches and artificial intelligence, prevention and early detection, personalised healthcare and pharmacogenomics, and novel platform technologies.

Building on the success of seven pilot CERSIs, which included work in advanced therapies, digital health and pharmacogenomics, the programme aims to enhance regulatory preparedness for emerging technologies, accelerate patient access to innovation, and reinforce the UK's position as a global leader in life sciences research and investment.

MHRA and Malaysia strengthen partnership on healthcare innovation and research

The MHRA has reaffirmed its commitment to strengthening regulatory collaboration with Malaysia following high-level discussions in Kuala Lumpur with Malaysia's Ministry of Health and leaders from healthcare, regulatory and research organisations. The discussions highlighted opportunities to support healthcare innovation, clinical research and life sciences development through closer international cooperation.

Key areas of potential collaboration include:

- medicines and medical devices regulation
- clinical trials, expert exchange programmes
- sharing regulatory knowledge and best practices
- advancing work on emerging technologies

Both parties also explored opportunities to support more efficient development and approval pathways while maintaining high standards of safety, quality and effectiveness. Please find further information [here](#).

Health Research Authority (HRA)

HRA updates model agreements

The HRA has published updated model agreements for use in health and social care research, helping to streamline study set-up and maintain consistency across research collaborations. The revised agreements reflect ongoing efforts to improve research

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processes and support the timely delivery of studies within the UK research environment. Researchers and sponsors are encouraged to review the updated templates and adopt the latest versions when establishing new studies. Please find further details [here](#).

EUROPE

European Medicines Agency (EMA)

Voluntary data submission pilot to advance innovative alternatives to animal testing

EMA has launched a voluntary data submission pilot to encourage the sharing of data generated using New Approach Methodologies (NAMs), such as organoids, microphysiological systems and computational models. The initiative aims to increase regulatory experience and confidence in these innovative approaches, which have the potential to reduce or replace traditional animal testing in the non-clinical development of medicines. Participants will receive non-binding feedback from experts across the European medicines regulatory network, helping to support the future integration of scientifically robust alternatives into medicines regulation.

The pilot supports EMA's commitment to the 3Rs principles (Replace, Reduce and Refine animal use) and may help accelerate regulatory acceptance of innovative non-animal testing methods, promoting more efficient and ethical medicines development. Please find further information [here](#).

Pre-submission interactions model pilot

EMA has introduced a pilot for a new pre-submission interactions model designed to enhance engagement with medicine developers before marketing authorisation applications are submitted. The pilot aims to improve regulatory planning, facilitate more structured scientific and procedural discussions, and support applicants in preparing high-quality submissions. By strengthening early dialogue between the EMA and applicants, the initiative seeks to promote efficient assessment processes and regulatory predictability for innovative medicines.

The pilot aims to achieve the following:

- Optimise submission readiness
- Identify "premature" applications and assessment issues at an early stage
- Support alignment between applicants, rapporteurs and EMA on realistic submission timelines

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The pre-submission interactions model pilot is open to applicants planning to submit an initial marketing authorisation application between February 2027 and September 2028. Please find further information [here](#).

Guideline on the clinical requirements for medicines intended for the treatment of sickle cell disease

EMA has released a [draft guideline](#) outlining the clinical evidence requirements for medicines intended to treat sickle cell disease (SCD). The draft provides recommendations on study design, patient populations, efficacy endpoints, safety assessments, and long-term follow-up to support the development and evaluation of new SCD therapies.

The guideline is particularly relevant for developers of advanced therapies, including gene therapies, as it aims to establish a consistent regulatory framework for demonstrating clinical benefit in a rapidly evolving treatment landscape. By clarifying expectations for clinical development programmes, the guideline seeks to facilitate the generation of robust evidence and support the assessment of innovative therapies for patients with SCD. The deadline for comments is on 31st January 2027.

Guideline on the clinical requirements for medicines intended for the treatment of thalassaemia

EMA has published a [draft guideline](#) setting out the clinical development requirements for medicines intended to treat thalassaemia. The consultation aims to provide greater clarity on study design, patient populations, efficacy endpoints, safety assessments, and long-term follow-up considerations to support the development and regulatory evaluation of new treatments.

The draft guideline is particularly relevant for developers of innovative therapies, including gene therapies, as it seeks to establish a consistent framework for demonstrating clinical benefit and generating robust evidence in thalassaemia. The guidance is expected to support more predictable clinical development programmes and facilitate regulatory assessment of emerging treatment approaches for this rare inherited blood disorder. The deadline for comments is on 31st January 2027.

EMA updates GVP Module III on Pharmacovigilance Inspections

EMA has [published](#) Revision 2 of Good Pharmacovigilance Practices (GVP) Module III – Pharmacovigilance Inspections, which came into effect on 10 September 2026. The updated guideline strengthens the EU pharmacovigilance inspection framework by incorporating new legal provisions on subcontracting arrangements, clarifying expectations for remote inspections, reinforcing risk-based inspection planning, and updating references to current EU legislation and inspection fee regulations. EMA has also published an [Introductory cover note](#) which reiterates the objectives of

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pharmacovigilance, including the prevention of harm from adverse reactions and the promotion of the safe and effective use of medicines. It also outlines the roles of regulatory authorities, marketing authorisation holders, healthcare professionals, and patients within the EU pharmacovigilance system, while providing an overview of the structure, legal basis, and ongoing development of GVP guidance.

USA

Food and Drug Administration (FDA)

FDA approval: **Fayuvi**

The FDA has [approved](#) **Fayuvi** (rebisuflogene etisparvovec-hopf), the first approved treatment for pediatric patients with mucopolysaccharidosis type IIIA (MPS IIIA), also known as Sanfilippo syndrome Type A. The one-time gene therapy uses an adeno-associated virus serotype 9 (AAV9) vector to deliver a functional copy of the *SGSH* gene, addressing the underlying cause of the disease and enabling production of the deficient sulfamidase enzyme. The approval represents a significant milestone for patients with this rare, progressive neurodegenerative disorder, for whom no disease-modifying treatment had previously been available.

FDA launches expedited Investigational new drug (IND) pilot, begins accepting applications

The FDA has launched the Expedited IND Pilot and is now accepting applications. The pilot is intended to accelerate the transition from drug discovery to first-in-human clinical trials by pairing sponsors with qualified research institutions that can provide scientific expertise during IND preparation. Under the programme, the FDA will evaluate whether earlier and more iterative regulatory interactions can reduce development timelines and facilitate more efficient clinical trial initiation while maintaining regulatory standards for safety and scientific oversight. Applications for participation are being accepted until 30 October 2026. Please find further information [here](#).

FDA updates regulations to advance innovative alternatives to animal testing

The FDA has issued a direct final rule clarifying that non-animal methods may be used, where appropriate, to evaluate the safety of drugs and biological products before human studies. The update reflects advances in science and technology, including the use of human cell-based systems, organ-on-chip platforms, computational models, and other innovative testing approaches. The rule replaces references to “animal tests” and “animal studies” with “nonclinical tests” and “non-clinical studies”, aligning FDA regulations with the Food and Drug Omnibus Reform Act of 2022 (FDORA).

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The FDA also launched a database containing examples of NAMs that have been used in regulatory review, providing sponsors with practical examples of how alternative methods can support drug development. The agency stated that the rule does not prohibit animal studies or alter evidentiary standards but provides greater flexibility in selecting scientifically appropriate approaches to generate safety data.

The clarification may support broader use of human-relevant models and innovative non-animal testing approaches during preclinical development of advanced therapies, potentially improving translational relevance and reducing reliance on traditional animal studies where scientifically justified. Please find further information [here](#).

FDA issues draft guidance on assessing hepatic impairment in drug development

The FDA has published a [draft guidance](#), “Pharmacokinetics in Patients with Impaired Hepatic Function: Study Design, Data Analysis, and Impact on Dosing and Labeling,” providing recommendations for sponsors on evaluating the effects of hepatic impairment on the pharmacokinetics (PK) and, where appropriate, pharmacodynamics (PD) of medicinal products, including therapeutic biological products.

The draft guidance outlines considerations for study design, data analysis, and interpretation of results to support dosing recommendations and product labelling in patients with impaired liver function. The FDA notes that the document is intended to help ensure that the impact of hepatic impairment on drug exposure is appropriately characterised during development and regulatory review.

The guidance is published as a Draft Level 1 Guidance and contains non-binding recommendations. The deadline for Stakeholder feedback is 1st December 2026.

Public consultations

European Medicines Agency (EMA)

	Title	Consultation Period	Category
1.	<u>Guideline on the clinical requirements for medicines intended for the treatment of sickle cell disease</u>	End Date: 31 st January 2027	Draft Guideline

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2.	<u>Guideline on the clinical requirements for medicines intended for the treatment of thalassaemia</u>	End Date: 31 st January 2027	Draft Guideline
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Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	<u>Container Closure Systems for Human Drugs and Biological Products Guidance for Industry</u>	End date: 13 October 2026	Draft guidance
2.	<u>Potency Assessment of Active Immunotherapy Products</u>	End date: 18 November 2026	Draft guidance
3.	<u>Pharmacokinetics in Patients with Impaired Hepatic Function: Study Design, Data Analysis, and Impact on Dosage</u>	End date: 01 December 2026	Draft guidance

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