What's New in Clinical Development Practices & Regulations Quarter 4 – 2024

WORLDWIDE INITIATIVES FOR CLINICAL TRIALS

World Initiatives

In June 2024, World Health Organisation (WHO) held a clinical trials stakeholder consultation, following their initial Global Clinical Trials Forum which took place last year. The aim is to generate a comprehensive Action Plan for a sustainable clinical research infrastructure, through dialog and collaboration among global clinical trial stakeholders. *WHO consults on action plan for sustainable clinical research infrastructure*

EU Initiatives

On 02 October 2024, the European Medicines Agency (EMA) announced new measures to improve the efficiency of the approval process for new medicines. The initiative includes:

- Better use of the network's expert resources
- Streamlining the assessment process
- Encouraging better and more comprehensive application dossiers from applicants at the time of initial submission

Improving efficiency of approval process for new medicines in the EU | European Medicines Agency (EMA)

The EMA has developed a platform to be used by marketing authorisation holders to report shortages of centrally authorised human medicines. After 02 February 2025, using the platform to report shortages will become mandatory. *European Shortages Monitoring Platform enables better monitoring of shortages in the EU | European Medicines Agency (EMA)*

The EMA and the Heads of Medicines Agencies have published an update to their original 5-year strategy (EMANS 2025 for 2021 to 2025) to continue to 2028. *seizing-opportunities-changing-medicines-landscape-european-medicines-agencies-network-strategy-2028-draft_en.pdf*

The European Commission (EC) has published a detailed report on European competitiveness, which includes proposals for the pharmaceutical industry. *The future of European competitiveness_ In-depth analysis and recommendations_0.pdf*

On 02 August 2024, the EMA announced a pilot programme for orphan medical devices. The programme will run until the end of 2025 and during this time, free expert advice will be on offer. The aim of the programme is to establish long-term process for orphan device support. *New pilot programme to support orphan medical devices | European Medicines Agency (EMA)*

All clinical trials authorised under the Clinical Trial Directive (2001/20/EC) need to transition to the Clinical Trial Regulation by 31 January 2025. The latest metrics from ACT EU (Accelerating Clinical Trials in the EU) initiative show that the transition is progressing well. *ACT EU KPI Report_November_2024.pdf*

UK Initiatives

The Medicines and Healthcare products Regulatory Agency (MHRA) has proposed legislative changes for clinical trials. The overall aim is for a patient-focused and risk-based approach, which supports innovative research. *Consultation on proposals for legislative changes for clinical trials - GOV.UK*

The MHRA has proposed new medical device legislation with the view to strengthen patient safety. The legislation will be delivered through four Statutory Instruments, including: *The Medical Devices (Post-market Surveillance Requirements) (Amendment) (Great Britain) Regulations 2024* and *Consultation on Medical Devices Regulations: Routes to market and in vitro diagnostic devices - GOV.UK*

From 01 January 2025, the MHRA will, under the Windsor Framework, be responsible for licencing all medicines in Northern Ireland. This will simplify the licencing of medicines in the UK and make the supply of medicines to the UK market easier. *MHRA Windsor Framework Hub - GOV.UK* The MHRA has produced webinars detailing the main changes: *MHRA Windsor Framework Webinar Recordings - GOV.UK*

On the 28 August 2024, the Voluntary Scheme for Branded Medicine Pricing, Access, and Growth Investment Programme (VPAG) opened. It will channel significant investment into the UK's health and life sciences sector. *2024 voluntary scheme for branded medicines pricing, access and growth - GOV.UK*

GUIDANCE DOCUMENTS

2024 Declaration of Helsinki

On 21 October 2024, the World Medical Association (WMA) announced the adoption of the 2024 revision of the Declaration of Helsinki. The updated declaration aims for a participant focused approach. The revision includes:

- Increasing the protection of vulnerable populations
- Increasing participant privacy protection
- Additional statements relating to free and informed consent

WMA Declaration of Helsinki – Ethical Principles for Medical Research Involving Human Participants – WMA – The World Medical Association

ICH Guideline E11A 'Pediatric Extrapolation'

On 21 August 2024, ICH E11A reached Step 4 ('Adoption by the Regulatory Members of the ICH Assembly'). *ICH_E11A_Guideline_Step4_2024_0821.pdf* and *ICH_Step_4_Presentation_ICHE11A_2024_0923_0.pdf*

Final Guidance

- July 2024: FDA 'Real-World Data – Assessing Electronic Health Records and Medical Claims Data to Support Regulatory Decision-Making for Drug and Biological Products' *54336794fnl-RWDAssessingElectronicHealthRecordsandMedicalClaimsDataSupportRegulatoryDecision.pdf*
- August 2024: EMA 'Guidelines on Good Pharmacovigilance Practices' *28-gvp-introductory-cover-note-m-xvi-rev-3-m-xvi-add-ii-i-rev-5_en.pdf*
- August 2024: FDA 'Optimising the Dosage of Human Prescription Drugs and Biological Products for the Treatment of Oncologic Diseases' *2023-632 - Dose Optimization Guidance.pdf*
- September 2024: EMA 'Reflection paper on establishing efficacy based on single-arm trials submitted as pivotal evidence in a marketing authorisation application' *reflection-paper-establishing-efficacy-based-single-arm-trials-submitted-pivotal-evidence-marketing-authorisation-application_en.pdf*
- September 2024: EMA 'Data Protection Notice for EudraVigilance Human (EV)' *european-medicines-agencys-data-protection-notice-eudravigilance-human-ev_en.pdf*
- September 2024: EC 'Health Technology Assessment (HTA) Guidance on Validity of Clinical Studies' *hta_clinical-studies-validity_guidance_en.pdf*
- September 2024: FDA 'Conducting Clinical Trials with Decentralized Elements' *57746937-fnl.pdf*
- September 2024: FDA 'Bioresearch Monitoring Technical Conformance Guide' *BIMO Conformance-Guide-Submission-of-NDA-BLA-Content-for-BIMO-V3.1.pdf*
- October 2024: FDA 'Electronic Systems, Electronic Records and Electronic Signatures in Clinical Investigations Q&A' *58358119fnl.pdf*
- October 2024: FDA 'Core Patient-Reported Outcomes in Cancer Clinical Trials' *core_patient-reported_outcomes_in_cancer_clinical_trials_508_0.pdf*

Draft Guidance

- September 2024: FDA 'Integrating Randomized Controlled Trials for Drug and Biological Products Into Routine Clinical Practice' *56255267dft_0.pdf*
- September 2024: FDA 'Considerations for Generating Clinical Evidence from Oncology Multiregional Clinical Development Programs' *draft_mrct_guidance_september_2024.pdf*
- September 2024: FDA 'Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle' *guidance-vlppi-tplc.pdf*

ARTIFICIAL INTELLIGENCE (AI)

In September 2024, the EMA issued a set of guiding principles describing how EU medicines regulators can use large language models (LLMs) in a safe, responsible and effective manner. LLMs are a category of AI whose application can significantly support medicines regulators in their tasks and processes. *guiding-principles-use-large-language-models-regulatory-science-medicines-regulatory-activities_en.pdf*

In September 2024, the FDA published a digital health and AI glossary. The glossary is a compilation of commonly used terms and definitions in the digital health, AI and machine-learning space. *FDA Digital Health and Artificial Intelligence Glossary – Educational Resource | FDA*

Thank you for taking the time to read this Industry Update from S-cubed

Prepared by:
Christina Hägglund, QA Manager
S-cubed Ltd
Email: christina.hagglund@s-cubed.co.uk
Website: www.s-cubed-global.com