What's New in Clinical Development Practices & Regulations Quarter 2 – 2023

EU PHARMACEUTICAL LEGISLATION

Proposal to Revise the EU's Pharmaceutical Legislation

The European Commission (EC) has recently published a proposal for a new Directive and a new Regulation which will revise and replace the existing general pharmaceutical legislation: *Reform of the EU pharmaceutical legislation (europa.eu)*

Additionally, there is a proposal for a Council Recommendation to step up the fight against antimicrobial resistance (AMR): *EU Action on Antimicrobial Resistance (europa.eu)* Including a FAQ document: *Q&A Antimicrobial Resistance (europa.eu)*

Further information will be available on the S-cubed website in due course.

UK PHARMACEUTICAL LEGISLATION & UPDATES

UK Clinical Trial Regulations

The Medicines and Healthcare products Regulatory Agency (MHRA) has announced an overhaul of UK's Clinical Trial Regulation, with the development of new legislation. The legislation aims to establish a robust baseline from which the UK can develop new regulatory approaches which are in line with emerging technologies and science. *MHRA to streamline clinical trial approvals in biggest overhaul of trial regulation in 20 years - GOV.UK (www.gov.uk)* and *Consultation on proposals for legislative changes for clinical trials - GOV.UK (www.gov.uk)*

The MHRA is to receive £10M from HM Treasury to fast-track patient access to cutting-edge medical products. *MHRA to receive £10m from HM Treasury to fast-track patient access to cutting-edge medical products - GOV.UK (www.gov.uk)*

A new model Clinical Investigation Agreement (mCIA) and a new Clinical Research Organisation model Clinical Investigation Agreement (CRO-mCIA) have been issued. From November 2023, these templates are the only ones accepted in IRAS submissions. *IRAS Help - Preparing & submitting applications - Templates for supporting documents (myresearchproject.org.uk)*

National Institute for Health and Care Research (NIHR) has launched a new National Health Service (NHS) app, aiming to make it easier for people in England to take part in health and care research. *Be Part of Research now available through the NHS App | NIHR*

The MHRA blog gives the definition of a temporary halt in a clinical trial and explains how regulators, ethics committees, investigators and others should be notified. *When is a clinical trial halt not a clinical trial halt? - MedRegs (blog.gov.uk)*

CLINICAL TRIAL INITIATIVES & PILOT SCHEMES

Accelerated Clinical Trials in the EU (ACT EU)

One of the priority actions of ACT EU is to develop and launch a multi-stakeholder platform in order to gain a better understanding of stakeholders' perspectives on a wide range of clinical trial aspects. *priority-action-3-concept-paper-eu-multi-stakeholder-platform-improving-clinical-trials-accelerating_en.pdf (europa.eu)*

EMA – Priority Medicines Scheme (PRIME)

The EMA is introducing a number of new and additional features to PRIME, following a review of the scheme's first five years. The features will add further opportunities and strengthen the scheme. *New features further strengthen Priority Medicines scheme (PRIME) | European Medicines Agency (europa.eu)*

EMA – Pilot Project on Raw Data

Raw data (also referred to as standardised study data) is individual patient data from clinical studies in a structured format from which standardised analysis are derived.

In July 2022, the EMA launched a Proof-of-Concept pilot project on the analysis of 'raw data' from clinical trials. The EMA has now issued a Q&A document in response to FAQs. *Q&A for industry on raw data pilot (europa.eu)*

Dynamic Regulatory Assessment (DRA)

A recent European Federation of Pharmaceutical Industries and Associations (EFPIA) blog suggests that DRA may offer a viable alternative to the classic model of authorising new medicines in the EU. The blog explains how DRA can be piloted, and ultimately implemented, in the EU to benefit patients, regulators and drug developers. *Dynamic Regulatory Assessment will support more efficient treatment development for patients: the time to pilot is now (Guest Blog) (efpia.eu)*

RWE, RWD & CLINICAL OUTCOME ASSESSMENTS (COA)

Darwin EU – First Studies Completed

As detailed in previous Newsletters (Q2 2022 and Q1 2023), the EMA has established a network of Real World Data (RWD) sources across the EU to give the EMA and EU Competent Authorities valid and trustworthy Real World Evidence (RWE).

Four studies using RWD from across the EU aiming at better understanding diseases, populations and the uses and effects of medicines have now been completed. *DARWIN EU® has completed its first studies and is calling for new data partners | European Medicines Agency (europa.eu)*

Diversity in Clinical Trial Populations

There is a need to move away from historically defined clinical trial eligibility criteria, which may be overly restrictive. This is to prevent population subgroups being unnecessarily excluded from clinical trials.

RWD can help define study eligibility criteria and ultimately ensure the new drug is safe and effective in all the relevant population groups. *Improving Diversity in Clinical Trials by Using Real-world Data to Define Eligibility Criteria | Oncology | JAMA Oncology | JAMA Network*

Patient Focused Drug Development

The FDA has released draft guidance (comments to be submitted by 05 July 2023) titled 'Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making'. It is part of a series of documents detailing how to collect and submit patient experience data for medical product development and regulatory decision making. *Patient-Focused Drug Development: Incorporating Clinical Outcome Assessments Into Endpoints For Regulatory Decision-Making (fda.gov)*

DIGITAL HEALTH TECHNOLOGIES (DHT)

EFPIA Workshop

A virtual workshop, held in December 2022, has concluded that under the right circumstances, DHT may benefit clinical trials in several ways, including establishing meaningful endpoints and ensuring patient-centric outcome measures. *pod final.docx (efpia.eu)*

New Framework Document

In March 2023, the FDA issued 'Framework for the use of DHT in Drug and Biological Product Development'. *Framework for the Use of DHTs in Drug and Biological Product Development (fda.gov)*

GUIDELINES

EC Q&As on the Protection of Personal Data when Using CTIS
March 2023 (part of ACT EU priority actions): *ACT EU_Q&A on protection of Commercially Confidential Information and Personal Data while using CTIS version 1.1 (europa.eu)*

EMA 'Single-armed Trials as Pivotal Evidence for the Authorisation of Medicines in the EU'
April 2023: *Single-arm trials as pivotal evidence for the authorisation of medicines in the EU | European Medicines Agency (europa.eu)*

EMA 'Guideline on Computerised Systems and Electronic Data in Clinical Trials'
March 2023: *Guideline on computerised systems and electronic data in clinical trials (europa.eu)*

EMA Actions to Support the Development of Medicines for Children
February 2023: *Actions to support the development of medicines for children | European Medicines Agency (europa.eu)* and *guidance-stepwise-pip-pilot_en.pdf (europa.eu)*

EMA Policy 'Multilingualism on the EMA Website and in External Communication'
February 2023: *Multilingual Policy_EN (europa.eu)*

FDA Q9(R1) Quality Risk Management
May 2023: *Q9(R1) Quality Risk Management (fda.gov)*

FDA Draft Guidance 'Decentralized Clinical Trials for Drugs, Biological Products and Devices'
May 2023 (distributed for comment purposes only): *Decentralized Clinical Trials for Drugs, Biological Products, and Devices Draft Guidance for Industry, Investigators, and Other Stakeholders (fda.gov)*

FDA Q&A Guide 'A Risk-Based Approach to Monitoring of Clinical Investigations'
April 2023: *A Risk-Based Approach to Monitoring of Clinical Investigations--Questions and Answers (fda.gov)*

FDA COVID-19 Related Guidance The notice indicates which of its COVID-19 related guidance documents will no longer be in effect once the period of COVID-19 public health emergency ends.
March 2023: *Federal Register :: Guidance Documents Related to Coronavirus Disease 2019 (COVID-19)*

FDA Draft Guideline 'Clinical Trial Considerations to Support Accelerated Approval of Oncology Therapeutics'
March 2023: *Clinical Trial Considerations to Support Accelerated Approval of Oncology Therapeutics Guidance for Industry (fda.gov)*

FDA Draft Guidance 'Research Involving Children as Subjects and Not Otherwise Approvable by an IRB: Process for Referrals to FDA and OHRP'
March 2023: *Research Involving Children as Subjects and Not Otherwise Approvable by an IRB: Process for Referrals to FDA and OHRP*

FDA Generic Drug User Fee Act (GDUFA) – Science and Research Report
2022: *CDER FY 2022 GDUFA Science and Research Report (fda.gov)*

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

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