Regulatory Round-up

September 2022

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EUROPE

European Commission (EC)

Labelling requirements for IMPs amended

Following a 1-month feedback period on the draft published in June this year the European Commission (EC) adopted on 6 September the revised Annex VI to Regulation (EU) No 536/2014 (Clinical Trials Regulation, CTR) as regards labelling requirements for IMPs. The Regulation will enter into force on the twentieth day following its publication in the Official Journal of the European Union and will be binding and directly applicable in all Member States.

More information is provided under Unauthorised medicinal products used in clinical trials (labelling rules).

Clinical Trials Regulation (EU) 536/2014- version 6.2 Questions and Answers

The latest version of the Q&A's in relation to the CTR guideline has been updated. Please find document here.

European Medicines Agency (EMA)

EMA pilot offers enhanced support to academic and non-profit developers of ATMPS

The EMA is launching a pilot scheme to support the translation of basic research developments into medicines that could make a difference in patients' lives in the European Economic Area (EEA). The pilot is open to academic sponsors and non-profit organisations that are developing advanced therapy medicinal products (ATMPs).

During the pilot, EMA will provide enhanced regulatory support for up to five selected ATMPs that address unmet clinical needs and are solely developed by academic and non-profit developers in Europe. EMA will guide the participants through the regulatory process with the aim to optimise the development of the ATMPs, starting from best practice principles for manufacturing to planning clinical development that meets regulatory standards.

Potential developer candidates can contact their national competent authority or EMA via advancedtherapies@ema.europa.eu to express their interest in participating in the pilot or to receive more information.

Clinical Trials Information System (CTIS) Webinar: 9 Months on and going forward

On the 16th of November 2022, the EMA will host a webinar on CTIS. The focus of this virtual information day 9 months after the CTIS launch is to share some practical advice regarding transitioning clinical trials from the Clinical Trials Directive (2001/20/EC) to the Clinical Trial Regulation (536/2014) as well as best practices on user management.

The event will feature the first insights from assessments of clinical trial applications and system metrics on the usage of CTIS. It will also outline the importance of understanding timelines in CTIS, as will upcoming training opportunities and events.

Please find details on the agenda and how to register here.

United Kingdom

Medicines and Healthcare products Regulatory Agency (MHRA)

The UK changes SUSAR Reporting for IMPs

The MHRA has now retired the eSUSAR website in favour of Individual Case Safety Reports (ICSR) Submissions, providing users "a more robust, stringent, and transparent way of expediting suspected unexpected serious adverse drug reactions (SUSARs) from Clinical Trials of Investigational Medicinal Products" (IMPs). The eSUSAR website used for the submission of SUSAR reports to the MHRA was decommissioned at the end of September 2022 and only SUSARS via the ICSR Submissions portal will be acceptable from 1 October 2022.

Read more in the MHRA Inspectorate blog Decommission of eSUSAR at GOV.UK.

USA

Food and Drug Administration (FDA)

Electronic Submission Template for Medical Device 510 (k) Submissions Guidance for Industry and Food and Drug Administration Staff

The announcement of the guidance was published in the **Federal Register** on September 22, 2022.

This guidance provides further standards for the submission of premarket notification (510(k)) submissions by electronic format, a timetable for the establishment of these standards, and criteria for waivers of and exemptions from the requirements to meet a statutory requirement. This guidance is also intended to represent one of several steps in meeting the FDA's commitment to the development of electronic submission templates to serve as guided submission preparation tools for industry to improve submission consistency and enhance efficiency in the review process.

Accelerated BLA Approval- Skysona

The US Food and Drug Administration (FDA) has awarded accelerated approval for Skysona (elivaldogene autotemcel), also known as eli-cel, for use in boys aged 4 to 17 years with early, active cerebral adrenoleukodystrophy (CALD). Skysona can be used to slow the neurological decline in boys caused by CALD. The approval letter can be found here.

Skysona has previously been approved in the EU last year, however, it was withdrawn shortly after as the company exited the region. In the US, the Skysona BLA was reviewed by the FDA under Priority Review and has previously been granted Orphan Drug Designation, Rare Paediatric Disease Designation, and Breakthrough Therapy Designation.

New FDA guidance offers an ethical roadmap for including children in clinical trials

Today, the U.S. Food and Drug Administration issued draft guidance that, when finalized, will provide the agency's perspective on the ethical considerations for including and protecting children in clinical trials. The draft guidance is intended to assist industry, sponsors and institutional review boards (IRBs) when considering the enrollment of children in clinical investigations of drugs, biological products and medical devices. The guidance was developed by the FDA's Office of Pediatric Therapeutics with contributions from the Center for Drug Evaluation and Research, the Center for Biologics Evaluation and Research and the Center for Devices and Radiological Health.

The public can provide comments on the draft guidance, however these should be submitted within 90 days to ensure that the agency considers them when finalizing the draft guidance.

National Institutes of Health (NIH)

NIH initiative to systematically investigate and establish function of every human gene

The National Institutes of Health is launching the Molecular Phenotypes of Null Alleles in Cells (MorPhiC) programme, managed by the National Human Genome Research Institute, to better understand the function of every human gene and generate a catalog of the molecular and cellular consequences of inactivating each gene. Please find more details of this programme here.

Public consultations

Medicines and Healthcare products Regulatory Agency (MHRA)

	Title	Consultation Period	Category
1.	*Proposals for changes to the Medicines and Healthcare Products Regulatory Agency's statutory fees*	*End Date: 23rd November 2022*	*Public Consultation*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Human Prescription Drug and Biological Products — Labeling for Dosing Based on Weight or Body Surface Area for Ready-to-Use Containers — "Dose Banding"*	*End Date: 19 Sep 2022*	*Draft Guidance*
2.	*Ethical Considerations for Clinical Investigations of Medical Products Involving Children*	*End date: 27 Dec 2022*	*Draft Guidance*