Regulatory Round-up

June 2023

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EUROPE

European Directorate for the Quality of Medicines (EDQM)

Joint USP-EDQM Symposium on "Pharmaceutical Reference Standards"

The USP has announced it is co-organising a conference with the European Directorate for the Quality of Medicines & HealthCare (EDQM), Council of Europe, entitled "Pharmaceutical Reference Standards". The event will take place in Rockville, Maryland (USA) from 27 to 28 September 2023. The programme will cover the use and establishment of reference standards, with a focus on reference standards for biologicals and small molecules, as well as regulatory expectations and harmonisation across different regions. Please find further information here.

2022 Highlights – EDQM annual report now available

EDQM has just published its 2022 annual report, providing an overview of its activities and achievements, as well as the present state of EDQM/Council of Europe public health initiatives. Some of these activities were the creation of a new Working Party on High Throughput Sequencing (HTS) and the publishing of the 5th edition of 'The Guide to the quality and safety of tissues and cells for human application' guidance document, complemented by the EDQM Microbiological Risk of Contamination Assessment (MiRCA) online tool. The EDQM annual report can be downloaded here.

European Medicines Agency (EMA)

EMA publishes review of its studies on the use of real-world evidence in regulatory decision-making

Real-word evidence (RWE) studies led by regulators can support both pre-authorisation and post-approval assessments of EMA's scientific committees, working parties and national competent authorities. The report titled "*Real-world evidence framework to support EU regulatory decision-making*" published by the EMA takes stock of the experience gained to date in conducting real-world data (RWD) studies aimed at addressing the needs of EU regulators as well as external stakeholders including health technology assessment (HTA) bodies and payers' organization. This initiative overseen by the EMA-HMA Big Data Steering Group, EMA and European Medicines Regulatory Network (EMRN) working towards setting up a sustainable framework that enables the uses and establishes the value of RWE in decision-making across the entire product lifecycle. Please find further information here.

Accelerating Clinical Trials in the EU Priority Action 4 (ACT EU PA04) to organize a multi-stakeholder workshop on ICH E6 R3 public consultation

As part of the "*Accelerating Clinical Trials in the EU (ACT EU) multi-annual workplan 2022-2026*" and acknowledging the important role of ICH E6 on "Good Clinical Practice", a multi-stakeholder workshop on ICH E6 R3 public consultation is being organised by ACT EU Priority Action 4 (PA4). The virtual workshop will take place on the 13th and 14th of July 2023. Further information here.

Tenth industry stakeholder platform on research and development support

The 10th regular meeting between regulators and representatives of industry stakeholder organisations will take place on the 11th of July 2023. It addresses all areas of evidence generation along the medicine's life-cycle and related to development support activities, such as scientific advice and qualification, as well as specifics for paediatric and orphan medicines. Please find further information on this event here.

United Kingdom

Medicines and Healthcare products Regulatory Agency (MHRA)

Windsor Framework medicines announcement

The MHRA has made an announcement regarding the Windsor Framework.The Windsor Framework provides a long-term solution for the supply of medicines into Northern Ireland ensuring that medicines can be approved and licensed in the UK by the Medicines and Healthcare products Regulatory Agency (MHRA). The following new measures will take effect on 1 January 2025:

- New medicines for the UK market will be authorised by UK authorities, and UK packaging must carry a clearly legible 'UK only' label to be allowed onto the UK market, including in Northern Ireland
- These products will only be able to be sold in the UK, and will not be available on the market in Ireland, or elsewhere in the EU
- Medicines entering Northern Ireland will not display features required under the EU Falsified Medicines Directive (FMD) including 2D barcodes and serialisation numbers that are compliant with the EU FMD Directive
- The MHRA will expect anti-tamper devices to remain on all medicine packaging.

The MHRA will continue to allow manufacturers to supply medicines in legacy EU packaging until 31 December 2024. Packs in existing packaging already on the UK market, and within the supply chain, will remain until the date of their expiry.

Guidance on the labelling requirements and MHRA authorisations on the requirements for medicines to be placed on the market in Northern Ireland will be provided in due course.

In advance of the new arrangements , the Northern Ireland MHRA Authorised Route (NIMAR) will continue to support the supply of medicines into Northern Ireland.

Until the Windsor Framework comes to effect on 1 January 2025, the Government brings into force a bridging mechanism to allow companies to supply Northern Ireland with innovative medicines (those presently subject to the EU Centrally Authorised Procedure) for up to 6 months when the MHRA licenses a product before the European Medicines Agency (EMA).

USA

Food and Drug Administration (FDA)

FDA Approves First Gene Therapy for Treatment of Certain Patients with Duchenne Muscular Dystrophy

On the 22nd of June 2023 the FDA approved Elevidys, the first gene therapy for the treatment of pediatric patients 4 through 5 years of age with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene who do not have a pre-existing medical reason preventing treatment with this therapy. Elevidys, is a recombinant gene therapy designed to deliver into the body a gene that leads to production of Elevidys micro-dystrophin, a shortened protein (138 kDa, compared to the 427 kDa dystrophin protein of normal muscle cells) that contains selected domains of the dystrophin protein present in normal muscle cells. Please find further information here.

FDA Approves First Gene Therapy for Adults with Severe Hemophilia A

The FDA has approved Roctavian, an adeno-associated virus vector-based, single dose gene therapy for the treatment of adults with severe hemophilia A without pre-existing antibodies to adeno-associated virus serotype 5 detected by an FDA-approved test. Hemophilia A is a rare genetic bleeding disorder that occurs due to a mutation on the gene which produces factor VIII (FVIII), a protein that enables blood to clot.

FDA Approves First Cellular Therapy to Treat Patients with Type 1 Diabetes

FDA has approved the first allogeneic pancreatic islet cellular therapy for the treatment of adults with Type 1 diabetes who are unable to approach target HbA1c because of current repeated episodes of severe hypoglycemia despite intensive diabetes management and education.

Public consultations

Medicines and Healthcare products Regulatory Agency (MHRA)

	Title	Consultation Period	Category
1.	*ICH Reflection on "GCP Renovation": Modernization of ICH E8 and Subsequent Renovation of ICH E6*	*End date: 31 Aug 2023*	*Public consultation*
2.	*Final Concept Paper ICH E6(R3): Guideline for Good Clinical Practice Dated 17 November 2019 Endorsed by the Management Committee on 18 November 2019*	*End date: 31 Aug 2023*	*Public consultation*

EUROPEAN MEDICINES AGENCY (EMA)

	Title	Consultation Period	Category
1.	*Reflection paper on establishing efficacy based on single arm trials submitted as pivotal evidence in a marketing authorisation*	*End date: 30 Sept 2023*	*Reflection paper*

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	*Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations Guidance for Industry*	*End date: 17 Jul 2023*	*Draft guidance*
2.	*Pediatric Drug Development: Regulatory Considerations — Complying With the Pediatric Research Equity Act and Qualifying for Pediatric Exclusivity Under the Best Pharmaceuticals for Children Act Guidance for Industry*	*End date: 17 Jul 2023*	*Draft guidance*
3.	*Decentralized Clinical Trials for Drugs, Biological Products, and Devices*	*End date: open*	*Draft guidance*

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International Conference on Harmonisation (ICH)

	Title	Consultation Period	Category
1.	*ICH HARMONISED GUIDELINE GOOD CLINICAL PRACTICE (GCP) E6(R3)*	*End date: September 2023*	*Draft guidance*

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