

Regulatory Round-up

August 2022

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EUROPE

European Commission (EC)

Document Revision – Manufacture of Sterile Medicinal Products, GMP Annex 1

On the 25th of August, the EC [announced](#) a revision to the document “Manufacture of Sterile Medicinal Products”. The GMP/GDP Inspectors Working Group and the PIC/S Committee jointly recommend that the current version of Annex 1, on manufacturing sterile medicinal products, be revised to reflect changes in regulatory and manufacturing environments. The new guideline should clarify how manufacturers can take advantage of new possibilities deriving from the application of an enhanced process understanding by using innovative tools as described in the ICH Q9 and Q10 guidelines. The revision of Annex 1 should also take into account related changes in other GMP chapters and annexes as well as in other regulatory documents. The revised guideline will seek to remove ambiguity and inconsistencies and will take into account the advances in technologies.

European Medicines Agency (EMA)

Accelerating Clinical Trials in the EU: Publication of 2022-2026 workplan

On the 30th of August, the European Commission (EC), HMA and EMA published the [2022-2026 Workplan of the initiative – Accelerating Clinical Trials in the EU](#) (ACT EU).

[ACT EU](#), launched in January 2022, seeks to transform how clinical trials are initiated, designed and run. The aim is to further develop the EU as a focal point for clinical research, promote the development of high-quality, safe and effective medicines, and to better integrate clinical research in the European health system. ACT EU will strengthen the European environment for clinical trials, whilst maintaining the high level of protection of trial participants, data robustness and transparency that EU citizens expect.

EMA reports a steady rise in clinical trial applications registered in the CTIS portal

Nearly 195 clinical trial applications (CTAs) have been submitted to the European Medicine Agency’s (EMA) Clinical Trial Information System (CTIS) portal from 1st Jan 2022 through to 31st July 2022, marking a substantial increase from a month earlier, according to the 4th Edition of the EMA’s “Key performance indicators (KPIs) to monitor the European clinical trials environment” [report](#). However, during the same period, most applications (224) were still being registered in the older EudraCT database,

28th July – 31st August 2022

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under the Clinical Trial Directive (CTD). To date only one ATMP has had a decision under the new Clinical Trial Regulation (CTR), whilst ten ATMPs have had decisions under the CTD.

EMA is encouraging clinical sponsors to submit their applications to the CTIS portal, asserting that the benefits of this new system are improved information sharing, collective decision-making on clinical trials, increased transparency on clinical trials and ensuring high safety standards for patients. The system, which is being implemented as part of the Clinical Trials Regulation (CTR) allows sponsors to apply to run clinical trials throughout the EU at the same time using the same documentation.

UK

Medicines and Healthcare products Regulatory Agency (MHRA)

Consultation on proposals for changes to the Medicines and Healthcare products Regulatory Agency's statutory fees

The aim of this [consultation](#) is to seek the views of stakeholders on proposals to amend statutory fees from the Medicines and Healthcare products Regulatory Agency (MHRA). The proposed adjustments fall into 3 categories:

- A 10% indexation uplift across statutory fees
- A further uplift for 61 significantly under recovering fees to achieve cost recovery
- The introduction of 22 new fees for services that require cost-recovery since the last fee changes in 2016/2017 for medicines and 2017/2018 for medical devices

The fee proposals set out in this consultation are designed to ensure the MHRA is resourced to provide the high-quality service that patients, the public and industry want and expect, and to achieve full cost recovery in line with HM Treasury's principles on Managing Public Money. This will ensure the MHRA is financially sustainable in the long term, enabling the Agency to deliver a responsive, innovative and efficient regulatory service that protects and improves patient and public health by facilitating access to high-quality, safe, effective and innovative medical products.

The consultation will run from Wednesday 31 August 2022 and close on Wednesday 23 November 2022. The proposed implementation date for these changes is 1 April 2023.

USA

Food and Drug Administration (FDA)

FDA OTAT Town Hall: Gene Therapy Chemistry, Manufacturing, and Controls

The FDA's Center for Biologics Evaluation and Research ([CBER](#)) Office of Tissues and Advanced Therapies ([OTAT](#)) is hosting a virtual town hall to answer stakeholder

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questions related to gene therapy chemistry, manufacturing, and controls. This event is the first in a series to answer questions from stakeholders about a variety of topics on which OTAT has regulatory oversight. In this town hall, subject matter experts from OTAT's Division of Cell and Gene Therapy will answer questions related to CMC for gene therapy product development.

The meeting will take place on Thursday 29th September, and participants can register [here](#). Please see this [FDA guidance document](#) for more information about CMC data to support gene therapy product applications.

FDA Approves Beti-Cel Gene Therapy for β -Thalassemia

The FDA has approved bluebird bio's gene therapy betibeglogene autotemcel (beti-cel), now marketed as ZYNTEGLO, the first cell-based gene therapy for the treatment of adult and pediatric patients with beta-thalassemia who require regular red blood cell transfusions. Beti-cel is an autologous, lentiviral gene therapy that delivers a modified form of the β -globin gene into patients' hematopoietic stem cells ex vivo which are then infused back into the patient to enable corrected levels of haemoglobin production without regular RBC transfusions. The therapy will only be available at qualified treatment centres with expertise in stem cells, cell and gene therapy, and β -thalassemia. The FDA news release article can be read [here](#).

FDA /PQRI Workshop on the Regulatory Framework for Distributed and Point of Care Pharmaceutical Manufacturing: An Opportunity for DM/POC Stakeholder Engagement

The FDA and [PQRI](#) have announced that they will host an FDA/PQRI Workshop on the Regulatory Framework for Distributed and Point of Care Pharmaceutical Manufacturing on November 14 – 16, 2022, as a virtual event. This FDA/PQRI Workshop will bring together leaders from regulatory agencies, industry, and academia to discuss critical topics in distributed manufacturing and point-of-care manufacturing. The Workshop aims to facilitate interaction among DM/POC stakeholders on critical areas for the development and implementation of DM and POC technologies including terminology, technical challenges to adoption, operation of Pharmaceutical Quality Systems, good manufacturing practice expectations, and the unique challenges and considerations that apply to complex biologics.

This virtual workshop is open to anyone interested in DM/POC, specifically to learn from experts and contribute ideas.

Registration can be found [here](#).

Visit the [Workshop website](#) for more information.

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INTERNATIONAL

International Conference on Harmonisation (ICH)

Addendum to S1B reaches Step 4 of the ICH Process

The Addendum to the ICH S1B Guideline reached *Step 4* of the ICH process on 4th August 2022, is integrated with the original guideline and published as ICH S1B(R1) Testing for Carcinogenicity of Pharmaceuticals. The new Addendum expands the evaluation process for assessing the human carcinogenic risk of pharmaceuticals by introducing an additional approach that is not described in the original S1B Guideline.

The application of this integrative approach reduces the use of animals by the 3R (reduce/refine/replace) principles and shifts resources to focus on generating more scientific mechanism-based carcinogenicity assessments while continuing to promote the safe and ethical development of new pharmaceuticals.

Further information can be found on the [S1B\(R1\) page](#), including the *Step 4* Informational Presentation for download.

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Public consultations

Food and Drug Administration (FDA)

	Title	Consultation Period	Category
1.	<u>Considerations for Rescinding Breakthrough Therapy Designation</u>	End Date: 23 Aug 2022	Draft Guidance
2.	<u>Human Prescription Drug and Biological Products — Labeling for Dosing Based on Weight or Body Surface Area for Ready-to-Use Containers — “Dose Banding”</u>	End Date: 19 Sep 2022	Draft Guidance

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: 23 rd November 2022	Public Consultation

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