Regulatory Round-up

May 2023

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EUROPE

European Commission (EC)

Proposal for a Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency

On 26 April 2023 the European Commission published the proposal for the envisaged revision of the EU pharmaceutical legislation.

Directive 2001/83 (Directive 2001/83/EC of the European Parliament and of the Council of 6 November 2001 on the Community code relating to medicinal products for human use) and Directive 2009/35/EC are to be replaced by a new Directive. The proposal for a new Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, amending Regulation (EC) No 1394/2007 and Regulation (EU) No 536/2014 and repealing Regulation (EC) No 726/2004, Regulation (EC) No 141/2000 and Regulation (EC) No 1901/2006.

The aim of this proposal is to make EU pharmaceutical legislation more agile, flexible and adaptable to the needs of patients and businesses in the EU.

European Medicines Agency (EMA)

Review of transparency rules for the EU Clinical Trials Information System (CTIS)

The European Medicine Agency (EMA) has opened a public consultation to review the transparency rules for the publication of information on clinical trials submitted through the Clinical Trials Information System (CTIS) in the European Union (EU). The review of the CTIS transparency rules seeks to stimulate the discussion on the best possible approaches to balance clinical trial transparency with confidentiality requirements while simplifying the modalities of use of CTIS to improve user experience and reduce the risk of data breaches. Stakeholders are invited to send their comments via an online form by midnight (CET) on 28 June 2023. Please find further details here.

United Kingdom

Medicines and Healthcare products Regulatory Agency (MHRA)

Extension of the European Medical Device Regulations transitional period: Implication for CE marked medical devices on the Great Britain market

26 April – 28th May 2023

The European Parliament has voted to adopt an extension of the transition period for the EU Medical Device Regulations (EU MDR; Regulation (EU) 2017/745), introducing more time to certify certain medical devices. These changes made to the EU MDR will apply automatically in Northern Ireland under the terms of the Northern Ireland Protocol. The MHRA is carefully considering the implications of these revisions for the acceptance of CE marked medical devices on the Great Britain (GB) market. A device with a valid CE mark can be placed on the GB market until 30 June 2023. That would include certificates valid under the latest EU's revised transitional arrangements, if they are adopted as proposed. Further information on the implementation of the future Medical Device Regulation can be found here.

MHRA and Genomics England to launch pioneering resource to better understand how genetic makeup influences the safety of medicines

The MHRA has announced it will be the first drug safety regulator in the world to pilot its own genetic 'biobank', launching 1 June 2023.

A new genetic research resource, known as a 'biobank', will be piloted by the MHRA and Genomics England to better understand how a patient's genetic makeup impacts on the safety of their medicines. The Yellow Card biobank, which will contain genetic data and patient samples, will operate alongside the MHRA's Yellow Card reporting site for suspected side effects and adverse incidents involving medicines and medical devices. Please find further details on this pilot here.

MHRA announces new recognition routes to facilitate safe access to new medicines with seven international partners

The MHRA has announced that a new regulatory recognition routes for medicines will be established using approvals from Australia, Canada, the European Union, Japan, Switzerland, Singapore and the United States. As a result, patients will have access to safe and effective medicines that have been approved by trusted regulatory partners in other countries. Please find further details here.

New regulatory pathway set to support safe patient access to innovative medical technologies

The Innovative Devices Access Pathway (IDAP), will be launched later in 2023, and to be operated by the Medicines and Healthcare products Regulatory Agency (MHRA), the National Institute for Health and Care Excellence (NICE), Health Technology Wales (HTW) and Scottish Health Technology Group (SHTG).

The aim of this new programme is to bring innovative technologies and solutions to the forefront of the National Health Service (NHS), through a new, integrated support service for developers that will include enhanced opportunities for engagement. Please find further information here.

MHRA launches public consultation on ICH Good Clinical Practice Guideline which encourages innovation in clinical trials

The MHRA is holding a 3-month long public consultation on the Good Clinical Practice (GCP) guideline revised by the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH).

The consultation seeks views from the public and other stakeholders on whether the resolutions set out in the ICH reflection and concept papers have been achieved, in addition to soliciting detailed comments on the text. The aim of the ICH reflection and concept paper update is to further encourage a proportionate, risk-based approach to the quality management of clinical trials.

Pro-innovation Regulation of Technologies Review: Life Sciences and the government response

The Pro-innovation Regulation of Technologies review explores the overarching barriers to new technologies faced by regulators and those who interact with regulation in numerous sub-sectors within the life sciences as well as explores opportunities for changes in the regulation of specific technologies.

This report provides recommendations on:

• Common regulatory challenges that impact innovation and growth across multiple areas of life sciences,

•Regulation of medicines, advanced therapeutics and medical devices in the UK, including with regard to the Medicines & Healthcare products Regulatory Agency (MHRA) and the wider regulatory system through the National Institute for Health and Care Excellence (NICE).

• Opportunities for better regulation in the life sciences beyond human health, including novel foods, waste valorisation and cell free systems.

USA

Food and Drug Administration (FDA)

FDA issues a new guidance document to reduce the risk of human immunodeficiency virus transmission by blood and blood product

The new guidance entitled "*Recommendations for Evaluating Donor Eligibility using Individual Risk-Based Questions to Reduce the Risk of Human Immunodeficiency Virus Transmission by Blood and Blood Products*" issues this May, supersedes previous guidance entitled, "*Revised Recommendations for Reducing the Risk of Human Immunodeficiency Virus Transmission by Blood and Blood Products*" dated April 2020. The recommendations contained in this guidance apply to the collection of blood and blood components, including Source Plasma. Please find further details here.

FDA releases of 2 draft guidances on Pediatric Drug Development

The FDA has announced the availability of the 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 draft guidance and the Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations draft guidance which address selected clinical, scientific, and ethical issues involved in developing drugs, including biological products, for pediatric use when such drug products are subject to the Pediatric Research Equity Act (PREA) and/or the Best Pharmaceuticals for Children Act (BPCA). These draft guidance documents are intended to assist industry in obtaining the data and information necessary to support the approval of drug products in pediatric populations.

Vyjuvek: BLA approval

The FDA has granted approval for Krystal Biotech's Vyjuvek (beremagene geperpavec-svdt) to treat dystrophic epidermolysis bullosa (DEB) in patients aged six months and above.

Vyjuvek is a non-invasive, topical, re-dosable gene therapy that delivers functional human COL7A1 gene copies to offer wound healing.

It is the first re-dosable gene therapy as well as the first and only FDA-approved treatment for both recessive and dominant types of DEB, a rare and serious genetic disease affecting the skin and mucosal tissues.

Rare Disease Endpoint Advancement (RDEA) Pilot Program Public Workshop

The FDA is establishing a Rare Disease Endpoint Advancement (RDEA) Pilot Program to support novel endpoint efficacy development for drugs that treat rare diseases. The RDEA Pilot Program fulfills a commitment under the Prescription Drug User Fee Act (PDUFA VII).

Academic investigators, pharmaceutical and biotechnology companies, patient advocacy organizations, and anyone interested in developing rigorous endpoints for rare disease drug development are encouraged to attend.

A workshop will be held on 7-8 of June 2023. Further details including how to register can be found here.

National Institutes of Health (NIH)

NIH launches $140 million effort to investigate genetic variation in normal human cells and tissues

The National Institutes of Health is launching a new program, the Common Fund's Somatic Mosaicism Across Human Tissues (SMaHT) Network, that aims to transform our knowledge of how much genetic variation there is in cells and tissues throughout our bodies. By cataloging somatic mosaicism in normal human tissues, the SMaHT

Network will provide foundational knowledge that will enable research on the role that somatic genetic variation plays in human development and aging, as well as a wide range of diseases and disorders. Please find further details here.

National Human Genome Research Institute (NHGRI) releases a new human "pangenome" reference

Researchers from the National Human Genome Research Institute (NHGRI) have released a new high-quality collection of reference human genome sequences that captures substantially more diversity from different human populations than what was previously available. The new "pangenome" reference includes genome sequences of 47 people, with researchers pursuing the goal of increasing that number to 350 by mid-2024. The current reference actually includes 94 distinct genome sequences, with a goal of reaching 700 distinct genome sequences by the completion of the project. Further information on the human pangenome reference can be found here.

The Foundation for the National Institutes of Health (FNIH) Announces Selection of Eight Rare Diseases for the Bespoke Gene Therapy Consortium Clinical Trial Portfolio

The Foundation for the National Institutes of Health (FNIH) has announced its plans to prioritise eight rare diseases to provide industry standards for manufacturing, preclinical testing and product analytical testing for gene therapy development.

The eight diseases that will make up the clinical trial portfolio are:

- Charcot-Marie-Tooth disease type 4J
- Congenital Hereditary Endothelial Dystrophy
- Morquio A Syndrome
- Multiple Sulfatase Deficiency
- NPHP5 Retinal Degeneration
- Propionic Acidemia (PCCB)
- Retinitis pigmentosa 45
- Spastic paraplegia 50

The Foundation's Accelerating Medicines Partnership (AMP) Bespoke Gene Therapy Consortium (BGTC) will study these eight conditions by focusing on gene therapies using adeno-associated vectors (AAV).

INTERNATIONAL

International Conference on Harmonisation (ICH)

Switzerland: New Declaration of the Responsible Person for foreign Manufacturers

Swissmedic has published a new "*Guidance document GMP compliance by foreign manufacturers*" and a form "*Declaration by the Responsible Person for foreign manufacturers*" with the aim of clarifying the conditions for the submission of an audit report as evidence of GMP compliance by foreign manufacturers from countries whose

GMP control system is not considered equivalent by Switzerland. Please find further details here.

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.	*Phage therapy active substances and medicinal products for human and veterinary use (5.31)*	*End Date: June 2023*	*Public consultation*
2.	*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*
3.	*Transparency rules for the operation of the Clinical Trials Regulation and its Clinical Trials Information System*	*End Date: 28/06/2023*	*Public consultation*

Food and Drug Administration (FDA)

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*
4.	*Methods and Approaches for Capturing Post-Approval Safety and Efficacy Data on Cell and Gene Therapy Products*	*End date: 26/05/2023*	*Public meeting*