International Society for Cellular Therapy ISCT TELEGRAFT

# Europe Legal and Regulatory Affairs Watchdog Update

REGULATORY WATCHDOG

This European watchdog is providing information relevant to ISCT areas of concern, including: 1) recent and upcoming workshops or meetings, 2) recently published regulatory documents and 3) guidelines currently opened for public consultation.

**Workshops for micro, small and medium-sized enterprises (SME) "Focus on non-clinical aspects"** organised by EMA on 3 October 2016 (London, UK)
This workshop aimed to provide an overview of non-clinical data requirements for the authorisation of human medicinal products, how to address these during the medicinal product development, and detail the approaches for biological and advanced therapy medicinal products (ATMPs). A regulatory brief on the new PRIME (PRIority MEdicines) scheme was also included in the programme.
The take home message of the session on ATMPs was that the non-clinical development for this kind of products can usually not strictly follow the guidelines. A risk and scientific-based approach should be applied to justify alternative options and the agency is open to discuss with developers.
All presentations will be published on the Agency's website beginning of November and the videos of the event end of November / beginning of December. You will be able to find them via this link:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2016/05/event_detail_001284.jsp&mid=WC0b01ac058004d5c3

**Third IABS Conference on Best Practice in Cell Therapy Medicinal Product Manufacturing & Testing** organized by the International Alliance for Biological Standardization (IABS) on 2 & 3 November 2016 (London, UK)
The 2016 Cell Therapy conference will identify the key issues to be addressed for the manufacture of cell therapies and provide scientific consensus on selected aspects to inform the drafting of future guidance. The meeting will bring together representatives from industry, academia, health services and regulatory bodies. It is intended that the conference output should provide core elements that will be useful in establishing international consensus on the requirements for manufacture of cell based medicines and enable progress towards a potential future WHO endorsed guidance.

More information is available on the meeting website:

http://3rd-iabs-conference-on-cell-therapy.iabs.org/index.html

**Committee for Advanced Therapies (CAT) workshop on "Scientific and regulatory challenges of genetically modified cell-based cancer immunotherapy products"** organised by EMA on 15 & 16 November 2016 (London, UK)

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The CAT is organising a two-day workshop to discuss the scientific developments and regulatory requirements for products manufacture and testing, non-clinical studies and clinical development of genetically modified cell-based cancer immunotherapy products. These novel cancer immunotherapy treatments based on genetically modified T-cells are being developed and tested in clinical trials in a variety of cancers, but there are still scientific and regulatory challenges or hurdles to overcome to bring these innovative products to the market.
Registration to attend the workshop in-person is now closed but the event will be broadcasted from the Agency's website. You will be able to find it via this link:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2016/08/event_detail_001318.jsp&mid=WC0b01ac058004d5c3

**Adaptive pathways workshop** organised by EMA on 8 December 2016 (London, UK). The EMA is organising this workshop in collaboration with the European Commission to gather the views and proposals from stakeholders on the adaptive pathways approach, in light of the practical experience gained during the pilot project EMA ran between March 2014 and August 2016, and to plan the next steps in the exploration of this concept. Adaptive pathways is a scientific concept of medicines development and data generation intended for medicines that address patients' unmet medical needs.

Registration is still opened until 1st Nov 2016 and the event will also be broadcasted from the Agency's website:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2016/09/event_detail_001324.jsp&mid=WC0b01ac058004d5c3

**Targeted stakeholder consultation on the draft Guidelines on Good Manufacturing Practice for Advanced Therapy Medicinal Products.** The European Commission (EC) consulted stakeholders involved in the development, manufacture and/or commercialisation of ATMPs. A first consultation on this topic was launched in 2015. On the basis of the comments received during the consultation, as well as input from consultation with the European Medicines Agency and competent authorities in the Member States, the Commission services have developed draft Guidelines on Good Manufacturing Practice specific to ATMPs, which was opened to a second consultation from 28 June to 26 September 2016.
ISCT, which already answered to the first consultation, sent its contribution to this second consultation, welcoming the flexibility introduced with the risk-based approach applicable to numerous areas, specificities for investigational/early phase ATIMPs and for non-substantially manipulated ATMPs. Nevertheless, ISCT highlighted its concern with the possibility that this guideline for GMP requirements will create a double standard, particularly on the national level. Whereas a unique position on the character of this document (to be standalone guidance or to be an annex of the current GMP guideline) was not specifically recommended, ISCT stressed that its integration in EudraLex as well as its inclusion in PIC/S is critical.

Official information from the EC, including comments from each stakeholder, summary of comments will be made available through this link:

http://ec.europa.eu/health/human-use/advanced-therapies/developments/index_en.htm

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**Good Cell Culture Practice for stem cells and stem-cell-derived models** published in August 2016. The first guidance on Good Cell Culture Practice dates back to 2005. This document expands this to aspects of quality assurance for in vitro cell culture focusing on the increasingly diverse cell types and culture formats used in research, product development, testing and manufacture of biotechnology products and cell-based medicines. It provides a set of basic principles of best practice. This workshop report is considered as a first step toward a revised GCCP 2.0.

The full workshop report is available at:

http://www.altex.ch/resources/Pamies_of_160823_v2.pdf

**Outcomes of the IMI consultation on advanced therapies** published in September 2016
In April 2016, the Innovative Medicines Initiative (IMI) launched a public consultation on ATMP with the goal of identifying the potential of IMI as a platform for enhancing ATMP research and development. The consultation mainly focused on the following questions:

- Have the key challenges that can be addressed through collaborative, public-private initiatives been properly identified?
- Which of the proposed potential initiatives should be prioritised?
- Are any areas missing?
- What are the key European or national initiatives that IMI shall synergise with?

The outcomes of the consultation are summarised in the following document:

http://www.imi.europa.eu/sites/default/files/uploads/documents/ATMPconsultation2016/ATMP_consultation_feedbacksummary.pdf

They have also been presented at the IMI stakeholder forum on 28 and 29 September. The presentations and video recordings from the whole forum are accessible on the IMI website:
http://www.imi.europa.eu/events/2016/06/24/imi-stakeholder-forum-2016

**Comprehensive overview of global initiatives on medicine regulation** published by EMA on 13 October 2016. The report lists all international projects and provides international regulatory agencies with comprehensive details on the number and scope of global initiatives that can support decision-making regarding future engagement, prioritisation and coordination. The aim of the mapping exercise was to raise awareness of on-going international regulatory activities, help establish a basis for a more strategic coordination to avoid duplication of efforts, and identify possible gaps.

The mapping was carried out by EMA on behalf of the International Coalition of Medicines Regulatory Authorities (ICMRA). The report is available at:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2016/10/news_detail_002619.jsp&mid=WC0b01ac058004d5c1

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**Guidelines opened for public consultation**

- *Guideline on the requirements for quality documentation concerning biological investigational medicinal products in clinical trials* until 31 December 2016

http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2016/07/WC500209618.pdf

Note: ATMPs are excluded from the scope of this guideline; nevertheless the guideline can be an interesting support as no equivalent guideline is dedicated to the quality documentation for investigational ATMPs.

- *ICH E11 (R1) guideline on clinical investigation of medicinal products in the pediatric population* until 13 April 2017

http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2016/10/WC500214185.pdf