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) upcoming events (workshops, meetings…), 2) recently published regulatory documents, 3) public consultations and guidelines currently opened for comments 4) follow-up on previously addressed events and 5) recall of albumin.

1) **Annual workshop of the European network of paediatric research at the EMA (Enpr-EMA)** organised on 16 May 2017 (London, UK)

The 9th annual workshop of the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA) aims to bring relevant stakeholders together to discuss requirements, barriers and opportunities for high-quality clinical studies in children. Among the highlights of this year's workshop are the recent efforts to increase cooperation between the European Union and the United States to facilitate global paediatric trials and medicine developments. Registration deadline is 28 April 2017.

More information including the draft meeting's agenda can be found here:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2017/02/event_detail_001394.jsp&mid=WC0b01ac058004d5c3

Meeting on PRIME experience after 1 year organised by EMA on 19 May 2017 (London, UK)

The EMA launched the PRIME (PRIority Medicines) scheme in March 2016. The scheme provides early and enhanced support to medicines that have the potential to address patients' unmet needs. This meeting is organised by EMA to review the experience gained with PRIME one year after it was launched. The aim of the meeting is to receive feedback from users and potential users of the scheme, provide information on how the rules on eligibility have been applied and what types of support applicants have received so far, and discuss practical examples that illustrate the benefits of PRIME and how it builds on the existing tools. Registration deadline is 21 April 2017. The event will also be broadcasted from the Agency's website.

More information including the draft meeting's agenda can be found here:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2017/03/event_detail_001407.jsp&mid=WC0b01ac058004d5c3

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Webinar on implementation of EMA policy on publication of clinical data (Policy 0070) held on 23 March 2017

This webinar was directed at industry associations to provide update on the implementation of the Clinical Data Publication (Policy 0070) and on the related guidance.

The presentations and the video of the meeting can be found here:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2017/03/event_detail_001420.jsp&mid=WC0b01ac058004d5c3

2) Framework and action plan of collaboration between the European Medicines Agency and academia published by EMA on 3 April 2017

In order to reinforce the collaboration with academia, the EMA has developed a framework to formalise, structure, and further develop interactions with the academic community in the context of the European medicines regulatory network.

The framework and an action plan for the next three-years can be found here:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2017/03/news_detail_002722.jsp&mid=WC0b01ac058004d5c1

In addition, EMA also published a new web page for academia providing links to content that is likely to be of interest, and a section describing the way EMA interacts with academia, with more detail on the collaboration framework and action plan and useful resources for academics:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/audience/alp_audiencetype_000006.jsp&mid=

Adjustment of fees for applications to EMA from 1 April 2017

General, non-pharmacovigilance fees payable to the EMA by applicants and marketing-authorisation holders have been adjusted to 2016 inflation rates and increased by 1.2%.

More information can be found here:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2017/03/news_detail_002721.jsp&mid=WC0b01ac058004d5c1

UK - Guidance on Common Issues in Clinical Trial Applications published by the MHRA on 23 March 2017

This guidance identifies common issues with validation and assessment of clinical trial applications and how to avoid them. Indeed, more than half of all applications for clinical trial authorisation received by the MHRA require additional information to be submitted before they are considered approvable. Many of the requests for further information are common and avoidable if available guidance is followed or if a satisfactory justification for not following the applicable guidance is provided in the application.

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The guidance itself is divided in five separate documents detailing the top issues related to: validation of the application, non-clinical, clinical and quality information about the product as well as a list of the useful resources. It can be found here:

https://www.gov.uk/government/publications/common-issues-identified-during-clinical-trial-applications

France - Enforcement of the European regulation on clinical trials on medicinal products: assessment one year into the pilot phase published by ANSM on 25 January 2017

The new Clinical Trials Regulation (Regulation (EU) No. 536/2014) was adopted in 2014 with the primary goal of providing more efficient, coordinated assessments for Clinical Trial Applications (CTAs) in the EU using a central portal. In order to prepare for enforcement of the regulation the French Medicines Agency (ANSM) began in 2015 a pilot phase with industrial and academic CTA sponsors as well as collaboration with ethics committees (EC).

According to ANSM, the pilot phase indicates a positive outcome for relevant stakeholders with strong participation and constructive exchanges between sponsors, the EC and the ANSM.

The ANSM report can be found here:

http://ansm.sante.fr/var/ansm_site/storage/original/application/6536cc922b13704dd92c049158accc2b.pdf

Revised ICH E6 (R2) guideline on Good Clinical Practice (EMA/CHMP/ICH/135/1995)

The guideline was amended to encourage the implementation of more efficient approaches to clinical trial design and conduct oversight in light of advances in electronic data recording and reporting. It will come into effect on 14 June 2017 and can be found here:

http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2009/09/WC500002874.pdf

In February 2017, ICH issues a training presentation on that guideline that can be found here:

http://www.ich.org/fileadmin/Public_Web_Site/ICH_Products/Guidelines/Efficacy/E6/E6_R2__Step_4_Presentation_06Feb2017.pdf

3) Public consultation on revised policy on access to documents launched by EMA and available until 18 May 2017

The EMA has launched a new consultation on the proposed revision to its policy on access to documents, which describes the rules to grant access to the documents held by the EMA. The new version extends the scope of the policy to include both scientific and corporate documents and takes into account the Agency's proactive approach to transparency that has led to the publication of many more documents on the EMA website since 2010.

More information can be found here:

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http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2017/02/news_detail_002697.jsp&mid=WC0b01ac058004d5c1

Draft reflection paper providing an overview of the current regulatory testing requirements for medicinal products for human use and opportunities for implementation of the 3Rs, open to consultation until 31 May 2017

This reflection paper has been developed by the CHMP as a follow-up to the draft guideline published in October, 2014. It provides an overview (in tabular format) of the main animal tests required for the regulatory testing of medicinal products for human use. It includes information on opportunities for limiting animal testing that can already be implemented as well as information on opportunities that may become available in the future. A section is dedicated to ATMPs.

More information about this consultation can be found here:

http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500216428&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

Public consultation on the Roadmap for the evaluation of the EU blood and tissues and cells legislation (closed)

The Commission is currently carrying out an evaluation of the EU blood and tissues and cells legislation. This is the first formal evaluation of this legislation since the adoption of the basic Acts in 2002 (blood) and 2004 (tissues and cells). This evaluation aims to assess whether the legislation has achieved its original objectives and whether it is still fit for purpose. The evaluation will consist of several steps starting with a Roadmap and including a study by an external contractor and extensive consultation of stakeholders. The final evaluation report is expected to be published by the end of 2018. The Roadmap released by EC in January 2017 is the first step in the evaluation process and outlines the purpose, content and scope of the evaluation.

More information on this evaluation and feedback from stakeholders can be found here:

https://ec.europa.eu/health/blood_tissues_organs/policy/evaluation_en

Guidelines opened for comments:

- *Q&A on implementation of risk based prevention of cross contamination in production and 'Guideline on setting health based exposure limits for use in risk identification in the manufacture of different medicinal products in shared facilities' (EMA/CHMP/CVMP/SWP/169430/2012)* available until 30 April 2017

http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500219500&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

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- *Concept paper on developing a guideline on quality requirements of medicinal products containing a device component for delivery or use of the medicinal product* available until 16 May 2017

http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500221747&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

- *Draft guideline on multiplicity issues in clinical trials* available until 30 June 2017

http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500224998&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

- *Draft guideline on good clinical practice compliance in relation to trial master file (paper and/or electronic) for content, management, archiving, audit and inspection of clinical trials* available until 11 July 2017

http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500225871&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

- *Draft reflection paper on statistical methodology for the comparative assessment of quality attributes in drug development* available until 31 March 2018

http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500224995&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

Tell OECD how health systems can improve sustainable access to innovative pharmaceutical therapies

The OECD is undertaking an international stakeholder dialogue to improve patient access to innovative pharmaceutical treatments and ensure the sustainability of health spending as well as continued innovation that meets patient needs. In this capacity, the OECD is inviting submissions to identify issues with the current system, to understand which topics are of most importance to stakeholders, and to canvass new ideas. Submissions will be used to inform the synthesis of evidence and develop recommendations for governments of OECD Member countries.

Contributions are sought from all sectors and stakeholders until 1 May. Both individuals and organisations are encouraged to submit. Contributions are not limited to any specific set of countries. Contributors may reflect on a country specific, regional and/or international perspective.

Visit http://www.oecd.org/els/health-systems/Sustainable-access-to-innovative-therapies-Online-consultation.htm for details on the process.

4) SME information day on the new clinical trial regulation (regulation EU no. 536/2014) organised by EMA on 20 March 2017 (London, UK)

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This event aimed to provide an overview of the key features and objectives of the new clinical trial regulation. It also covered the future clinical trial authorisation process, the functionalities of the EU CT portal and database, transparency aspects of the new regulation, and safety reporting requirements.

The presentations and the video of the meeting can be found here:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2017/01/event_detail_001385.jsp&mid=WC0b01ac058004d5c3

EMA adaptive pathways workshop held on 8 December 2016 (London, UK)

Adaptive pathways is a scientific concept of medicines development and data generation intended for medicines that address patients' unmet medical needs. The EMA organised 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.

In addition to the presentations, the video of the workshop and stakeholders interviews are also available here:

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 development of 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.

After a first round of consultation in 2015, a second consultation was opened from 28 June to 26 September 2016. A summary of the stakeholder responses to this second consultation has been published by the EC in December 2016 and can be found here:

http://ec.europa.eu/health/human-use/advanced-therapies/2016_pc_atmp_en

The publication of the final guideline, that should be a standalone document, was expected by Q1 2017 but is still pending.

5) The Human Tissue Authority of the UK has issued a Regulatory Alert due to the recall of albumin by Biotest. Low levels of contamination have been found. Batch numbers and expiry dates are provided. More information is available here:
https://www.hta.gov.uk/policies/regulatory-alert-0012017-%E2%80%93-recall-human-albumin