ISCT International Society for Cellular Therapy
TELEGRAFT
MEMBER NEWSLETTER

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) other topics.

EMA ANNUAL REPORT 2016 PUBLISHED

In 2016, the Agency recommended a marketing authorization for 81 medicines for human use, including 27 new active substances. Many of these innovative substances build on the advances made in biomedical science and have the potential to make a difference for patients.

Approximately half of the applicants who were granted a positive opinion for their medicine had received scientific advice from EMA during the development phase of their product. As a result of the safety monitoring of all medicines marketed in the European Union (EU), the product information for over 300 medicines for human use was updated on the basis of new safety data. The revised information allows patients and healthcare professionals to make informed decisions based on the latest evidence when using or prescribing the medicine.

EMA's annual report also highlights some of the Agency's main projects, initiatives and achievements in 2016. These include the launch of PRIME (PRIority MEdicines), an initiative to support the development of medicines that address unmet medical needs and the policy on the publication of clinical trial data for new medicines, a groundbreaking new initiative that turned EMA into one of the most transparent medicines regulators worldwide. Other developments showcased in the report include new ways to collect data on medicines such as big data, patient registries and real world data, and EMA's contribution to addressing public health challenges.

For ATMPs, the report shows a further increase in the number of adopted ATMP classifications. In 2016, one ATMP marketing authorization application was received and two ATMP were recommended for marketing authorization: Strimvelis and Zalmoxis. And 40% of the Innovation Task Force meetings were on innovative ATMPs.

The report can be found here.

Guidelines on Good Manufacturing Practice for Advanced Therapy Medicinal Products

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The European Commission (EC) consulted stakeholders involved in the development, manufacture and/or commercialization of ATMPs. After a first round of consultation in 2015, and a second round of consultation in 2016, the CAT endorsed the new Guidelines at its June 2017 meeting. The EC is expected to publish the Guidelines.

PUBLIC CONSULTATION FOR THE EVALUATION OF EU LEGISLATION ON BLOOD, TISSUES AND CELLS (until 31 AUG 2017)

The purpose of this consultation is to support a comprehensive evaluation of the Union legislation on blood and tissues and cells - Directives 2002/98/EC and 2004/23/EC respectively and their implementing (technical) Directives, examining their functioning across the EU. In particular the consultation aims to gather views on the extent to which the Directives have met their original objectives and whether they remain fit for purpose. The evaluation is expected to provide a sound evidence base which will be used to consider the need for any changes to the legislation.

The Questionnaire for Citizens can be found here.

The Questionnaire for Administrations, Associations and Organizations can be found here.

Public consultation for Concept Paper on the revision of the Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells (until 31 OCT 2017)

The CAT recommends a multidisciplinary revision of the current guideline with the aim to:

- reflect significant development and experience gained since the publication of the current guideline
- reassess the validity of the existing guidance text in light of the existing experience
- provide, where needed, specific quality, non-clinical and clinical guidance for the development of CAR-T cells and related products,
- include considerations on the genome-editing tools when applied for the ex vivo genetic modification of cells

The concept paper can be found here.

It is anticipated that a draft revised guideline will be available by Q1 2018.

Further guidelines open for comments:

- **Draft reflection paper on statistical methodology for the comparative assessment of quality attributes in drug development** (until 31 MAR 2018): here
- **Concept paper on revision of the guideline on clinical development of vaccines** (until 30 SEP 2017): here

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RESULTS OF FP7 PROJECTS : SMALL-POPULATION RESEARCH AND REGULATORY APPLICATIONS

The European Union funded three projects, Asterix, IDeAl and InSPiRe, within the Seventh Framework Programme (FP7) to develop new methodology on design and analysis of small population clinical trials since 2013. The aim was to translate and promote results and novel methodologies into tangible recommendations to advance the clinical research and development of medicines and new treatments for patients with a rare disease and personalized medicine. The results and recommendations for application were discussed by international representatives of patient organizations, pharmaceutical industry, academic statisticians and clinical trial methodologists, clinical researchers, pharmaceutical industry and regulators.

The presentations can be found here.

Report of Workshop of the European Network of Paediatric Research at the EMA

The European Network of Paediatric Research at the EMA (Enpr-EMA) held its 9th annual workshop on 16 May 2017 at the premises of the EMA in London, UK.

Enpr-EMA is a network of research networks, investigators and centers with recognized expertise in performing clinical studies in children, with the aim to foster high-quality ethical research in children, providing expertise and support regarding quality, safety and efficacy of medicines to be used in children. The main objective of the annual workshop is to foster interaction and communication between all stakeholders: networks' representatives, pharmaceutical industry, regulators and patient/parent organizations.

This year's workshop emphasized the need for international, global collaboration and provided a dedicated session on interaction between the EU and the US for global pediatric research.

The report and presentations can be found here.

Report of Meeting on PRIME experience after 1 year

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 was organized by EMA to review the experience gained with PRIME one year after it was launched. The aim of the meeting was 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.

The report and presentations can be found here.

EMA delays implementation of new Clinical Trial Regulation to 2019

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Due to technical difficulties concerning the development of new IT systems for the European Clinical Trial Portal, the 'go-live' date for the new regulation has been postponed by the EMA until 2019. The agency had previously indicated that the new clinical trial regulation, finalized in May 2014, would come into force by October 2018.

The EU clinical trial portal and database supports the modernization of the processes for authorization and oversight of clinical trials in the EU. This new system is a significant shift from the current process involving independent clinical trial authorization (CTA) by each member state, into a single portal for submission and maintenance of applications and authorizations, coordinated assessment and supervision. The EMA is working closely with its IT service provider to ensure that corrective measures are implemented and will closely monitor its progress. The EMA management board will provide a progress update at its next meeting in October 2017.

The press release can be found here.

OTHER TOPICS

The European Commission granted a marketing authorization valid throughout the European Union for Spherox on 10 July 2017

Spherox provided by CO.DON AG (Germany) is a medicine used to repair defects to the cartilage in the knee in adults who are experiencing symptoms (such as pain and problems moving the knee). It is used where the affected area is no larger than 10 cm^2. Spherox is a type of advanced therapy medicine called a 'tissue engineered product'. This is a type of medicine containing cells or tissues that have been manipulated so that they can be used to repair, regenerate or replace tissue. Spherox contains spheroids (spherical aggregates) of chondrocytes, cells found in healthy cartilage.

Further information can be found here.

Meeting of the International Council for Harmonization (ICH) in Montreal, Canada on 27 May to 1 June 2017

The ICH Assembly approved the China Food and Drug Administration (CFDA) as a new Regulatory Member, and Pharmaceutical Inspection Co-operation Scheme (PIC/S) as a new Observer. Moreover, two new topics were adopted: the revision of the ICH E8 guideline on general considerations for clinical trials, and extrapolation for pediatric medicines. The next ICH meeting will be held in November 2017 in Geneva, Switzerland.

Further information can be found here.