



Europe Legal and Regulatory Affairs

Watchdog Update



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 and 4) follow-up on previously addressed events and 5) other topics.

1) ISCT European Meeting (Firenze, Italy – 12-14th September 2018)

Information can be found [here](#).

2) New Regulation 2018/781 on the definition of the concept 'similar medicinal product'

Commission Regulation (EU) 2018/781 of 29 May 2018 amending Regulation (EC) No 847/2000 lays down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concepts "similar medicinal product" and "clinical superiority".

Regulation (EC) No 847/2000 provides a definition of 'similar medicinal products' and a number of cases defining what kind of products are to be regarded as similar for the purposes of the application of the incentives provided under Regulation (EC) No 141/2000 on orphan medicinal products. However, due to major developments in the field of biological medicines including advanced therapy medicinal products (ATMPs), the definitions of Regulation (EC) No 847/2000 required adaption to technical progress. This amending Regulation, prepared in collaboration with the EMA, was discussed with Member States in the Standing Committee meeting and published for several open consultations.

A Q&A document has been developed with a view to address questions that have been raised by developers of ATMPs regarding the application of the concept of similarity in an ATMP setting. The Q&A document will be updated in light of experience accumulated with the assessment of this type of medicinal products.

The new Regulation can be found [here](#).

The Q&A on ATMP aspects can be found [here](#).

**EDQM guide on How to read a CEP**

EDQM published a guide on How to read a Certificate of suitability to the Monographs of the European Pharmacopoeia (CEP). This document has been created with the intention of clarifying the information to be concluded from a Certificate of suitability to the Monographs of the European Pharmacopoeia (CEP) for industry and the competent authorities. It provides definition and content of the different CEPs. For instance, a Transmissible Spongiform Encephalopathy (TSE) CEP certifies that the substance complies with monograph 1483 of the Ph. Eur. “*Products with risk of transmitting agents of animal spongiform encephalopathies*”, and reminds that this type of CEP “*does not certify that the quality of the substance is suitably controlled by a specific Ph. Eur. Monograph*”.

The guide can be found [here](#).

3) Public consultation for :

Draft guideline on the responsibilities of the sponsor with regard to handling and shipping of investigational medicinal products for human use in accordance with good clinical practice and good manufacturing practice
(until 31 AUG 2018)

This guideline complements the Delegated Regulation (EU) No 2017/1569 of 23 May 2017, on good manufacturing practice (GMP) for investigational medicinal products (IMP) and arrangements for inspections, and lays down the principles for the two-step release and shipping of the investigational medicinal products by the qualified person and the sponsor. The guideline also describes the areas of interface between the manufacturer and the sponsor and the required contractual agreements.

The draft Guideline can be found [here](#).

4) Report on EMA Workshop on CAR T Registries

Chimeric Antigen Receptor T-cell (CAR T-cell) therapies pose particular challenges for regulators and healthcare providers. The benefit-risk profile of these products need to be assessed as part of their integration into our healthcare systems. In that context, EMA hosted a workshop in February to explore the opportunities and challenges of using existing patient registries to support CAR T-cell therapy benefit-risk evaluations and post-authorisation follow-up.

The participants with clinical, regulatory, or development experience with CAR T-cell products included representatives from two large registry holders; the European Society for Blood and Marrow Transplantation (EBMT) and the United States-based Centre for International Blood and Marrow Transplant Research (CIBMTR). The observations/recommendations made during the workshop were compiled in a report.



Key priorities from this workshop include:

- to collect a set of core commonly-defined data elements – Appendix 1 provides the proposed data to be collected for efficacy and safety, its priority, the current capture status in EBMT and CIBMTR.
- to harmonise data element definitions across registries,
- to establish measures that ensure data are collected systematically with appropriate verification and quality assurance,
- to ensure arrangements are in place to permit data sharing, and
- to improve communications between registry holders, regulators and marketing authorisation holders and applicants.

The full Report can be found [here](#).

The Appendix on proposed data to be collected can be found [here](#).

5) Other topics:

EMA report on 2 years PRIME experience

The European Medicines Agency (EMA) launched the PRiority MEdicines (PRIME) scheme in March 2016. The scheme provides early and enhanced scientific and regulatory support to medicines that have the potential to significantly address patients' unmet medical needs.

In the document, EMA continues to report on the scheme after two years of experience, in particular on how the criteria for eligibility have been applied and the types of support that applicants have received so far. The report reviews practical examples that illustrate some of the benefits of PRIME and how the scheme makes optimal use of existing tools supporting regulatory and scientific advice.

- 177 requests for eligibility to PRIME received and assessed since launch in March 2016.
- Quality of applications received is good; guidance can be considered sufficiently clear for applicants to understand the scope and requirements of PRIME.
- Requests have been received in a wide range of therapeutic areas, being the majority for oncology or haematology products.
- 21% of requests have been accepted in the scheme, totalling 36 medicines accepted into PRIME scheme. The rate eligible/granted indicator has remained stable since launch.
- Of the total 36 medicines included 30 are for rare diseases.
- High number of requests for advanced therapy medicines, representing 40% of products granted eligibility.

The full PRIME report can be found [here](#).

**EMA Management Board meeting: Brexit update**

EMA and the Netherlands have signed the Seat Agreement which describes the relationship among the Dutch Government and the Agency, its bodies and its employees once they start operating in the Netherlands. The Agreement will provide assurance that EMA staff members and their families who need to relocate early to the Netherlands can do so under the protection of the Seat Agreement immediately.

The Board heard an update on progress with the redistribution of the UK's centrally authorised product portfolio for human and veterinary medicines. Re-allocation of over 370 products to new (co-) rapporteurs from the EU27 Member States, plus Iceland and Norway, has been successfully concluded and marketing authorisation holders were informed of the new rapporteurships at the end of April. The new (co-)rapporteurs will only take full responsibility for the reallocated products as of 30 March 2019 when the United Kingdom leaves the European Union (EU).

EMA Management Board meeting: Clinical Trial Portal and Database update

The Board was updated on the quality and progress of the ongoing development of the EU Clinical Trial and Portal and Database, both of which are being carefully monitored. The Board heard that development of the auditable release of the portal and database (release 0.7) is nearing completion. The release is now in an intensive phase of testing. Where needed, the development resource is being adjusted to ensure quality and progress are maintained in line with the project plan. The Board noted that as details of the Agency's relocation plans are being finalised, some further adjustments to the project planning may be required. The User Acceptance Testing (UAT) of release 0.7 is planned for November 2018 to allow for completion of the relocation of the development data centre, before UAT commences. The audit, which will start in early 2019, will need to accommodate the Agency's staff relocation to Amsterdam in early March 2019.

Planning adjustments for these events are underway. These are not currently expected to have a major impact on overall timing for the project, but will require careful management. Further impact of the relocation is being carefully monitored, and mitigation measures are being put in place where possible, in particular in the event of loss of key Agency or developer staff, unable to relocate. The Clinical Trial system continues to be prioritised in the context of the Agency's relocation business continuity planning. The Board also heard an interim report from the external review of the project and agreed refinements to the governance, reporting and user engagement to support the project through the vital phases of its development.