



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) Implementation Guidelines on Good Manufacturing Practice specific to Advanced Therapy Medicinal Products (Eudralex Vol 4 Part IV)

As this document has been adopted by the European Commission at 22nd November 2017, EU ATMP manufacturers should comply with these new Guidelines no later than **22nd May 2018**.

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

Procedural advice on the evaluation of advanced therapy medicinal product in accordance with Article 8 or Regulation (EC) No 1394/2007

At 25th January 2018, EMA updated its procedural advice on the evaluation of ATMPs. The aim of the update is to clarify the evaluation procedure, to help developers of these medicines navigate the regulatory process in the European Union. The updated guidance:

- reinforces timely and effective interactions between the applicants, EMA and its committees;
- details the roles and responsibilities of the three of EMA's scientific committees involved in the evaluation: the Committee on Advanced Therapies (CAT), the Committee for Medicinal Products for Human Use (CHMP) and the Pharmacovigilance Risk Assessment Committee (PRAC);
- streamlines the processes for adopting the list of questions and list of issues by the committees;
- clarifies in which situations oral explanations might be needed;



- gives developers more time to respond to questions raised by the Committees by allowing longer clock stops.

This guidance concentrates on the initial evaluation of new ATMPs, but its principles also apply to post-authorisation procedures.

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

A slide deck with more information on the changes introduced by the updated guidance can be found [here](#).

3) Public consultation for the Guideline on safety and efficacy follow-up and risk management of Advanced Therapy Medicinal Products (revision 1)

(until 30 APR 2018)

The aim of this guideline is to provide the guidance for the Safety and Efficacy (S&E) follow-up and risk management for advanced therapy medicinal products (ATMPs) according to Article 14(4) of Regulation (EC) No 1394/2007. This regulation requires the European Medicines Agency (EMA) to develop a detailed guideline relating to the post-authorisation follow-up of efficacy and adverse reactions, and risk management for these products.

This is the 1st revision of the original ATMP guideline on safety and efficacy follow-up and risk management ; the guideline has been revised to take into consideration the experience gained with the authorisation of these products and to define their risks and their risk minimisations measures. In addition, guidance on methodology in order to design post-authorisation S&E follow-up studies is provided.

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

4) 2nd International awareness session for international regulators, academia and non-governmental organisations

This two-day awareness session for international regulators, academia and non-governmental organisations (NGOs) at 8-9 March 2018 gave an insight into how the European medicines regulatory network works, the role of European Medicines Agency (EMA), scientific aspects of EMA's work and its interaction with scientific experts. Patrick Celis on behalf of the CAT gave a comprehensive overview over the ATMP regulation and results and examples of regulatory assessment of ATMPs to date.

The CAT presentation can be found [here](#).

Full information on the presentations can be found [here](#).

5) Other topics:

Marketing authorization approval for darvadstrocel (Alofisel)



TiGenix and Takeda announced that the European Commission (EC) has approved darvadstrocel (Alofisel), previously Cx601, for the treatment of complex perianal fistulas in adult patients with nonactive/mildly active luminal Crohn's disease, when fistulas have shown an inadequate response to at least one conventional or biologic therapy. This is the tenth ATMP and the first allogeneic stem cell therapy to receive central marketing authorization approval in Europe.

The full public assessment report of darvadstrocel (Alofisel) can be found [here](#).

Update on development of the EU Clinical Trial Portal and Database

The EMA reported that the first EU clinical trial portal development version (release 0.6) has been received and has met the acceptance criteria. Further experience will enable greater confidence in the plan to be gained and an external party will also be asked to review this and report to the EMA Board. The plan shows that release 0.7 should be available for audit, as required by Article 82 of the Clinical Trial Regulation, early in 2019. More precise information on timelines will be communicated after the audit.

Update Committee for Advanced Therapies (CAT) on achievements and challenges

Dr Martina Schüssler-Lenz, chair of the CAT, also deputy head of advanced therapy medicinal products at the Paul-Ehrlich Institute (PEI) in Germany, presented to the EMA Board the achievements and ongoing challenges in the area of ATMPs. Ten advanced therapies have been granted an EU-wide marketing authorisation since the creation of the CAT in 2009. Four advanced therapies are currently under evaluation, including one cell-based and three gene-based therapies. In 2018, the CAT expects to start evaluating four additional medicines. "We are observing rapidly evolving scientific and technical innovation entering the field of advanced therapies," explained Dr Schüssler-Lenz, "but the Committee is well set up to cope with the scientific and regulatory challenges ahead due to its expertise and the way members interact and learn from each other."

Dr Schüssler-Lenz also noted that requests for scientific advice for advanced therapies have increased significantly between 2012 and 2017 and that the CAT is now routinely involved in all scientific advice procedures for these medicines.