News from the EMA

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A common EU approach to data transparency in medicine regulation

December 18, 2024

EMA and HMA (Heads of Medicines Agencies) have published a comprehensive overhaul of their guidance on the identification of commercially confidential information (CCI) and personal data in marketing authorisation applications for human medicines.[1]

With the adoption of the initial guidance in 2012, European regulatory authorities agreed for the first time a common and consistent approach to identifying which parts of an application dossier can or cannot be released to the public, regardless of whether the medicine concerned has been authorised using the centralised, mutual-recognition or decentralised procedures.

As a general rule, the overwhelming majority of data in marketing authorisation applications is not considered CCI. The exceptions mainly relate to information about the manufacturing of a medicine, as well as information about facilities or equipment and some contractual arrangements between companies. While considered CCI at the time of the initial guidance, general information related to quality is now mostly considered releasable.

Instead of applying a "yes / no" rule as to whether an entire section of the dossier can be released, the updated guidance considers information as releasable by default. It provides detailed practical orientations as to which specific points could be redacted or anonymised within each section of the dossier. The annex of the guidance document has been updated and now includes examples of information that may be considered CCI or protected personal data.

The guidance also sets out how personal data will be protected if it can lead to the identification of a person. In doing so, it now considers the more recent EU legislation on data protection, namely the EU General Data Protection Regulation (GDPR) and Data Protection Regulation for the European Union institutions, bodies, offices and agencies (EUDPR). The document gives further guidance on how to identify personal data relating to experts, staff, or patients, which should be anonymised.

References

1. HMA/EMA guidance document on the identification of personal data and commercially confidential information within the structure of the marketing authorisation application (MAA) dossier. 12 December 2024. Available from: https://www.ema.europa.eu/en/documents/other/heads-medicines-agencies-european-medicines-agency-guidance-document-identification-commercially-confidential-information-personal-data-within-structure-marketing-authorisation-application_en.pdf

New EU rules for health technology assessments become effective

January 10, 2025

EMA is ready to support the implementation of the new health technology assessment regulation (HTAR) (Regulation (EU) 2021/2282) when it becomes applicable on January 12, 2025.[1]

The regulation is an important step forward in accelerating and widening access to new medicines. In the EU, a centrally authorised medicine is accessible to patients when it has first gone through regulatory assessment by EMA and is authorised for use in patients, and secondly has been evaluated by health technology assessment (HTA) bodies to help Member States make decisions about the use, price, and reimbursement level of a new health technology taking into account its impact on the sustainability of the healthcare systems.

The regulation also creates an EU framework for the assessment of selected high-risk medical devices to help national authorities to make more timely and informed decisions on the pricing and reimbursement of such health technologies.

EMA will support the implementation of the new piece of legislation in three areas. It will:

- Support timely conduct of **joint clinical assessments (JCA)** by the HTA Coordination Group establishing relative clinical effectiveness and relative clinical safety of a new health technology as compared with new or existing technologies. In this context, EMA will provide relevant information from its own regulatory assessments
- Collaborate with the HTA Coordination Group in **parallel joint scientific consultations (JSC)** to give scientific advice to technology developers and facilitate generation of evidence that satisfies the needs of both regulators and HTA bodies
- Exchange information on upcoming applications and future health technologies, both for planning purposes and for horizon scanning.

The regulation recognises the value of cooperation between decision-makers, namely regulators who evaluate the benefits and the risks of medicines and HTA bodies who then assess their effectiveness compared to existing products. It builds on the longstanding cooperation between EMA and HTA bodies, developed with the European Network for Health Technology Assessment (EUnetHTA) until September 2023.

The new rules will initially apply to new active substances to treat cancer and to all advanced therapy medicinal products (ATMPs). They will be expanded to orphan medicinal products in January 2028, and to all centrally authorised medicinal products as of 2030. Selected high-risk medical devices will also be assessed under the HTAR as of 2026.

EMA now has a legal obligation to notify the European Commission, which serves as the secretariat to the HTA Coordination Group (HTACG), ensuring that procedures are followed and joint work is produced in a timely and transparent manner when it receives submissions for marketing authorisation applications for medicinal products in the scope of JCA. From June 2024, the Agency started identifying such applications.

References

1. Regulation (EU) 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU. Available from: https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:32021R2282

Human medicines in 2024

January 16, 2025

In 2024, EMA recommended 114 medicines for marketing authorisation. Of these, 46 had a new active substance which had never been authorised in the EU before. Among these are a number of medicines that stand out due to their contribution to address public health needs or the innovation they represent. The Agency recommended the first medicine to treat early Alzheimer's disease, the first needle-free and smaller form of adrenaline to treat allergic reactions, the first treatment for tumours associated with von Hippel-Lindau disease, and two new antibiotic medicines for the treatment of certain severe infections.

EMA also recommended several new vaccines, including one to protect against Chikungunya disease and a new mRNA vaccine against lower respiratory tract disease caused by respiratory syncytial virus (RSV), and extended the use of an mpox vaccine to protect adolescents from 12 to 17 years of age.

As in previous years, cancer was the strongest therapeutic area, with 28 recommendations for oncology products. There were also 28 recommendations for new biosimilar products, covering a wide range of diseases, including several types of cancer, osteoporosis, macular degeneration, and diseases that involve an abnormal immune response like plaque psoriasis, ulcerative colitis, and Crohn's disease. This is good news for patients, as biosimilars make treatments more accessible and can provide broader access to potentially life-changing medicines.

The overview of the 2024 key recommendations published today includes figures on the authorisation of medicines and a selection of new treatments that represent significant progress in their therapeutic areas.

Once a medicine is authorised by the European Commission and prescribed to patients, EMA and the EU Member States continuously monitor its quality and benefit-risk balance and take regulatory action when needed. Measures can include a change to the product information, the suspension or withdrawal of a medicine, or a recall of a limited number of batches. An overview of some of the most notable safety-related recommendations is also included in the document referenced below.

References

1. Human medicines in 2024. Available from: https://www.ema.europa.eu/en/documents/report/human-medicines-2024_en.pdf

European Shortages Monitoring Platform fully operational for monitoring of shortages in the EU

January 29 , 2025

The European Shortages Monitoring Platform (ESMP) is now live with the full scope of functionalities. This will enable marketing authorisation holders (MAHs) and national competent authorities (NCAs) to directly report information on supply, demand, and availability of nationally and centrally authorised medicines during crises and preparedness actions led by EMA's Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG).

The new release facilitates monitoring and management of critical medicines during public health emergencies and major events and in the context of preparedness activities. It follows the release of the functionalities for routine shortage reporting of centrally authorised medicines for MAHs in November 2024.

The use of the ESMP has become mandatory for MAHs and NCAs as of 2 February 2025.

The ESMP is a key requirement of EMA's extended mandate, enhancing shortages monitoring and preparedness across the EU/EEA. It gives MAHs and NCAs a platform to report accurate, complete, and timely information on the supply and demand of medicines. Harmonised reporting standards in the ESMP will lead to enhanced usability of data, and this will speed up the EU/EEA's ability to put in place coordination actions to prevent and mitigate shortages.

Publicly available information on shortages of individual medicines is accessible via the ESMP in EMA's shortages catalogue and national shortages catalogues.[1] To ensure readiness to use the ESMP, EMA invites all MAHs and NCAs to attend the webinars offered and to make use of the information material available on EMA's website.[2]

References

1. Public information on medicine shortages. Available from: https://www.ema.europa.eu/en/human-regulatory-overview/post-authorisation/medicine-shortages-availability-issues/public-information-medicine-shortages
2. European Shortages Monitoring Platform (ESMP), Guidance and training materials. Available from: https://www.ema.europa.eu/node/241136#guidance-and-training-materials-69020

Veterinary medicines in 2024

January 23, 2025

EMA has published an overview of its key recommendations of 2024 regarding the authorisation and safety monitoring of veterinary medicines.

In 2024, EMA recommended 25 veterinary medicines for marketing authorisation – the highest ever number of recommendations in a year. Of these, two had a new active substance which had not previously been authorised in a veterinary medicine in the EU; 14 were vaccines, including seven that had been developed by means of a biotechnological process. Among the medicines recommended for marketing authorisation in 2024, 13 were for food-producing animals, such as chickens, pigs, and cattle, and 11 were for companion animals, such as dogs and cats.

A selection of these recommendations can be found in the veterinary medicines highlights document published today.[1]

References

1. Veterinary medicines in 2024. Available from: https://www.ema.europa.eu/en/documents/report/veterinary-medicines-2024_en.pdf

Photo: Freepik

Clinical Trials Regulation becomes fully applicable

January 31, 2025

From today, all clinical trials in the European Union (EU), including ongoing trials that were approved under the previous legal framework, the Clinical Trials Directive (CTD), are governed by the Clinical Trials Regulation (CTR). This marks the end of a three-year transition period, during which more than 5,000 clinical trials were transitioned to the CTR through submission to the Clinical Trials Information System (CTIS), the single-entry point for sponsors and regulators for the submission and assessment of applications for clinical trials in the EU.

Remaining trials that are ongoing after January 30 and that were not moved to the new system may be subject to corrective measures applied by EU Member States. Transition procedures are no longer available and sponsors of ongoing CTD trials are required to submit a new application via CTIS.

CTIS includes a public searchable database for healthcare professionals, patients, and the general public to deliver the high level of transparency foreseen by the regulation. The authorisation and oversight of clinical trials is the responsibility of EU/EEA Member States while EMA is responsible for maintaining the CTIS. The European Commission oversees the implementation of the Clinical Trials Regulation. Throughout 2025, the performance and the user experience of CTIS will continue to be improved.

The full implementation of the CTR strengthens Europe as an attractive location for clinical research. The regulation streamlines the processes for the application and supervision of clinical trials, and their public registration: all clinical trial sponsors use the same system and follow the same procedures to apply for the authorisation of a clinical trial, no matter where they are located and which national competent authority (NCA) or national ethics committee they are dealing with.

Activities related to the CTR are supported by the Accelerating Clinical Trials in the EU (ACT EU) initiative,[1] a collaboration between the Heads of Medicines Agencies (HMA) in the Member States, the European Commission and EMA, which seeks to transform how clinical trials are initiated, designed and run. ACT EU features focus areas that are the basis for the ACT EU multi-annual workplan 2025-2026.[2]

References

1. Accelerating Clinical Trials in the EU (ACT EU). Available from: https://www.ema.europa.eu/en/human-regulatory-overview/research-development/clinical-trials-human-medicines/accelerating-clinical-trials-eu-act-eu
2. ACT EU multi-annual workplan 2025-2026. Version 3, December 2024. Available from: https://accelerating-clinical-trials.europa.eu/document/download/0f53c5bd-292f-4407-b891-6259f2d187aa_en?filename=ACT%20EU_workplan%202025-2026.pdf

New Chikungunya vaccine for adolescents from 12 and adults

January 31, 2025

EMA has recommended granting a marketing authorisation in the European Union (EU) for Vimkunya (applicant, Bavarian Nordic A/S), the first vaccine in the EU to protect adolescents from the age of 12 against Chikungunya. This vaccine, also intended for adults, is given as a single dose.

Chikungunya, also called CHIK fever, is a viral disease caused by Chikungunya virus (CHIKV), a virus transmitted to humans by infected mosquitoes (primarily *Aedes aegypti* and *Aedes albopictus*). Most people infected with CHIKV develop symptoms within 3–7 days. The most common symptoms of acute disease are fever and joint pain. Most patients recover within a week, but some develop joint pain for several months or longer, which can be disabling, and a small proportion of patients may develop severe acute disease, which can lead to multiorgan failure.

CHIKV infections affect people mostly in the tropics and subtropics. Chikungunya is not endemic in Europe. The majority of cases in the EU concern travellers who were infected outside of mainland Europe. Spread of the *Aedes albopictus* mosquito due to climate change could lead to cases of Chikungunya in regions so far spared.

Vimkunya was supported through EMA's PRIority MEdicines (PRIME) scheme, which provides early and enhanced scientific and regulatory support to medicines that have a particular potential to address patients' unmet medical needs.

The CHMP's opinion is largely based on data from two placebo-controlled studies. Study 1 assessed the immunogenicity and safety of the vaccine in 3,258 individuals from 12 to 64 years of age, and Study 2 in 413 older adults. The immune response was evaluated in 3,355 participants (2,748 with Vimkunya and 607 with placebo). The clinical efficacy of Vimkunya was inferred from a post-vaccination CHIKV-specific neutralising antibody titre threshold selected as a surrogate marker and referred to as seroresponse. Eight days after vaccination, the difference in seroresponse rates (SRRs) between those vaccinated with Vimkunya and those with placebo in Study 1 was 46.1%. This rose to 96% at Day 15, 96.6% at Day 22 and 84% at Day 183. In Study 2, the difference in SRRs was 79.5% at Day 15, 86.2% at Day 22 and 74.4% at Day 183.

The safety profile of Vimkunya is based on pooled data from five completed clinical studies with 3,522 participants with a 6-month follow-up. The most common side effects reported were tiredness, headache, muscle pain and injection site pain.

The CHMP has requested a post-authorisation efficacy study to confirm the effectiveness of Vimkunya in preventing Chikungunya in adolescents and adults.

EMA establishes regular procedure for scientific advice on certain high-risk medical devices

February 10, 2025

EMA, in close collaboration with the European Commission, has established a standard procedure for manufacturers of certain high-risk medical devices to request scientific advice on their intended clinical development strategy and proposals for clinical investigation.

Manufacturers of class III devices and class IIb active devices intended to administer or remove medicines can now submit their request for advice via a portal and consult the medical device expert panels at different stages of the clinical development. Advice given by the medical device expert panels is a key tool to foster innovation and promote faster patient access to safer and more effective devices.

This regular scientific advice procedure follows a pilot launched in February 2023, which has helped to establish this procedure and gathered positive feedback from manufacturers and panel experts. EMA will publish a report on the pilot in the coming weeks.

There are currently no fees associated with these requests. More information on the submission process, including step-by-step instructions for applicants and monthly submission deadlines is available on EMA's website. Manufacturers of high-risk medical devices intended for the treatment of a rare condition should apply for advice via the ongoing pilot programme to support orphan medical devices. EMA provides the secretariat to support the expert panels, based on Regulation (EU) 2022/123