News from the EMA

The articles included in this section are a selection from the European Medicines Agency (EMA)'s News and Press Releases archive.
More information can be found on the Agency's website: www.ema.europa.eu.

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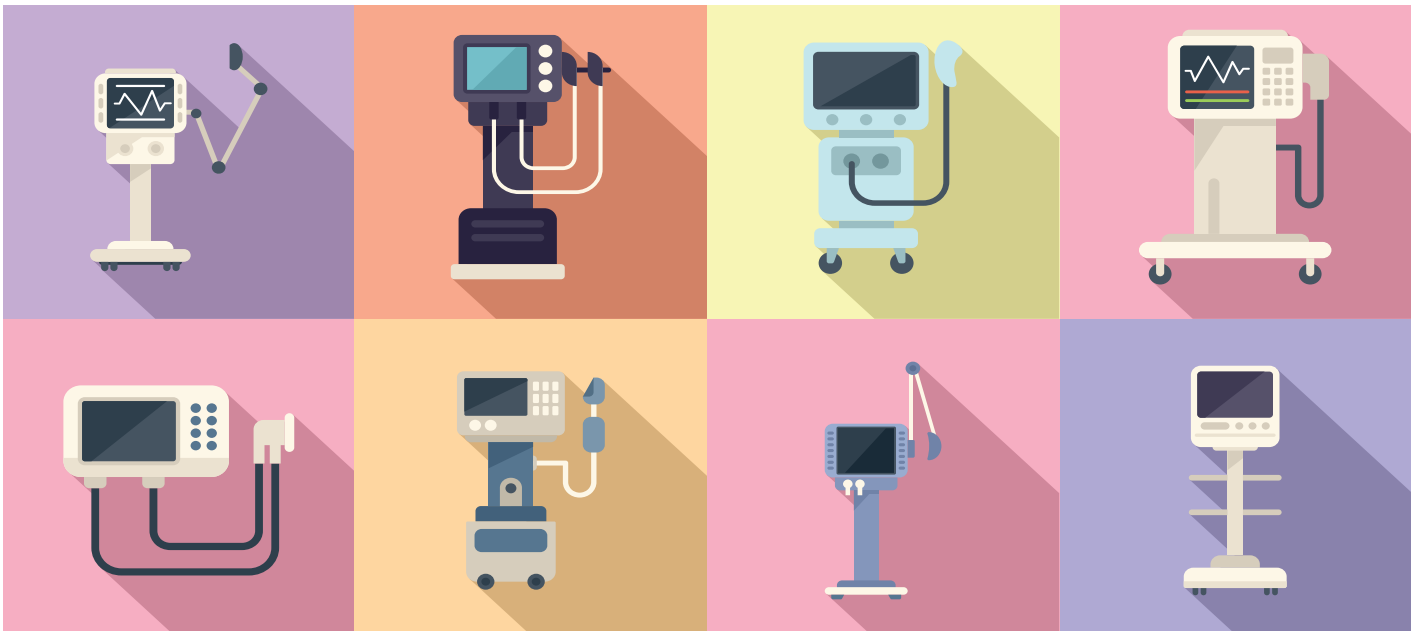


Photos: Freepik

New pilot programme to support orphan medical devices

August 2, 2024

EMA has launched a pilot programme for expert panels to support the development and assessment of orphan medical devices in the European Union (EU). The pilot programme offers free advice from the medical device expert panels to selected manufacturers and notified bodies on the orphan device status and the data needed for their clinical evaluation. While the pilot programme is currently scheduled to run until the end of 2025, the aim is to establish a long-term process for orphan device support.

Orphan devices are medical devices which are intended to be used for diseases or conditions affecting only a small number of individuals each year (not more than 12,000 individuals in the EU per year). Often, they are used to treat or diagnose rare diseases or conditions for which no or insufficient alternative diagnostic or therapeutic options exist, thereby fulfilling an unmet medical need.

Manufacturers can consult the expert panels at different stages of the development of the clinical strategy for their device, while notified bodies can request advice at specific moments of the ongoing conformity assessment of the device. As part of the pilot programme, EMA will prioritise certain types of orphan medical devices, such as devices for treating a medical condition that is life-threatening or that could cause permanent impairment of a body function, devices intended for children, and novel devices with potential major clinical benefit.

In June 2024, the European Commission announced new guidance on the clinical evaluation of orphan medical devices issued by the Medical Device Coordination Group, which is composed of representatives of all EU Member States.[1] This guidance provides the criteria to determine when a medical device should be regarded as an orphan device under the EU Medical Devices Regulation and aims to guide manufacturers and notified bodies when applying the clinical evidence requirements.

This pilot programme is part of EMA's regulatory support for the expert panels on medical devices, following the introduction of new legislation in the EU. Since March 1, 2022, the Agency supports the medical device expert panels that provide opinions and views to notified bodies on the scientific assessment of clinical and performance evaluations of certain high-risk medical devices and in vitro diagnostic medical devices.

Early advice to manufacturers, particularly to small and medium-sized enterprises, is a key tool to foster innovation and accessibility to safer and effective devices that address patients' needs. The orphan device pilot will run in parallel to the scientific advice pilot to manufacturers which already prioritised advice to manufacturers on the clinical development strategy and clinical investigations of devices addressing unmet needs.

Reference

1. Medical Device Coordination Group Document, MDCG 2024-10. June 2024. Available from: MDCG 2024-10 Clinical evaluation of orphan medical devices

Photos: Freepik

ICH guideline E11A on pediatric extrapolation – Scientific guideline

August 27, 2024

The ICH E11A guideline provides recommendations for harmonised approaches for paediatric extrapolation to support the development and authorisation of paediatric medicines. It provides a framework for using extrapolation as a tool to support paediatric drug development that encompasses an iterative process for understanding the existing information available, the gaps in information needed to inform development, and ways to generate additional information when needed.

Pediatric extrapolation is defined in the ICH E11(R1) guideline as "an approach to providing evidence in support of effective and safe use of drugs in the pediatric population when it can be assumed that the course of the disease and the expected response to a medicinal product would be sufficiently similar in the pediatric [target] and reference (adult or other pediatric) population." The reference population can include other pediatric age subsets. Pediatric extrapolation can extend what is known about the reference population (e.g., pharmacokinetics (PK)/dosing, efficacy, and safety) to the target population based on an assessment of the relevant similarities of disease, drug pharmacology, and response to treatment between the two populations.

The ICH E11A on pediatric extrapolation guideline is to be read in conjunction with the ICH E11 guideline on clinical investigation of medicinal products in the paediatric population and the ICH E11(R1) addendum, both available on page ICH E11(R1) step 5 guideline on clinical investigation of medicinal products in the pediatric population – Scientific guideline. Furthermore, applicants wishing to follow an extrapolation approach are suggested to look also at EMA's Structured guidance on the use of extrapolation.

Harnessing AI in medicines regulation: use of large language models (LLMs)

September 5, 2024

EMA and the Heads of Medicines Agencies (HMA) have published high-level principles and recommendations for all staff across the European medicines regulatory network (EMRN) using large language models (LLMs) in their work.[1]

LLMs are a category of generative AI, whose applications can significantly support medicine regulators in their tasks and processes. Whether they are used to query the extensive documentation regulators routinely receive, to automate knowledge/data mining processes, or as virtual AI assistants in everyday administrative tasks – LLMs have enormous transformative potential.

However, LLMs also present challenges, e.g. variability in results, returning of irrelevant or inaccurate responses (so-called hallucinations), and potential data security risks. The purpose of the guiding principles is to build understanding of the capabilities and limitations of these applications among staff at regulatory agencies across the EU so that they can harness the potential of LLMs effectively and avoid pitfalls and risks.

The guiding principles cover various aspects of using LLMs, from ensuring safe input of data, to applying critical thinking and cross-checking outputs, to knowing whom to consult when concerns arise. Responsible use of LLMs requires familiarity with the tools. The importance of continuous learning is emphasised to keep pace with the fast-changing field.

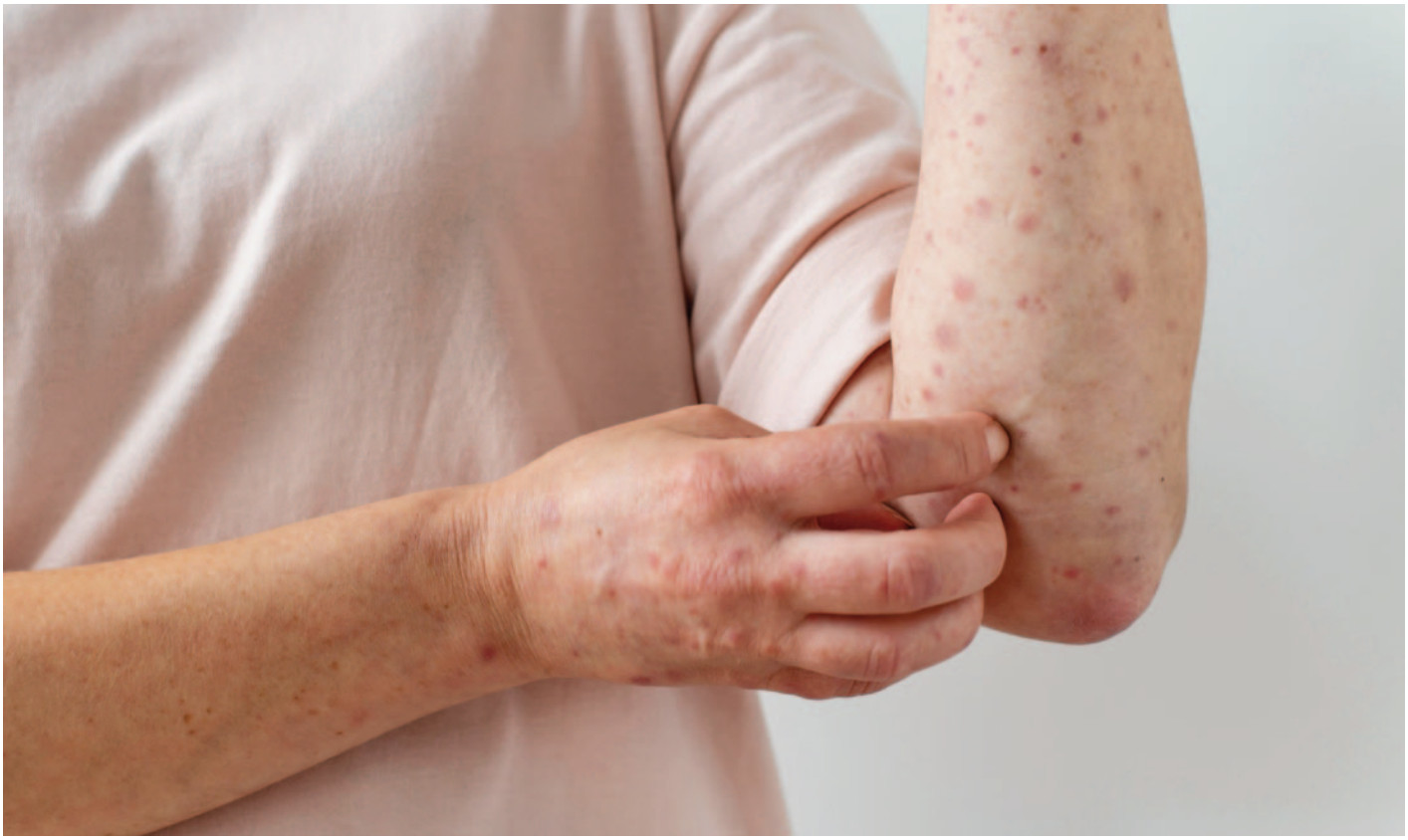
Additionally, the principles encourage regulatory agencies to make efforts to support their staff in using LLMs. This includes defining governance on the use of LLMs, specifying permitted use cases, providing training and monitoring risks.

The guiding principles are one of the deliverables of the multiannual AI workplan to 2028 by EMA and the Heads of Medicines Agencies (HMA). This workplan guides EMA and the EMRN in their use of AI, maximising the benefits while managing the risks, and facilitating information sharing.

The guiding principles are a living document that will be regularly updated.

Reference

1. Guiding principles on the use of large language models in regulatory science and for medicines regulatory activities. August 29, 2024. Available from: https://www.ema.europa.eu/en/documents/other/guiding-principles-use-large-language-models-regulatory-science-medicines-regulatory-activities_en.pdf



EMA recommends extending indication of mpox vaccine to adolescents

September 19, 2024

EMA has recommended extending the indication of the smallpox and mpox vaccine Imvanex to adolescents from 12 to 17 years of age.

Imvanex is already authorised in the EU to protect against smallpox, mpox, and the disease caused by the vaccinia virus in adults. It contains a live, highly weakened form of a virus called "modified vaccinia virus Ankara" (MVA-BN), which is related to the smallpox virus. EMA's human medicines committee (CHMP) based the recommendation to extend the use of Imvanex to adolescents on the interim results of a study that compared the vaccine's ability to generate an immune response (produce virus-specific antibodies) in 315 adolescents and in 211 adults.

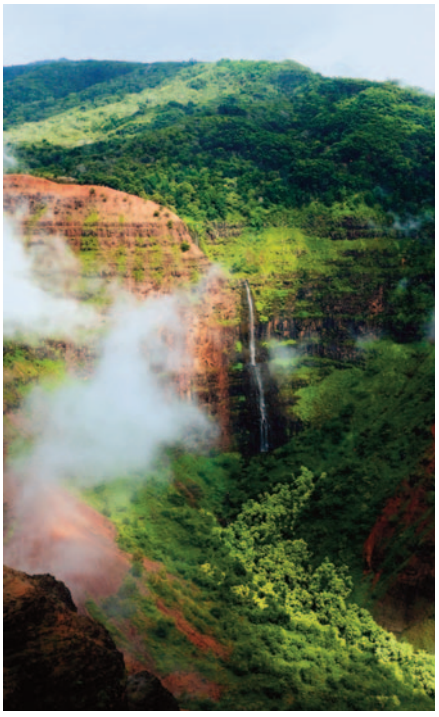
The immune response in adolescents was similar to adults. Therefore, it is inferred that the vaccine will provide similar protection in adolescents to that expected in adults. According to the submitted data, the safety profile of Imvanex in adolescents was comparable to that seen in adults and no additional risk has been identified. As part of its recommendation, EMA has requested the marketing authorisation holder to submit the final results of the study by May 30, 2025, to further characterise the information about safety in adolescents.

The Agency's assessment has important implications for the global response to the mpox outbreak in the Democratic Republic of the Congo (DRC) and other countries, which was declared a public health emergency of international concern (PHEIC) by the World Health Organization (WHO) on August 14, 2024. EMA is the regulatory agency of record for prequalification of this vaccine by WHO on September 13, 2024.

Mpox is a disease that is transmitted to people by animals, mainly rodents, but can also spread between people with direct contact. It is endemic in certain parts of Central and West Africa. The current surge in cases in the DRC and several neighbouring countries is driven by the mpox clade I strain that is known to cause a more severe form of mpox in humans than the mpox clade II strain that spread during the 2022/2023 PHEIC. Mpox can be fatal for people with weak immune systems.

Data indicate that Imvanex protects against both the clade I and clade II mpox strains.

In the EU, decisions on how vaccinations should be given are the prerogative of the expert bodies guiding vaccination campaigns in each Member State. The European Centre for Disease Prevention and Control (ECDC) published advice for public health authorities for mpox on their website.



Photos: Freepik

Don't miss!

The June 2025 edition

Communicating with the Public

When we communicate effectively with patients and the public, we empower them to make informed decisions about their health. This issue will cover the latest guidelines and standards to be considered when writing and designing information for patients and the public. 73It will also feature articles from thought leaders on plain language writing, inclusive communication, and patient involvement in research. With this issue, we hope to provide insights that will strengthen the role of medical writers as advocates for the patient voice, and as powerful and effective communicators of understandable science.

Guest Editors: Sampoorna Rappaz and Lisa Chamberlain James
The deadline for feature articles is March 1, 2025.

Updated advice to minimise risks of interaction between weight loss medicine Mysimba and opioids

November 15, 2024

After re-examining its initial opinion, EMA recommends updating the advice aimed at minimising the risks of interaction between the weight loss medicine Mysimba (naltrexone/ bupropion) and opioid-containing medicines (including painkillers such as morphine and codeine, other opioids used during surgery and certain medicines for cough, cold, or diarrhoea).

Opioid medicines may not work effectively in patients taking Mysimba, because one of the active substances in Mysimba, naltrexone, blocks the effects of opioids. There is also a risk of rare but serious and potentially life-threatening reactions, such as seizures and serotonin syndrome (a potentially life-threatening condition that results from having too much serotonin in the body), in people taking Mysimba together with medicines for treating depression and opioids.

To minimise these risks, patients and healthcare professionals are reminded that Mysimba must not be used in people who are dependent on opioids, people receiving treatment with opioid agonists such as methadone or buprenorphine, and people going through acute opioid withdrawal.

Mysimba is a medicine used along with diet and exercise to help manage weight in adults who have obesity (have a body-mass index – BMI – of 30 or more) or who are overweight (have a BMI between 27 and 30) and have weight-related complications such as diabetes, abnormally high levels of fat in the blood, or high blood pressure. Mysimba was granted marketing authorisation on March 26, 2015.

People using Mysimba will be given a patient card to be carried with them at all times. The card will remind them to inform their doctor, in case of surgery, that they are using Mysimba. This is because Mysimba should be stopped for a minimum of three days before starting treatment with opioids, which are often used to prevent pain and discomfort during surgery and medical procedures.

The product information for Mysimba is being updated to reflect these changes.

Photo: Freepik

Medical Writing in
SPANISH

Medical writing

Despite our diverse backgrounds and countries of origin – Beatriz is a Spanish molecular biologist, Yanina is an Argentinean biochemist, and Valentina is an Italian physicist – our professional journeys share some common points. We all started as scientists, spent several years in research, and then transitioned to the linguistic aspects of science: medical writing, translation, and proofreading. We also share the subjective side of our career change: after many years of reading and writing highly specialised English texts, we assumed that conveying the same content in Spanish would be straightforward. Instead, we discovered that this endeavour was far from easy: more often than not, we found ourselves at a loss for specialised Spanish words. We quickly understood that those years in research were both a gift and a curse, revealing a common struggle for medical writers who need to write in a language other than English.

One obvious challenge for medical writers and translators writing in Spanish is geographical diversity: Spanish is spoken in 21 countries[1] on three continents, each using a specific language variant with different words, phonetics, and even syntax. Many of these variants are strongly influenced by close contact with neighbouring Anglo-Saxon countries, which encourages the introduction of "false friends", such as constipado (which means "common cold") instead of estreæimiento for "constipation", or injuria (which means "offence") instead of lesión for "injury". The Anglo-Saxon influence is also a consequence of the fact that English is the lingua franca in the dissemination of science: although English is only the third most spoken language[2] in the world, with 330 million native speakers, after Mandarin Chinese (929 million) and Spanish (475 million), it accounts for 94% of articles in the scientific research database Web of Science, compared to only 1.3% for Spanish (2022 data).[3]

Thus, writing scientific content in Spanish almost