# News from the EMA

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#### SECTION EDITOR





## Global regulators call for international collaboration to integrate real-world evidence into regulatory decision-making

#### July 22, 2022

MA has endorsed a joint statement calling for international collaboration to enable the generation and use of real-world evidence for regulatory decision-making published today by the International Coalition of Medicines Regulatory Authorities (ICMRA).

The use of real-world data and real-world evidence in the development, authorisation and monitoring of medicines to support regulatory decision-making is rapidly increasing. Although real-world evidence can play an important role in bridging knowledge gaps, there are still challenges that need to be addressed, such as heterogeneous data sources across the globe and different levels of quality of the data. Interested parties also need to deal with various processes for data sharing and access.

During the COVID-19 pandemic, international medicines regulators and researchers have worked together to establish or reinforce collaboration allowing efficient sharing of data and experience in relation to real-world evidence. They agreed to further such collaboration beyond the pandemic.

In their statement, ICMRA members pledge to foster global efforts and further enable the integration of real-world evidence into regulatory decision-making. They identify four focus areas for regulatory cooperation:

- harmonisation of terminologies for real-world data and real-world evidence;
- regulatory convergence on real-world data and real-world evidence guidance and best practice;
- readiness to address public health challenges and emerging health threats; and transparency.

Global regulators emphasise their commitment to steer the work in these areas which could be taken forward through a variety of existing fora, including the International Conference on Harmonization (ICH), international standardisation bodies, and clusters of interested regulators.

The joint statement was developed following an ICMRA workshop on real-world evidence coorganised by EMA, US FDA and Health Canada, held in Amsterdam in June 2022. Participants from more than 40 countries, representing medicines regulatory authorities globally as well as representatives from the World Health Organization (WHO), shared their accomplishments and challenges in generating real-world evidence to support the evaluation of medicines. As a next step, international medicines regulators will discuss concrete actions to implement the abovementioned four areas of collaboration.

#### New medicine for multiple myeloma patients with limited treatment options

July 22, 2022

MA has recommended a conditional marketing authorisation in the European Union (EU) for Tecvayli (teclistamab) for the treatment of adult patients with relapsed and refractory multiple myeloma, who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody, and whose cancer has worsened since receiving the last treatment.

Multiple myeloma is a rare cancer of a type of white blood cells called plasma cells. Normal plasma cells are found in the bone marrow and are an important part of the immune system. Plasma cells make the antibodies that enable the body to recognise and attack germs, such as viruses or bacteria. In multiple myeloma, the division of plasma cells becomes uncontrolled, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. When plasma cells become cancerous, they no longer protect the body from infections and produce abnormal proteins that can cause problems affecting the kidneys, bones, or blood.

A range of new medicines for the treatment of multiple myeloma have been developed and approved in recent years, leading to a steady overall improvement in patient survival. However, for patients who have already been treated with three major classes of drugs (immunomodulatory agents, proteasome inhibitors and monoclonal antibodies) and no longer respond to these drugs, the outlook is still bleak. Therefore, new medicines are needed for these patients.

Tecvayli is a monoclonal antibody that targets two proteins at the same time: a protein called

B-cell maturation antigen (BCMA), which is present on the surface of the multiple myeloma cells, and CD3, a protein that is present on T cells (cells of the immune system responsible for destroying abnormal cells). By attaching to BCMA and CD3 at the same time, the medicine activates the T cells to kill the multiple myeloma cells.

Tecvayli 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. EMA's human medicines committee (CHMP) reviewed the application for marketing authorisation under an accelerated timetable to enable faster patient access to this medicine.

The CHMP based its recommendation for a conditional marketing authorisation on a phase 1/2, multicentre, open label, single-arm clinical trial. The study investigated the efficacy and safety of Tecvayli in 165 patients with relapsed or refractory multiple myeloma who had received at least three prior therapies (including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 antibody) and who didn't respond to the last treatment regimen. 63% of patients enrolled in the study responded to the treatment with Tecvayli and lived without their disease getting worse for about 18 months on average. The most common side effects reported in the clinical trial for Tecvayli were hypogammaglobulinaemia (a condition in which the level of immunoglobulins (antibodies) in the blood is low and the risk of infection is high), cytokine

release syndrome (CRS) (i.e. a condition causing fever, vomiting, shortness of breath, headache and low blood pressure), and neutropenia (low levels of neutrophils, a type of white blood cell).

Tecvayli is recommended for a conditional marketing authorisation, one of the EU regulatory mechanisms to facilitate early access to medicines that fulfil an unmet medical need. This type of approval allows the Agency to recommend a medicine for marketing authorisation with less complete data than normally expected, if the benefit of a medicine's immediate availability to patients outweighs the risk inherent in the fact that not all the data are yet available.

In order to better characterise the safety and effectiveness of the medicine, the company will have to submit data from a randomised phase 3 confirmatory study comparing the efficacy of teclistamab in combination with daratumumab SC with the treatment regimen daratumumab SC, pomalidomide, and dexamethasone (DPd) or daratumumab SC, bortezomib, and dexamethasone (DVd) in adults with relapsed or refractory multiple myeloma. The company is also required to submit the final results of the pivotal study.

The opinion adopted by the CHMP is an intermediary step on Tecvayli's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role or use of this medicine in the context of the national health system of that country.





### Big data use for public health: Publication of Big Data Steering Group workplan 2022-25

#### July 28, 2022

he Big Data Steering Group set up by EMA and the Heads of Medicines Agencies (HMA) has published its PDF icon third workplan that sets key actions to be delivered between 2022–25.

The new workplan will allow to further enhance the efficient integration of data analysis into the evaluation of medicinal products by regulators. Using novel technologies and the evidence generated from big data will benefit public health by accelerating medicine development, improving treatment outcomes, and facilitating earlier patient access to new treatments.

The former Big Data Task Force carried out a thorough assessment of the challenges and opportunities posed by big data in medicines regulation, which culminated in 2020 in the publication of priority recommendations for regulators on the best approaches to use and generate data. The joint HMA-EMA Big Data Workplan 2022–2025 follows the key recommendations and includes mainly activities related to medicines for human use. However, the scope of some activities covers veterinary aspects, and a separate section in the workplan is fully dedicated to veterinary medicines.

The workplan lays out deliverables and timelines including for the following areas:

- The Data Analysis and Real World Interrogation Network (DARWIN EU), EMA's network of data and services in Europe for a better use of real-world evidence when assessing medicines: the workplan foresees more than one hundred DARWIN EU studies per year by 2025.
- Data quality: a data quality framework for the EU regulatory network is to be delivered by the end of 2022, following the analysis and exchanges on data quality with a wide range of stakeholders including patients, healthcare professionals, regulators, pharmaceutical industry and academia.
- Data discoverability: the workplan foresees the publication of a good practice guide on real-world metadata and a public catalogue of European real-world data. In addition, search-

ing for information from regulatory documents will be enhanced through the development of analytics tools and the development of standardised clinical trial protocols.

• EU network skills: the workplan includes the delivery of training on biostatistics, pharmacoepidemiology and data science for regulators with targeted access for patients, healthcare professionals and academics.

Big data are extremely large, rapidly accumulating datasets captured across multiple settings and devices, for example through wearable devices and electronic health records. Coupled with rapidly developing technology, big data can complement the evidence from clinical trials by filling knowledge gaps on a medicine, and can help to better characterise diseases, treatments, and the performance of medicines in individual healthcare systems.

The work carried out by the Big Data Steering Group builds on the Regulatory Science Strategy to 2025, published in March 2020, and will support the European Medicines Agencies Network Strategy to 2025.

#### New medicine to protect babies and infants from respiratory syncytial virus (RSV) infection

#### September 16, 2022

MA has recommended a marketing authorisation in the EU for Beyfortus (nirsevimab; from AstraZeneca AB) for the prevention of Respiratory Syncytial Virus (RSV) lower respiratory tract disease in newborn babies and infants during their first RSV season (when there is a risk of RSV infection in the community).

RSV is a common respiratory virus that usually causes mild, cold-like symptoms. Most people recover within one to two weeks, but RSV can be serious, especially in infants. It is the most common cause of lower respiratory tract infections, such as bronchiolitis (inflammation of the small airways in the lungs) and pneumonia (infection of the lungs) that may lead to hospitalisation or even death in newborn babies and young infants. For instance, in 2015, RSV caused an estimated 33 million lower respiratory tract infections in children younger than five years globally; 3.2 million of them required hospitalisation. Approximately 59,600 children died, the vast majority (43,600) in low- and middle-income countries. Despite a decrease in the number of RSV infections during the pandemic in 2020 and 2021, a resurgence in infections is expected following the easing of COVID-19 mitigation measures. In the EU, the virus is usually more common during the winter. Nirsevimab, the active substance in Beyfortus, is an antiviral monoclonal antibody (a type of protein), which has been designed to attach to the F (fusion) protein that RSV needs to infect the body. When nirsevimab is attached to this protein, the virus becomes unable to enter the body's cells. This helps to prevent RSV infection. Because the medicine is removed slowly from the body, over a period of several months, a single dose of Beyfortus protects infants against RSV disease during the entire RSV season. Beyfortus should be given before the RSV season (when there is a risk of RSV infection in the community) or as soon as possible after birth for infants born during the RSV season. In the northern hemisphere, this is from December to March.

Beyfortus was accepted into EMA's PRIME scheme on January 31, 2019 scheme. This scheme provides early and enhanced scientific and regulatory support to promising new medicines that address unmet medical needs. Beyfortus was also evaluated under EMA's accelerated assessment mechanism because prevention of RSV infection in all infants is considered to be of major public health interest.

The opinion by EMA's CHMP is based on data from two randomised, double-blind, placebo-controlled multicentre clinical trials that investigated the efficacy and safety of nirsevimab in healthy preterm (premature) and full-term infants entering their first RSV season. These studies demonstrated that Beyfortus prevents lower respiratory tract infection caused by RSV requiring medical attention (such as bronchiolitis and pneumonia) in term and preterm infants during their first RSV season.

The safety of nirsevimab was also evaluated in a phase II/III, randomised, double-blind, multicentre trial in infants who were born five or more weeks prematurely (less than 35 weeks gestation) at higher risk for severe RSV disease and infants with chronic lung disease of prematurity (i.e. long-term respiratory problems faced by babies born prematurely) or congenital heart disease. The results of this study showed that Beyfortus had a similar safety profile compared to Synagis (palivizumab). The most common side effects reported for Beyfortus were rash, pyrexia (fever) and injection site reactions (such as redness, swelling, and pain where the injection is given).

The opinion adopted by the CHMP is an intermediary step on Beyfortus' path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.



### EMA pilot offers enhanced support to academic and non-profit developers of advanced therapy medicinal products

#### September 29, 2022

MA is launching a pilot to support the translation of basic research developments into medicines that could make a difference in patients' lives in the European Economic Area (EEA). The pilot is open to academic sponsors and non-profit organisations who are developing advanced therapy medicinal products (ATMPs). These medicines for human use are based on genes, tissues, or cells and might offer groundbreaking treatment options to patients.

The pilot will focus on the needs of non-profit academic developers. They are a major contributor to the development of ATMPs and diagnostic and delivery devices, but experience has shown that navigating regulatory requirements can be challenging.

During the pilot, EMA will provide enhanced regulatory support for up to five selected ATMPs

that address unmet clinical needs and are solely developed by academic and non-profit developers in Europe. EMA will guide the participants through the regulatory process with the aim to optimise the development of the ATMPs, starting from best practice principles for manufacturing to planning clinical development that meets regulatory standards.

The pilot's first participant has already been selected. This ATMP is ARI-0001, a chimeric antigen receptor (CAR) product based on patients' own T-cells, that is developed by the Hospital Clínic Barcelona. In December 2021, the product was granted eligibility to PRIME, EMA's scheme to support the development of medicines that target an unmet medical need.

Importantly, no new regulatory tool will be introduced as part of this pilot. However, the aim

is to assess what further support or regulatory tool may be provided to enhance the number of ATMPs reaching patients in the EEA. In the process, EMA is keen to learn how to better interact with and support academic developers.

The pilot participants will benefit from all the available regulatory flexibilities and development support measures, such as fee reductions and waivers. The progress will be closely monitored, and initial results of the pilot are expected to be available in 3-4 years. Upon completion, a report will be published and a workshop with relevant stakeholders may be organised to discuss the learnings.

Potential developer candidates can contact their national competent authority or EMA via advancedtherapies@ema.europa.eu to express their interest in participating in the pilot or to receive more information.

