14 December 2023
EMA/573939/2023
Human Medicines Division

Committee for Medicinal Products for Human Use (CHMP): Work Plan 2024

Adopted by the Committee on 14 December 2023

Table of Contents

The activities outlined in the CHMP work plan for 2024 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2023-2025

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

1. Evaluation activities for human medicines

1.1. Pre-authorisation activities

1.1.1. Scientific consultations involving other decision makers to facilitate optimisation of clinical evidence generation in drug development programmes

Clinical evidence generated during drug development is intended to serve different decision making. It is therefore desirable that evidence requirements do address regulatory needs as well as those of other down-stream decision makers.

Key objectives

- To engage with other decision makers in multi-stakeholder consultations on evidence generation planning.

Activities in 2024

CHMP activities to achieve the objectives set for this area:

- Collaborate with HTA bodies on prospective evidence planning for development programmes through provisions of parallel EMA/HTA scientific advice until the new HTA Regulation is in operation.
- Explore with healthcare payers opportunities for sharing views on prospective evidence planning, focusing on post-licensing evidence needs.

CHMP topic leader: Bruno Sepodes

Other contributors:

Member/alternate	Name	MS
Co-opted member	Carla Torre	PT
SAWP Chair	Paolo Foggi	IT
Member	Helena Panayiotopoulou	CY

1.1.2. Contributing to Accelerating Clinical Trials in the EU (ACT EU)

In accordance with the CHMP mandate, CHMP provides advice for undertakings on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of medicinal products particularly regarding the development of new therapies.

Clinical Trial applications are in the remit of the member state where the application has been submitted.

Given the complex nature of medicines development, advice on closely related aspects of clinical trials being given by different regulatory actors, and the importance of building a consensus regulatory view in an efficient way, the objective is to develop a consolidated scientific advice process.

Key objectives

- Optimise European scientific advice through provision of consolidated scientific advice, bringing advice for clinical trial authorisation and for evidence to support marketing authorisation together, respecting roles and remits.

- As a first focus for consolidated advices and strengthening of scientific advice, to deliver the best evidence for decision making, in particular, for unmet medical needs, rare diseases, and on vaccines and therapeutics for public health crises and pandemics, with an emphasis on multinational clinical trials, where appropriate.

Activities in 2024

- As part of ACT EU priority action 7 on scientific advice, to support via the SAWP and the ETF, and collaborating with EMRN, the development of a consolidated advice process across relevant clinical trial regulatory stakeholders, respecting roles and remits.
- Provide input to the development of a methodology guidance roadmap in collaboration with ACT EU priority action 8 and the Methodology Working Party.

CHMP topic leader: Ewa Balkowiec Iskra

Other contributors:

Member/alternate	Name	MS
Co-opted member	Bruno Delafont	FR

1.2. Initial-evaluation activities

1.2.1. Benefit/Risk methodology and communication

Activity areas

Benefits and risks require continuous evaluation throughout the lifecycle of a medicine. The objective is to balance benefits and risks in a way that is as robust, consistent and transparent as possible.

Key objectives

- Continued overview of developments in assessing and communicating benefits and risks. Develop training material about assessing and communicating benefits and risks.
- Build assessors' knowledge and experience with different approaches and describing value-judgments in the current benefit-risk assessment framework/template.

Activities in 2024

CHMP activities to achieve the objectives set for this area:

- Finalise a reflection paper on single-arm trials that are submitted as pivotal evidence in marketing authorisation dossiers across therapeutic areas following the public consultation.
- To produce training material on benefit-risk assessment and communication in the new optimised assessment report templates (see 1.2.3).
- Set up a focus group in collaboration with CAT to explore the usefulness of preference elicitation in the context of advisory meetings with experts (e.g. SAGs, AHEGs).

CHMP topic leaders: Peter Mol and Thalia Marie Estrup Blicher

Other contributors:

Member/alternate	Name	MS
Chair	Harald Enzmann	DE

Member/alternate	Name	MS
Alternate	Filip Josephson	SE
Co-opted member	Jan Mueller-Berghaus	DE
Vice-Chair	Bruno Sepodes	PT
Member	Kristina Dunder	SE
Alternate	Christian Gartner	AT

1.2.2. Patient and Healthcare Professional involvement in assessment work

The objective is to facilitate engagement of patients and healthcare professionals in benefit/risk evaluation and related activities and reflect their input in CHMP assessments.

In addition, to facilitate the collection and use of patient experience data, so their perspectives and preferences can be considered in benefit/risk evaluations and related activities, along the medicine regulatory lifecycle.

Key objectives

- Maintain current and explore additional processes to capture and include patient experience data within CHMP benefit/risk evaluations.
- To capture and include patient and healthcare professionals' views within CHMP benefit/risk evaluations and reflect this input in assessment reports.

Activities in 2024

CHMP activities to achieve the objectives set for this area:

- Monitor and improve methodologies to capture input to CHMP procedures (including participation in oral explanations, written consultations, and engaging with patient and healthcare professional organisations at start of Marketing Authorisation Applications).
- To contribute to the elaboration of a reflection paper to provide advice on the best EU approach to generate and collect patient experience data.
- Continue to explore how best to reflect in the assessment reports the way that patient and healthcare professional input and patient experience data is assessed and the rationale for acceptance/exclusion for benefit/risk decision-making, linked with the assessment report templates optimisation (see 1.2.1 and 1.2.3).
- Participate in drafting of new ICH guidance on patient experience data and patient preference elicitation.

CHMP topic leaders: Fátima Ventura and Maria Concepcion Prieto Yerro

Other contributors:

Member/alternate	Name	MS
Vice-Chair	Bruno Sepodes	PT
Alternate	Edward Laane	EE
Co-opted member	Carla Torre	PT

1.2.3. Documenting medicines evaluation – an efficiency and stakeholder focus on the CHMP AR and the EPAR

Activity areas

Improve/optimise the initial evaluations assessment report with the aim to simplify, avoid replication of work and meet/consider stakeholders' expectations. Examine the best use of available resources at CHMP and EMA to achieve this goal.

Key objectives

- Review ways to improve the efficiency, robustness, consistency and soundness of outputs throughout the initial MAA evaluation process.

Activities in 2024

CHMP activities to achieve the objectives set for this area:

- Optimise the related assessment report templates (e.g. benefit-risk section, efficacy section of overview template) to avoid duplication of information while facilitating inclusion of all relevant information (e.g. explanation of the therapeutic indication, efficacy and safety in subgroups and outcomes of SAG meetings and oral explanations).

CHMP topic leaders: Daniela Philadelphy

Other contributors:

Member/alternate	Name	MS
Member	Kristina Dunder	SE
Member	Jayne Crowe	IE
Member	Margareta Bego	HR

1.2.4. Digital technologies

Activity areas

Meet the challenge of assessing the benefit/risk of the increasing number of medicines and outcome measures utilising digital technologies. This activity will foster expertise growth and cross-agency cooperation in a fast-developing field while respecting the remit of different stakeholders.

Key objectives

- Ensure coordination and dissemination of learnings from cases (advices, qualifications, MAAs) across EMA activities.
- Continued enhanced cooperation in the area of medical devices.

Activities in 2024

- Qualification of digital technologies: discuss all digital qualification procedures (with additional focus on procedures with AI elements) from Scientific Advice (SA) in CHMP by development of a Digital Technology framework; monitor marketing authorisation applications (MAAs)/extensions with digital aspects (including applications with AI elements) in general and discussions from SA. Feed learnings into discussions on possible future guidance development.
- Expand EMA expert base in the areas of digital, biomechanics and devices, to provide support to different activities, including evaluation and scientific advice.

CHMP topic leaders: Bruno Sepodes

Other contributors:

Member/alternate	Name	MS
Chair	Harald Enzmann	DE
Alternate	Fátima Ventura	PT
Member	Ewa Balkowiec Iskra	PL
Member	Martina Weise	DE
Expert	Sabine Mayrhofer	DE

1.2.5. Contribution of real-word data to evidence generation

Enhanced analysis of data from the development and real-world use of medicinal products has the potential to further support regulatory decision-making. In this area, real-world evidence (RWE) offers the possibility to provide an additional perspective on the use and performance of medicines in everyday clinical use, complementing the evidence obtained from randomised control trials.

Real-world evidence has the potential to enhance decision-making through the lifecycle of medicinal products.

Key objectives

- Identify and test real-world evidence use cases to support evidence generation in Scientific Advice (SA) and CHMP decision-making.
- Ensure expert advice and guidance on real-world evidence is available to support CHMP decision-making.

Activities in 2024

- Continue the pilot initiated on RWE studies to support the provision of SA on the generation of robust RWE.
- Continue the pilot on RWE studies to support CHMP decision-making (including the feasibility to provide RWE on disease epidemiology and standard of care in the elderly). Provide expert input to a review of the experience gained with RWE studies conducted across the regulatory network to support regulatory decision making.
- Expand the existing group of experts on RWE to broaden the support to CHMP and their associated working parties in regulatory activities through the Methodology ESEC.
- Develop a roadmap of RWE guidance to support high-quality RWE generation and continue strengthening the use of RWE for regulatory decision-making. Provide expert input in support to the development of guidance on use of RWE for regulatory purpose, including in the field of pharmacogenomic data linked to real-world data sources.

CHMP topic leaders: Alar Irs

Other contributors:

Member/alternate	Name	MS
Alternate	Christian Gartner	AT
Co-opted member	Carla Torre	PT
Member	Peter Mol	NL

1.2.6. Contribution of individual patient data to evidence generation

Enhanced access to clinical trial data from the marketing authorisation dossier has the potential to further support regulatory decision-making. Targeted analysis and visualisation of 'raw data', i.e. individual patient data from clinical trials in an electronic structured format, will be piloted to understand its impact on benefit-risk assessment and regulatory decision-making through the lifecycle of medicinal products.

Key objectives

- Explore the analysis of raw data from MA dossiers to support the assessment of initial marketing authorisation applications and selected post-authorisation procedures.

Activities in 2024

- Continue the proof-of-concept pilots of analysis and visualisation of raw data from marketing authorisation dossiers to learn of the practicalities and benefits of such an approach.
- Expand Network Community on Raw Data to regularly share developments on the raw data proof-of-concept pilots and foster close collaboration across the Network into using raw data for regulatory decision-making.
- Produce the proof-of-concept pilots' interim report to reflect on the pilot conduct.

CHMP topic leaders: Christian Gartner

Other contributors:

Member/alternate	Name	MS
Co-opted member	Carla Torre	PT
Co-opted member	Bruno Delafont	FR
SAWP	Joerg Zinserling	DE

1.2.7. Strengthening the assessment of Companion Diagnostics

Activity areas

As per the Regulation (EU) 2017/746, a companion diagnostic (CDx) is essential for defining patients' eligibility for specific treatment with a medicinal product. As part of the conformity assessment of a CDx, the notified body shall seek a scientific opinion on the suitability of the CDx with the concerned medicinal product(s) from the competent authorities in accordance with Directive 2001/83/EC before issuing an EU technical documentation assessment certificate or an EU type-examination certificate, or a supplement to them for the CDx. The CHMP seeks to strengthen the assessment process.

Key objectives

- Collaborate in the framework for identification of overarching issues in the assessment of CDx consultation procedures.
- Continued identification of general principles that can be later used for training assessment teams and to update the procedural guidance or assessment templates.

Activities in 2024

- Collaborate with the CDx expert group to consolidate the evaluation of consultation procedures across CHMP members.
- Monitor assessments to capture input in CHMP procedures at iMAAs.

CHMP topic lead: Harald Enzmann

Other contributors:

Member/alternate	Name	MS
Alternate	Fátima Ventura	PT

1.3. Other specialised areas and activities

1.3.1. Geriatric medicines strategy

The rapid aging of the population worldwide means that people over 80 years are the fastest growing subpopulation group. The EMA geriatric medicines strategy aims to ensure that the benefit/risk balance of medicines is researched and evaluated with respect to the epidemiology of the disease, and that findings are adequately reflected in the CHMP assessment documents.

Key objectives

- Make sure the geriatric population is addressed in CHMP assessment reports and product information.

Activities in 2024

- Perform an ex-post control on recently approved initial marketing authorisations to monitor for geriatric information presentation in assessment reports.
- Review selected scientific advice and Day 120 initial marketing authorisation assessment reports to monitor for geriatric information intake.
- Propose amendments to assessment reports and guidance following CHMP review of recently approved and ongoing initial marketing authorisation applications.
- In the context of the CHMP pilot on RWE studies, investigate the feasibility to provide RWE on disease epidemiology, frailty and standard of care in older patients to support the committee decision-making (see 1.2.5).

Reinstate collaborative working with geriatric experts, identified through a public call for expression of interest in 2023.

CHMP topic leader: Andrea Laslop

Other contributors:

Member/alternate	Name	MS
SAWP	Mario Miguel Rosa	PT
SAWP	Elina Rönnemaa	SE
Vice-Chair	Bruno Sepodes	PT
Member	Martine Trauffler	LU
Expert	Sabine Mayrhofer	DE
Co-opted Member	Carla Torre	PT

2. Horizontal activities and other areas

2.1. Committees and working parties

2.1.1. Special populations and product guidance

Certain specific population groups require consideration in the conduct of assessment. This topic channels the Committee's expertise into the development of population specific guidance in terms of risk assessment of medicinal products on human reproduction and lactation in collaboration with PRAC committee.

Key objectives

- Strengthen assessment by industry and regulators through dedicated guidance on specific populations.
- Strengthen systematic generation of information on the benefits and risks of medicines in pregnancy and breastfeeding.

Activities in 2024

- Update of 'CHMP Guideline on risk assessment of medicinal products on human reproduction and lactation: from data to labelling'.
- Revision of the Guideline's text and preparation for the public consultation phase

CHMP topic leader: Jan Mueller Berghaus

Other contributors:

Member/alternate	Name	MS
Member	Ewa Balkowiec Iskra	PL

2.2. Partners and stakeholders

2.2.1. International Regulatory Science Cooperation

Given the increasing complexity of global developments in the pharmaceutical sector there is a drive to achieve greater harmonisation, building a convergence of regulatory tools and standards worldwide to ensure that safe, effective and high-quality medicines are developed, registered and maintained in the most resource efficient manner whilst meeting high standards.

This cooperation is mandated by the globalisation of medicine: in its supply chains, its research and development and its expertise. Reliance, where an authority relies on work done by another authority but retains its full power of decision, is supported by EMA. OPEN (**O**pening our **P**rocedures at **E**MA to **N**on-EU authorities) was established by EMA in 2020 as a framework to increase international collaboration and share scientific expertise on the evaluation of COVID-19 vaccines and therapeutics, initially as a pilot. The benefits and experience gathered during this pilot highlighted the advantages of collaboration across regulatory agencies. Following the success of the pilot, the OPEN scope has been extended to include marketing authorisation applications for: medicines targeting AMR; medicines supported through EMA's PRIority MEdicines (PRIME) scheme, but currently not including advanced therapy medicinal products (ATMPs); and other products that address a high unmet medical need; and medicines responding to health threats or public health emergencies.

Key objectives

- Facilitate the assessment of the same data by multiple authorities.
- Continue to develop a solid and agile framework that strengthens the collective scientific assessment.

Activities in 2024

- Consolidate OPEN scheme process with selected international collaborators.

CHMP topic leader: Sol Ruiz

Other contributors:

Member/alternate	Name	MS
Vice-Chair	Bruno Sepodes	PT
Member	Ewa Balkowiec Iskra	PL
Member	Outi Mäki-Ikola	FI
SAWP	Andrea Laslop	AT