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Human Medicines Division

# EMA-EUnetHTA three-year work plan

2017–2021

## Introduction

The EMA-EUnetHTA collaboration, which began in 2010 based on recommendations from the High-level Pharmaceutical Forum[1], aims to harness synergies between regulatory evaluation and health technology assessment (HTA) along the lifecycle of a medicine. Following initial work to improve the way data published by EU regulators as part of their benefit-risk assessment can contribute to relative effectiveness assessments by HTA organisations, additional topics of mutual interest were identified.

A first EMA-EUnetHTA work plan had been established for the years 2012-2015; a report on the outcomes of this joint work plan has been published in April 2016. Following up on the achievements and developments, a new joint work plan for the years 2017-2021[2] has been agreed. The overall goal of the collaboration is to improve efficiency and quality of processes, whilst respecting their respective remits and ensure mutual understanding and dialogue on evidence needs, to facilitate access to medicines for patients in the European Union.

## Areas for the EMA-EUnetHTA collaboration

EMA and EUnetHTA have jointly identified several areas as focus of their European regulatory-HTA collaboration during 2017-2021. The work plan is complementary to actions foreseen in EUnetHTA Joint Action 3, which runs until 2021. Furthermore, the activities in this work plan will feed into the implementation of the areas for collaboration identified in the reflection paper from the HTA network on Synergies between regulatory and HTA issues on pharmaceuticals and will be developed in close cooperation with the European Commission.

The identified priority areas for collaboration are:

- Early Dialogue / Scientific Advice.

[1] http://ec.europa.eu/enterprise/sectors/healthcare/competitiveness/pharmaceutical-forum/index_en.htm
[2] In view of the extension of EUnetHTA Joint action 3 the duration of this work plan has also been extended to May 2021.

**See websites for contact details**

**European Medicines Agency** www.ema.europa.eu
**EUnetHTA** www.eunethta.eu

- "Late dialogues" / peri-licensing advice.
- Information exchange between regulators and HTA bodies.
- Methodologies to identify the treatment eligible population.
- Significant benefit vs. added therapeutic value for orphan medicines.
- Unmet medical need and therapeutic innovation for priority setting.
- Patient and clinician engagement.
- Shared understanding of methodological approaches for design, analysis and interpretation of clinical trials and observational studies.
- Population-specific or Intervention-specific areas.

Progress on the EMA-EUnetHTA work plan will be monitored and updates will be made when deemed necessary by both parties. The EMA-EUnetHTA collaboration will continuously explore future directions and areas for cooperation, whilst respecting their respective remits.

## *Activities in each of the areas of collaboration*

| Activity | Expected outcomes |
|---|---|
| **Early Dialogue / Scientific Advice** | |
| Design and implement a single, common, European procedure for Parallel Consultation (previously known as parallel scientific advice/early dialogue) | A single process that reflects the evidence generation needs of both regulators and HTABs<br><br>Milestones for launch of single platform for parallel consultation and process reviews<br><br>Communication with stakeholders at each critical design change |
| Facilitate learning and understanding of evidence needs | Mutual observership in scientific advice / early dialogue |
| **"Late dialogues" / peri-licensing advice** | |
| Gaining experience with peri-licensing advice on post-licensing data generation plans with a focus on specific products (e.g., ATMPs) or regulatory processes or tools (e.g., CMA, Adaptive Pathways, or PRIME) | Provision of parallel consultation on requirements for post-authorisation data collection plans (including registries) |
| Optimise utilisation of post-licensing evidence generation for decision making | Collaboration in requirements for data collection and analysis of real world data including registries |
| **Information exchange between regulators and HTA bodies** | |
| Timely provision of the outcome of the regulatory assessment to support joint REA production | Implement a process for the exchange at market authorisation (including timely provision of the REA) to support EUnetHTA joint REAs and align with the needs of most |

| Activity | Expected outcomes |
|---|---|
| | national HTA programmes.<br>Provision of information regarding joint REAs at EMA's pre-submission meetings |
| Respecting the remit and perspectives of both regulators and HTABs, create a mechanism for reciprocal learning opportunities between regulatory reviewers and HTA assessors. | Increased understanding of the regulatory outcome by HTA assessor<br>Increased understanding of the HTA outcome by regulatory reviewers |
| Further optimisation of the regulatory output to facilitate uptake of regulatory outcome by HTAB | Review of the CHMP assessment report template to address findings from the webinars as appropriate |
| **Methodologies to identify the treatment eligible population** | |
| Share experience on how regulators define therapeutic indications and the impact of their wordings in HTABs' definition of the treatment-eligible population. | Clarity on the patient population covered by the approved indication, for the subsequent REA |
| Mutual understanding of the extrapolation concept, including its application for the paediatric population | Discussion and exchange, in parallel with respective guideline development |
| **Significant benefit vs. added therapeutic value for orphan medicines** | |
| Understanding of the similarities and differences between the concepts of significant benefit and added therapeutic value in the context of orphan drugs | Clarity on the concepts for individual products |
| Exchange on product specific reviews at time of authorisation | Increased transparency and understanding of the review of significant benefit |
| **Unmet medical need and therapeutic innovation for priority setting** | |
| Explore how HTABs and regulators interpret the concepts of unmet medical need and therapeutic innovation | A greater understanding of the respective interpretations with the aim of enabling mutually shared perspectives for individual products |
| Explore opportunities to collaborate on monitoring of new medicines' approvals ("horizon scanning") | Clarification of expectations and potential synergies of monitoring approaches by regulators and HTA bodies |
| **Patient and clinician engagement** | |
| Share respective practices and experiences related to the involvement of patients and clinicians in activities | Mutual learning concerning practices for engagement with patients and clinicians in assessment activities |
| Assess the feasibility of developing a shared pool/list | Better utilisation of expertise from patients and |

| Activity | Expected outcomes |
|---|---|
| of contacts | clinicians across decision makers |
| **Shared understanding of methodological approaches for design, analysis and interpretation of clinical trials and observational studies** | |
| Provision of guidance on evidence needs for regulators and HTA bodies, through therapeutic-area-specific guidance, methodological guidance, non-product specific qualification advice and opinions, workshops. | Collaboration in order to allow developers to generate evidence able to address both regulatory and HTA information needs<br>Training to assessors (regulators and HTAs), including potential use of EUNTC as platform to include HTA assessors |
| Better utilization of patient-reported outcomes as part of evidence generation plans | Joint position on the principles for development and validation of patient reported outcomes |
| **Population-specific or Intervention-specific areas** | |
| Address the specific needs for paediatric medicines | Engagement in existing research networks like Enpr-EMA |
| Share practices and experiences with combination products/companion diagnostics | In alignment also with the IVD regulation, establish ways to engage on development programmes also involving Notified Bodies |
| Share information and experiences with ATMPs | Increased understanding of regulatory processes for ATMPs<br>Increased understanding of the reimbursement landscape for ATMPs |

## Regular EMA-EUnetHTA joint meetings

EMA-EUnetHTA meetings will be held on a half-year basis and hosted interchangeably by the EMA and EUnetHTA. Minutes of the meetings will be made publicly available on the websites of the EMA and EUnetHTA.