EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

European collaboration between regulators and Health Technology Assessment (HTA) bodies

Annual PCWP/HCPWP meeting with all eligible organisations

24 November 2021

Presented by Michael Berntgen (EMA) and Bruno Sepodes (CHMP)

European cooperation between regulators and HTA bodies

- Since 2010, EMA and the European Network for Health Technology Assessment (EUnetHTA) have been working closely on topics of mutual interest
- The cooperation was facilitated through coordination of HTA bodies in EU-financed Joint Actions, of which the last one ended in May 2021

  ➔ *Future work arrangements are currently being established*
- The aim of this regulatory/HTA cooperation is to build synergies between regulatory evaluation and HTA along the medicine lifecycle

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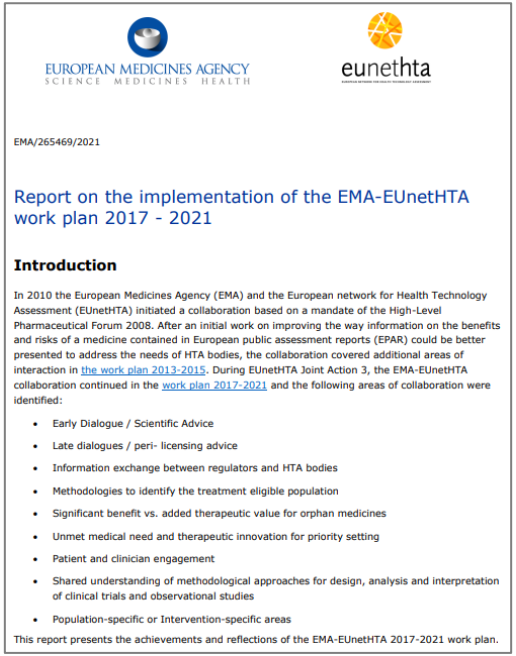
EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

Report on the EMA / EUnetHTA work plan 2017-2021

- Early Dialogue / Scientific Advice
- 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



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EMA/265469/2021

Report on the implementation of the EMA-EUnetHTA work plan 2017 - 2021

Introduction

In 2010 the European Medicines Agency (EMA) and the European network for Health Technology Assessment (EUnetHTA) initiated a collaboration based on a mandate of the High-Level Pharmaceutical Forum 2008. After an initial work on improving the way information on the benefits and risks of a medicine contained in European public assessment reports (EPAR) could be better presented to address the needs of HTA bodies, the collaboration covered additional areas of interaction in the work plan 2013-2015. During EUnetHTA Joint Action 3, the EMA-EUnetHTA collaboration continued in the work plan 2017-2021 and the following areas of collaboration were identified:

- Early Dialogue / Scientific Advice
- 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
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- Shared understanding of methodological approaches for design, analysis and interpretation of clinical trials and observational studies
- Population-specific or Intervention-specific areas

This report presents the achievements and reflections of the EMA-EUnetHTA 2017-2021 work plan.

[Technical reporting - work plan 2017-2021 May 2021]

Highlights (1/7): Early Dialogue / Scientific Advice

Single process for Parallel Consultation:

- 31 completed Consolidated Parallel Consultations
- 28 completed Individual Parallel Consultations
- 2 Parallel consultations on registry qualification
- Parallel consultation on the qualification of an IMI project

Reflections

- Demand from developers exceeded the capacity by EUnetHTA JA3 members (adequate financial resourcing necessary)
- EUnetHTA paused Parallel consultation due to COVID-19 reprioritisation
- Reviewing templates and procedures allowed to implement some HTAs requirements

Highlights (2/7): Post-licensing data generation plans

Use of Parallel Consultation platform:

- 2 Parallel consultations on registry qualification; 12 Parallel consultations including recommendations on PLEG; 2 Parallel consultations specifically on PLEG

Scientific article "Regulatory and health technology assessment advice on PLEG"

Collaborations on requirements for data collection and analysis of real world data

Reflections

- More proactive proposals from developers for discussion in Parallel Consultation needed
- Identification of PLEG requirements as a result of joint REA follow-up activities needs to be expanded
- Collaborative work on registry methodologies identified as priority

Highlights (3/7): Exchange at time of decision making

Framework for provision of the final CHMP assessment report in the context of joint REA production developed

- Exchange of reports for 14 products, with follow-up webinar for 13 of these

Additional product-specific webinars

Identification of elements for optimisation of the regulatory assessment report

Reflections

- Value of direct discussions confirmed by participants (regulators and HTAs)
- Administrative burden due to the need for project-specific confidentiality arrangements was considerable
- Experience reviews recommended, also to be complemented with information sessions / trainings

Highlights (4/7): Identification of treatment eligible population

Value of the EMA/CHMP guide to assessors on the wording of therapeutic indication for HTA purposes (interpretation of the information contained in SmPC)

Discussion on the reflection paper on the use of extrapolation, followed by exchange on assessment guidance

Joint workshop held on extrapolation, with special focus on development of paediatric medicines

Reflections

- Future development of guidance on SmPC section 5.1, including information on sub-populations
- Continue sharing concrete experience from using labelling and EPARs for decision making
- Important to understand each other's reasoning for accepting extrapolation, acknowledging the different remits

Highlights (5/7): Orphan medicines / Unmet medical need

Article "Assessment of significant benefit for orphan medicines by European regulators may support subsequent REAs by HTAs"

Development of the Orphan Medicines Maintenance Reports (OMARs) concept

Article "Unmet Medical Need: An Introduction to Definitions and Stakeholder Perceptions"

Reporting format for HTA horizon scanning

Reflections

- Improved the understanding from all participants about the different concepts relevant for orphan medicines
- Follow-up review how OMARs are being taken up by HTAs
- Need to explore further collaborative work on the UMN concept

Highlights (6/7): Patient and clinician engagement / Evidence generation methodologies

Mutual learning regarding patient engagement practices in assessment

EMA provided contacts for 11 targeted consultation on EUnetHTA assessments

17 patients in 17 parallel EMA/EUnetHTA scientific advice procedures

Commenting by HTA bodies on relevant scientific guidelines (e.g. estimands)

Joint participations in external events on PRO methods

Reflections

- Further collaboration regarding patient / expert engagement focus on sharing respective practices and experiences
- Work on methodological issues and guidelines is considered of importance
- Future collaboration on better utilization of patient-reported outcomes (e.g. discussion of methodologies)

Highlights (7/7): Population-specific or Intervention-specific topics

Discussion on principles of benefit assessment of paediatric medicines by European HTA bodies

HTA response to the EMA concept paper on development and lifecycle of personalised medicines and companion diagnostics

Joint ATMP workshop to increase mutual understanding of the regulatory process and the reimbursement landscape

Reflections

- Increase awareness of the specificities and possible limitations of evidence generation in paediatric medicines
- Importance to share practices and experiences with combination products/companion diagnostics
- Joint training session between EMA and HTA bodies on ATMPs, as suggested in the EC/EMA's action plan on ATMPs

Continued cooperation post-Joint Action

EMA and EUnetHTA at their last bilateral during Joint Action 3 (report) agreed to establish a list of priority areas for future collaboration between regulator and HTA at European level to continue future collaborative work

The overall goal of such collaboration is to improve efficiency and quality of processes, whilst respecting the respective remits of different decision makers, and ensure mutual understanding and dialogue on evidence needs, to facilitate access to medicines for patients in the European Union.

DRAFT priority areas for future collaboration

Product-specific work	***Methodological work***	***Operational work***
Joint scientific consultation on evidence generation, including PLEG	Study methods and guide-lines of real-world evidence, including for registries	Continuous optimisation of regulatory outputs
Exchange of information on the respective assessments of medicinal products by regulators and HTA bodies	Generation of patient relevant data / information to support decision making	Methodologies for engage-ment of patients and HCPs
Evidence planning and assessment for advanced therapy medicinal products	Extrapolation / evidence transfer as tool to support assessment in smaller populations	Practices in the context of companion diagnostics
		Horizon scanning and preparedness of HTA and regulatory systems

Take home messages

- The joint technical work of the EMA/EUnetHTA cooperation has been the foundation for establishing mutual trust and understanding
- Based on the experiences (see technical report), priority areas for future collaboration between regulator and HTA at European level have been developed to continue future collaborative work
- The consortium EUnetHTA21, which was awarded the “Service Contract for the Provision of Joint Health Technology Assessment (HTA) Work Supporting the Continuation of EU Cooperation on HTA”, will be instrumental in this delivery