“Sequential decision making”: how regulatory and HTA activities inter-connect under the new HTA Regulation

PCWP/HCPWP annual meeting with all eligible organisations

Presented by Michael Berntgen on 15 November 2022
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An agency of the European Union

Outline

1. Background of the collaboration between regulators and HTAs on European level
2. Experience with such collaboration: concrete examples
3. Looking ahead: collaboration under the new HTA Regulation
4. Ongoing implementation activities under the joint EMA / EUnetHTA21 work plan

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European collaboration between regulators and health technology assessment bodies

Joint work plan (2021-2023) between EMA and European HTA bodies facilitated through EUnetHTA21

Introduction

EMA and the previous European Network for Health Technology Assessment (EUnetHTA), which was established through consecutive Joint Actions of which the last one concluded in May 2021, started their collaboration in 2010 based on recommendations from the High-level Pharmaceutical Forum[1], with the aim to harness synergies between regulatory evaluation and health technology assessment (HTA) along the lifecycle of a medicine. A first EMA-EUnetHTA work plan was established for the years 2012-2015 and a report on the outcomes of this joint work published in April 2016. Subsequently, a second joint work plan for the years 2017-2021 was agreed and a report published in June 2021.

Following up on the achievements through such cooperation and the mutual trust and understanding developed through this joint technical work, EMA and EUnetHTA at their last bilateral during Joint Action 3 (minutes) agreed to establish a list of priority areas for future collaboration between regulators 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.

Subsequent to the award of the "Service contract for the provision of joint Health Technology Assessment (HTA) work supporting the continuation of EU cooperation on HTA" to the EUnetHTA21 consortium, the European Commission has invited EMA and EUnetHTA21 to establish a joint work plan for delivering on the previously identified priorities. Deliverables on HTA side will either be actioned by EUnetHTA21 if related to their service contract delivery, or alternatively through individual HTA bodies from the consortium or beyond, who are from a European (EU/EEA) Member State and express interest to participate. In the latter situation, individual HTA bodies represent their own position and not the views of EUnetHTA21. In addition, all deliverables part of EUnetHTA21 will be subject to a public consultation, to which EMA is invited to participate.

EMA and the EUnetHTA21 secretariat will keep an oversight of all activities.

1 "Sequential decision making": how regulatory and HTA activities interconnect under the new HTA Regulation

The common ground: evidence as basis for decision making

Starting point: decision makers have different objectives

- Medicines regulators (like EMA) assess the benefit/risk of a medicinal product
- HTA bodies contextualise the use of the medicine compared to other technologies

Hypothesis: Alignment of thinking across decision makers should facilitate access → decision makers come together early to discuss

- the planned development including populations / comparators / design of trial / endpoints
- the requirements for post-licensing evidence generation

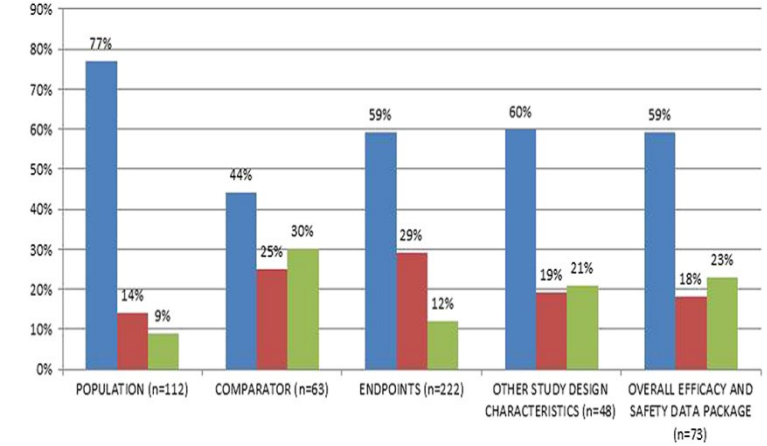
Expectation: Optimised evidence generation plan meeting the needs for different type (scope) of decision making

European collaboration 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 initially facilitated through coordination of HTA bodies in EU-financed Joint Actions, and now continues under a tender for service
- The aim of this regulatory/HTA cooperation is to build synergies between regulatory evaluation and HTA along the medicine lifecycle
- **The new HTA regulation in Europe recognises the value of such collaboration**

Value of cross-decision maker engagement – some examples

Evidence planning

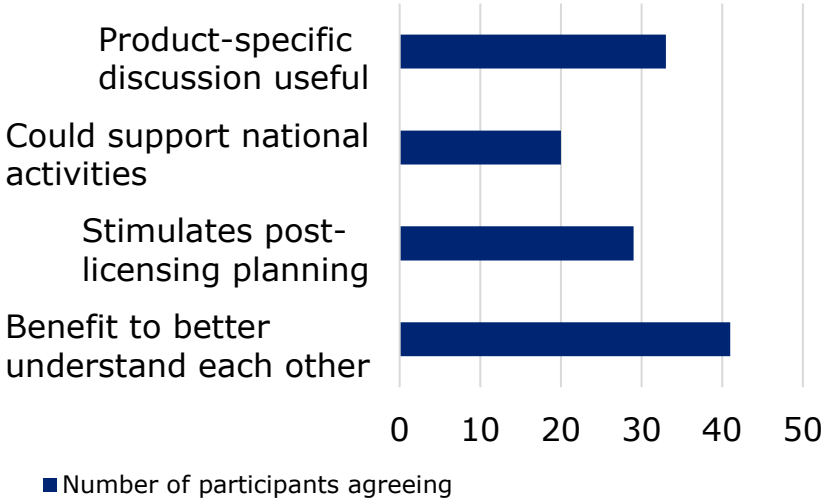


Level of agreement (position of HTA bodies *vs.* regulators; review of clinical trial features based on of 31 scientific advice procedures):

full agreement partial agreement disagreement.

Tafuri et al., British J Clin Pharm, Volume 82, Issue 4, 965-973

Discussion of assessments

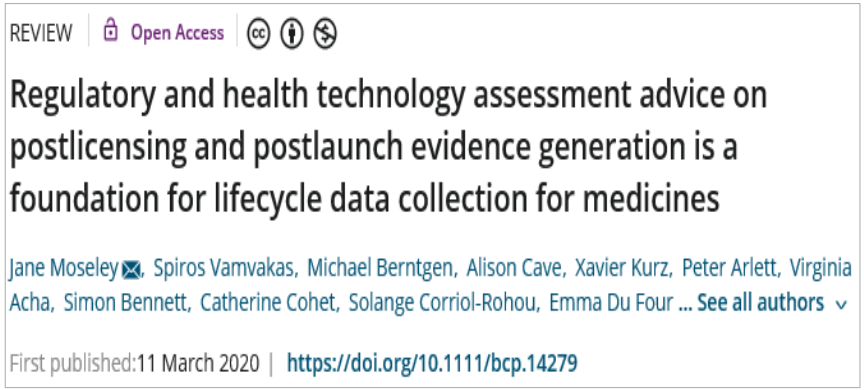


Survey responses for REA001-004, 007-009

Joint discussion on methodologies for Post-licensing/launch evidence generation

PLEG* is part of the continuum of evidence development for a medicinal product:

- complementing earlier evidence
- facilitating further elucidation of a product's benefit/risk profile or value proposition
- exploring broader aspects of disease management and provision of healthcare



REVIEW | Open Access

Regulatory and health technology assessment advice on postlicensing and postlaunch evidence generation is a foundation for lifecycle data collection for medicines

Jane Moseley, Spiros Vamvakas, Michael Berntgen, Alison Cave, Xavier Kurz, Peter Arlett, Virginia Acha, Simon Bennett, Catherine Cohet, Solange Corriol-Rohou, Emma Du Four ... See all authors

First published:11 March 2020 | https://doi.org/10.1111/bcp.14279

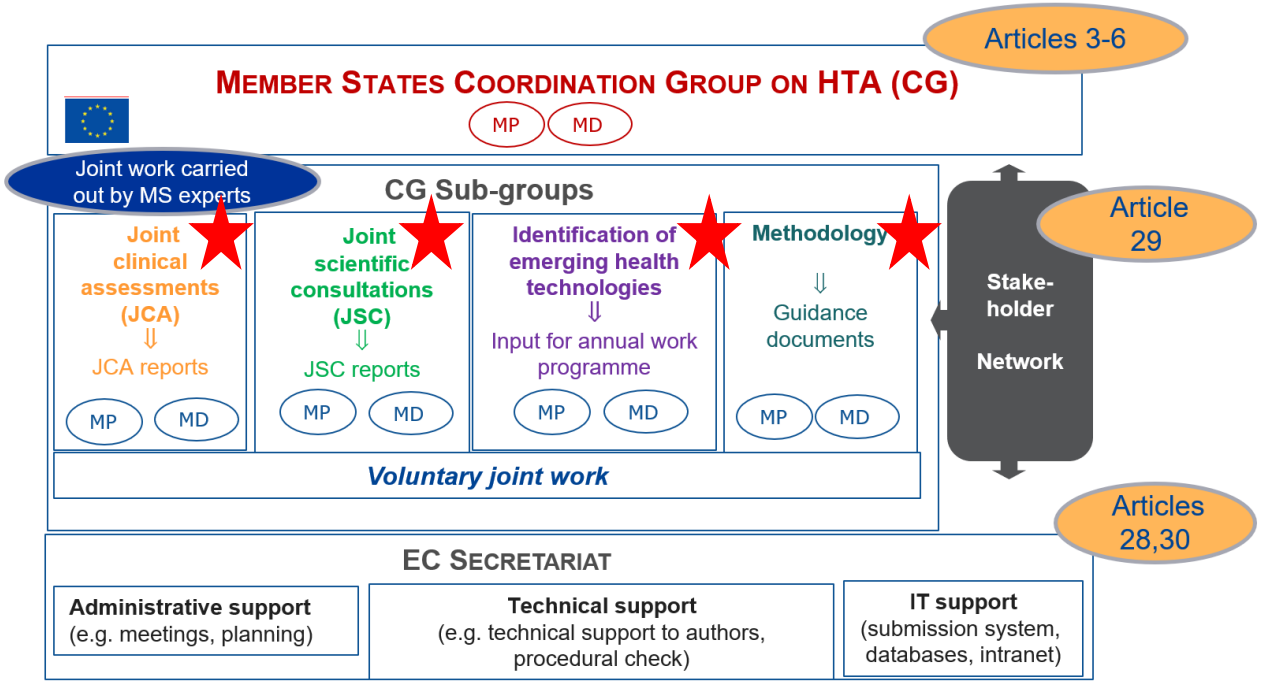
British Journal of Clinical Pharmacology

Advice involving different decision makers aims to optimise the PLEG plan to address remaining uncertainties after licensure and launch

* PLEG = Post-licensing/launch evidence generation

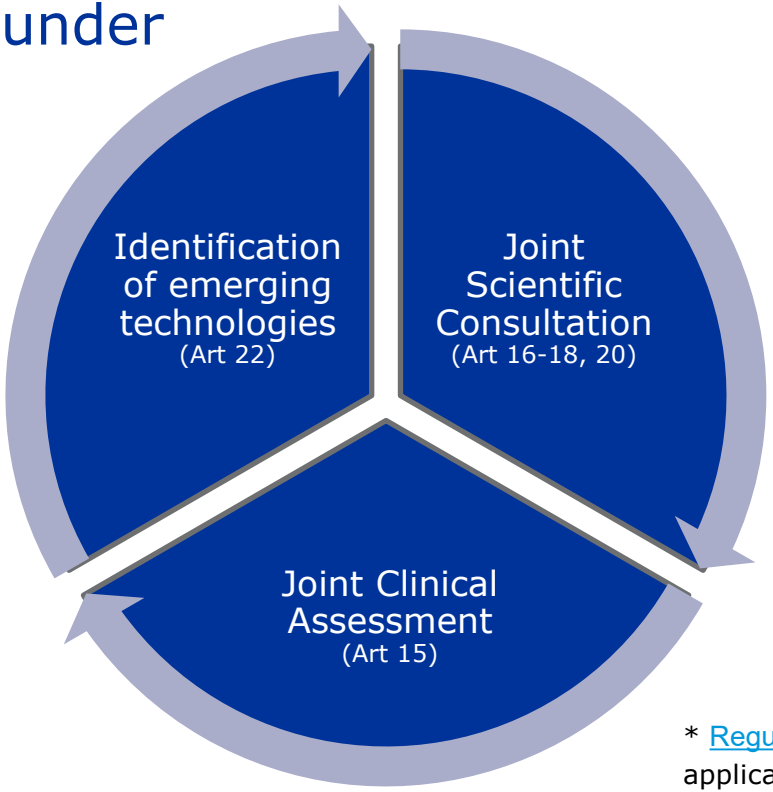
Governance of HTA cooperation under the new Regulation

★ Touchpoints for engagement with medicines regulators



Collaboration with EMA under the HTA Regulation*

Further details through Implementing Acts



Note: "Joint" refers to collaborative work amongst HTA bodies

Transversal elements in the Regulation to facilitate such work, supported by the European Commission, include the sharing of confidential information

* Regulation (EU) 2021/2282, applicable from January 2025

Supporting the implementation work together with the EUnetHTA 21 consortium

EMA and the EUnetHTA 21 consortium set priorities for their collaboration

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News 12/04/2022

The European Medicines Agency (EMA) and the European Network for Health Technology Assessment (EUnetHTA) 21 consortium have published a joint work plan until 2023. The focus of the plan is to prepare, from a methodological and operational perspective, for the coming into application of the Regulation on Health Technology Assessment (EU) 2021/228 in January 2025 after a 3-year implementation period.

The work plan continues the close collaboration between EMA and Health Technology Assessment (HTA) bodies in Europe, which began in 2010 through consecutive EUnetHTA Joint Actions of which the last one concluded in May 2021. This collaboration has demonstrated many synergies between regulatory evaluation and HTA along the lifecycle of a medicine and aims to facilitate patients' access to medicines in the European Union.

Contribution through the joint EMA/EUnetHTA21 work plan

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

- Oversight through biannual EMA/EUnetHTA 21 bilaterals (most recent on 17th June 2022)
- In addition, exchange on selected EUnetHTA 21 deliverables

Take home messages

- Discussions across decision-makers are crucial to **better guide on evidence requirements** throughout the medicine's lifecycle, including post-authorisation evidence
- The **HTA Regulation recognises the value** of collaboration at the regulatory/HTA interface and implementation work is ongoing together with all stakeholders
- There are multiple opportunities to **progress evidence methodologies** together to facilitate the design of development plans and support assessment work, within the respective remits

Thank you for your attention

Further information

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