



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

European Medicines Agency collaboration with Health Technology assessment Bodies

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An agency of the European Union





Shared interests between Regulators and HTA bodies

Goal- Why?

Stakeholders - Who?

Scientific principles – What?

Environment - Where?



Life-cycle overview

Discovery/Manufacture

Non-clinical

Pharmacovigilance
Risk Management

Clinical

Human
Pharmacology

Therapeutic
Exploratory

Therapeutic
Confirmatory

Therapeutic
Use

("Phase I")

("Phase II")

("Phase III")

("Phase IV")

Scientific Advice

ITF

Paediatric Investigation Plan

Orphan Drug
Designation

Extension
Application

Maintenance
Procedures

Marketing
Authorisation
Application

Health
Technology
Assessment



Strengthening collaborations

Ongoing activities developing and strengthening synergy

- Parallel scientific advice,
- EMA-EUnetHTA cooperation, public assessment reports
- Post Authorisation data, registries and studies
- Guidelines, ENcePP HTA working group, GetREAL

HTAN Strategy Paper



Parallel EMA HTA scientific advice- why

Newly licensed medicines do not reach all patients in need

Regulators and HTAs

- answer different questions
- have different requirements in terms of evidence

Aim: stakeholders come together early

- to discuss the planned development including
- Populations/Comparators/design of trial/endpoints

Optimised development plan - Improve access for patients



EMA HTA parallel advice: experience to date

- **35 parallel EMA – SA procedures** with EU HTA bodies variously from England, Italy, Germany, Sweden, France, Netherlands, Spain, Belgium
- **Broad range of indications:** Lung cancer, Breast cancer, Pancreas cancer, Melanoma, Asthma, COPD, Diabetes, Heart Failure, Depression, Alzheimer's, Migraine, Infections, Rare diseases, Myasthenia Gravis



Parallel scientific advice: co-operating on process

- EMA/HTAs **equal** partners- multi-stakeholder procedure
- Maintain respective **roles** and **responsibilities**
- **Common Briefing** document- Present concise but comprehensive data-value proposition for HTAs
- **Closed** sessions, Process working group including HTAs; further co-operation and harmonisation on process





Parallel advice: Co-operating on science

- Interaction between HTA and regulators; **listening** to each others views, improves **understanding**
- **Alignment** on data requirements if possible or efficiency gains if not
- **Willingness** to promote efficient data collection
- Avoiding excess burden on **patients** /'gold plating' programme
- Applicants appreciate knowing **divergences** - leading to informed decision making by the developer; mechanisms to handle divergence



Shaping European Early Dialogues (SEED)

September 2013, under the coordination by HAS

14 HTABs initiated the SEED project

Objective: to **perform** 10 multi-HTA Body early dialogues and explore possible **scenarios** for future.

EMA pleased to be associated with 3 of 7 pharmaceutical procedures as EMA SEED procedures

See **closer working** to facilitate Tripartite meetings

EMA and SEED partners agreed need for more premeeting interactions



SEED is funded by the European Union in the framework of the EU Health Programme 2008-2013



Scientific advice interactions - future?

Continue to develop collaboration

- EMA-Multi HTA parallel scientific advice
- SEED – further experience
- Build on Workshop – lessons learned- positive common vision
- Anticipate **all outputs** will be carefully taken into account and assessed to lead possibly to a revised workflow/ process to best meet the objective of the Early Dialogue exercise in the medium term.
- EMA HTA consultation and collaboration essential to find a process that meets the **needs of all**



EMA-EUnetHTA 3 year work plan 2013-2015

- To identify **opportunities** for, and to implement, **improvements** to the efficiency of the process and conditions for patients' timely access to an effective medicine.
- Semi annual **meeting** equal partners; decision makers
- Coordination and broad scientific **issues**
- **Transparent-** published minutes
- Fruitful exchanges and communication
- Progress check on work plan items



European Public Assessment Reports (EPARs) “EPAR improvement”

Mandate from the High Level Pharmaceutical Forum:

6.4 Member States, with the involvement of the European Medicines Agency, should continue their efforts to consider how European Public Assessment Report and the National Public Assessment Report can further contribute to relative effectiveness assessments.

2 year project ; revised **templates**; better understanding of data needs

Result: Improved data presentation for the **usability** for HTA bodies



Completion of first joint project on European level

Improving the Contribution of Regulatory Assessment Reports to Health Technology Assessments—A Collaboration between the European Medicines Agency and the European network for Health Technology Assessment

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COLLABORATION BETWEEN REGULATORS AND HTA ORGANIZATIONS TO IMPROVE HEALTH CARE DECISION MAKING

Posted: Thursday, June 26, 2014

[Outcome report on first European collaboration between regulators and HTA organisations: improving the contribution of regulatory assessment reports to health technology assessment](#)

PRINCETON, NJ, June 25, 2014 (ISPOR) -- As announced [today](#) by the European network for Health Technology Assessment (EUnetha) and European Medicines Agency (EMA), *Value in Health*, the official journal for the International Society for Pharmacoeconomics and Outcomes Research (ISPOR), was pleased to publish the following article, "[Improving the Contribution of Regulatory Assessment Reports to Health Technology Assessments – A Collaboration between the European Medicines Agency and the European network for Health Technology Assessment](#)," which details the results of a recent collaboration between the EMA and EUnetha. This is the first joint project of such collaboration between regulators and HTA bodies on a European level.



Post-authorisation

- Risk management plans
- **Opportunity** for dialogue between regulators and HTA bodies
- maximises **convergence** of information needs
- Data and information **sharing**



Registries

- Registries: **Organised** observational data collection
- **Challenges**
- Ongoing EU initiatives e.g. **PARENT** joint action
- Could we apply standard **tools** as increases value and comparability of the collected data



Post authorisation efficacy studies

- Delegated Regulation 357/2014 Entered into force (April 2014)
- Expands framework for PAES imposition beyond existing legal frameworks
- May be required **At the time of granting the marketing authorisation**: concerns on some aspects of efficacy of the product can be resolved only after the medicinal product has been marketed
- **After granting the marketing authorisation**: the understanding of the disease or the clinical methodology or the use of the medicinal product under real-life conditions indicate that previous efficacy evaluations might have to be revised significantly



Adaptive Licensing (Adaptive Pathways)

- Promote **efficient** drug development programmes - to inform licensing, reimbursement and prescribing decisions.
- Optimal use of **available tools** with multi-stakeholder dialogue
- The discussion is a non binding, **safe-harbour** brainstorming. Not a new procedure, not a new approval route.
- Lifecycle outlook: better use of **RWD** (Real World Data),
- Request for **parallel** EMA/HTA advice is expected to follow (in depth discussion of requirements).
- Positive **Benefit/Risk** will be required at Approval stage



Guidelines

EMA guidelines sent to EUnetHTA:

- All guidelines under public consultation
- General and Disease specific

EUnetHTA guidelines sent to EMA:

- EMA set up a group of representatives from CHMP, SAWP and EMA to review the nine general EUnetHTA guidelines available
- Comments provided at public consultation
- First disease specific pending- Principle for exchange agreed



ENCePP* HTA Working Group

- Academics and others with relevant expertise to build **capacity** for the conduct of **post-authorisation studies** that meet the needs of regulators and HTA bodies in a resource-conscious and efficient manner
- 2014 survey of the network:
 - ✓ confirmed a **proportion** of ENCePP centres have experience in conducting studies with endpoints directly relevant to HTA.
 - ✓ Identified the 'top three' **training** needs were comparative effectiveness research (60% of respondents), healthcare resource utilisation (43%) and patient reported outcomes methods (43%).
- Priority is to **further enrich** the group with best available expertise from EUnetHTA and others



* *European Network of Centres for Pharmacoepidemiology and Pharmacovigilance.*



IMI GetReal <http://www.imi-getreal.eu/>

- Contribution of **Real World Data** (RWD) to development plans is explored via simulations on case studies (1st: Multiple sclerosis)
- Stakeholders: regulators, HTA bodies, companies, academia/health care professionals, patients
- Looking at RWD and
 - **decision making** during development
 - **statistical/analytical techniques** incorporating both CT and RWD
 - Simulation of different **development options**.
 - the **relevance/robustness** of knowledge generated during development, including quality of RWD

Aiming to better inform **decision making** and improve the efficiency of the R&D process



European Medicines Agency and the Health Technology Assessment Network (HTAN)

- Common interest between HTA and regulators are many
- **Strategy of Network** adopted Oct 2014. Chapter 2.3
- **Regulatory collaboration with HTAs** appears important in view of mutual interests, complementary activities, experience and ultimate goal of patient benefit
- Interactions positive and developing
- EMA will work with the Network to ensure Regulatory-HTA interactions are congruent and sympathetic to needs of each
- http://ec.europa.eu/health/technology_assessment/docs/2014_strategy_eucooperation_hta_en.pdf



Thank you

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