



# **Safe and Timely Access to Medicines for Patients (STAMP) EC Expert Group - An update -**

**Presented by: Olga Solomon, Deputy Head of Unit D5  
Medicinal Products – Authorisations, European Medicines Agency  
Directorate General for Health and Food Safety**

# Challenges for patient access to innovative medicines\*

- Need to facilitate the translation of scientific advances into innovative medicines that meet regulatory standards, accelerate patients' access to innovative therapies with added value for patients and are affordable to the EU Member States' health systems
- Development of innovative medicines is costly, time consuming and includes risks; this may result in insufficient investment in R&D
- Due to high prices of some innovative medicinal products in relation to their benefit to patients and to the public health expenditure capacities of some Member States, patients do not always have access to innovative treatments

*(\*from Council Conclusions of 1.12.2014 on innovation for the benefit of patients)*

# European Medicines Agency's Project on Pathways *(previously Adaptive Licencing)*

- initial approval in a well-defined **patient subgroup** with a **high medical need** and **subsequent widening** of the indication to a larger patient population, where **uncertainty is reduced** through the collection of **post-approval data** on the medicine's use in patients.
- relevant for medicines with the potential to treat serious conditions where there is an unmet medical need.
- cooperation between a wide range of stakeholders including HTA bodies.
- EMA Pilot launched in March 2014





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# Reflection process in the Pharmaceutical Committee

**What is the relation between  
pharmaceutical regulatory framework  
and timely access of medicines to  
patients?**



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## Scope and objectives

**On advice of the  
Pharmaceutical  
Committee, Commission  
Group of experts on  
“Safe and Timely Access  
to Medicines for  
Patients” (STAMP)**

- **to provide advice and expertise to the Commission services in relation to the implementation of the EU Pharmaceutical legislation**
- **Exchange views and information about Member States’ experience and initiatives**
- **Identify ways, to use more effectively the existing EU regulatory tools: conditional marketing authorisation, exceptional circumstances, accelerated assessment**
- **Explore, where possible, ways to increase information-sharing and cooperation among Member States.**

**Main goal: Optimise the use of current regulatory tools to further improve safe, timely access and availability of medicines for patients**

## Limitations

- The expert group is not mandated to provide advice with the aim to revise the basic acts Directive 2001/83 and Regulation 726/2004
- **HTA as well as pricing and reimbursement policies will not be the primary focus of the STAMP**

## Synergies

- **Synergies** will be created with other groups and European activities: **HTA network**, the Network of Competent Authorities on Pricing and Reimbursement (CAPR), the Process on Corporate Responsibility in the field of Pharmaceuticals, EMA pilots, the SEED Consortium and the Council Working Party on Public Health at Senior Level.



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## Members

- **Member States, EMA and EEA countries**
- **Representatives appointed by the Member States for their expertise on the topics to be discussed: permanent representatives or representatives on an *ad hoc* basis depending on the meeting agenda.**
- **The Commission services may invite experts with specific competence in a subject on the agenda to make a presentation or take part in the work of the group on an *ad hoc* basis.**

## Meeting frequency and duration of the activity

- **Max. four meetings per year.**
- **STAMP is temporary working group for the period of time needed to complete its tasks.**

## Activity Report

- Meetings documents and summary records will be published on the dedicated webpage under the following link:  
[http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index\\_en.htm](http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm)



# STAMP 1<sup>st</sup> meeting on 27 January 2015

- Exchange of experiences from national routes (other than clinical trials) for making available medicines to patients before authorisation
- Regulatory tools for early access
  - **Experience with conditional marketing authorisations (CMA)**
  - **Experience with Accelerated assessment procedure**
- EMA's adaptive pathway pilot project

# Member State early Access Schemes

- **Federal Agency for Medicines and Health products – Belgium**

national legislation on early temporary authorisation through (i) compassionate use and (ii) medical need programs. changes in law entered into force on 1 July 2014

- **Ministry of Health – France**

French scheme of Temporary Authorisation for Use (TAU), based on two types of TAU status: (i) named-patient basis and (ii) cohort basis approach

- **Agency of Medicines and Medical Devices - Spain**

The regulation in Spain allowing patient access to medicines before authorisation through (i) compassionate use and (ii) off-label use was presented.



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# EMA's pilot project on Adaptive Pathways

## The criteria for the candidate selection in the pilot project:

1. An iterative development plan
2. Ability to engage HTAs and other downstream stakeholders
3. Proposals for the monitoring, collection and use of real-world data, post-authorisation, as a complement to RCT data.
4. Unmet medical need.

39 products were submitted as candidates; 11 were selected for in-depth discussion of which 4 from SMEs, 5 orphans and 2 Advanced Therapy Medicinal Products (ATMPs)

## Issues for further discussion:

- How to facilitate **harmonisation/interchange between** data sources
- Tools to control **prescription**/input; partnership **PRAC/HTA**
- Quality of real world data/build on national experience on **registries**

# Conditional Marketing Authorisation

**When:** For certain categories of medicinal products, in order to meet unmet medical needs of patients and in the interest of public health, it may be necessary to grant marketing authorisations on the basis of less complete data than is normally required. Granting of a marketing authorisation subject to certain specific obligations to be reviewed annually ('conditional marketing authorisation').

**How to optimise the use of CMA.** Several issues identified for further discussion. Relevant for HTA:

- ✓ Difficulties with HTA bodies due to lower evidence and higher uncertainty at time of authorisation.
- ✓ Difficulties to demonstrate significant benefit in case of orphan medicines.
- ✓ Overview of regulatory tools appropriate for cases on non-compliance with SOs.
- ✓ Early discussion of development product including with HTA bodies.



# Thank you!

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***Public Health information:***

***[http://ec.europa.eu/health/index\\_en.htm](http://ec.europa.eu/health/index_en.htm)***

***STAMP documents and reports:***

***[http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index\\_en.htm](http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm)***