EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Health systems and products
Medicinal products – authorisations, European Medicines Agency

STAMP 3/18

Record

STAMP Commission Expert Group

20 October 2015

3rd meeting

RECORD

The Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) held its 3rd meeting on 20 October 2015, in Brussels, chaired by Unit SANTE D5 - *Medicinal products – authorisations, EMA*. Representatives from 24 Member States and the European Medicines Agency participated at the meeting.

1. APPROVAL OF PREVIOUS MINUTES

The record of the first STAMP meeting (STAMP 2/12) was approved without changes.

2. ADOPTION OF THE AGENDA

The draft agenda (STAMP 3/13) was adopted without changes.

3. REGULATORY TOOLS FOR EARLY ACCESS:

a. Conditional marketing authorisations (CMA)

The European Medicines Agency (EMA) presented a summary of comments received during the public consultation on the CMA guideline. The Committee for Medicinal Products for Human Use (CHMP) will discuss the guideline and its adoption is planned in Q1 2016.

The 2nd STAMP meeting had identified some legal/regulatory aspects that needed to be analysed.

The discussion during the 3rd STAMP meeting focused on defining the problem, if any, and exploring possible solutions and options that should be further investigated by the Commission services with regard to the existing legal framework and within the better regulation context.

The first element discussed concerned the possibility to amend an existing marketing authorisation to include a new 'conditional' indication, as opposed to the current situation where a separate new marketing authorisation would need to be requested. The group considered the advantages and disadvantages of the two options. In order to have this possibility of amending an existing marketing authorisation, Commission Regulation (EC) No 507/2006 on the conditional marketing authorisation[1] would need to be amended.

There was discussion on other possible changes to this Regulation in order to:

- reinforce actions in case of non-compliance with specific obligations,
- reinforce the prospective planning of CMA application,
- address the particular difficulty of conditional marketing authorisation of orphan medicinal products

The STAMP generally supported the ideas that had been put forward and the Commission services will further consider them, including legal aspects, following the better regulation rules.

b. Accelerated assessment

- **Revised CHMP guidelines and summary of comments received during public consultation**

The EMA gave an overview of proposed changes and comments received during public consultation on the CHMP guideline on accelerated assessment. Its finalisation is expected in Q4 2016.

- **New scheme to support development of innovative medicines for unmet medical needs**

The EMA presented the new scheme to support development of innovative medicines for unmet medical needs – Priority Medicines (PRIME). The intention is that the proposed PRIME scheme would provide regulatory and scientific support and accelerated assessment to selected medicines.

Several points were raised during the discussion covering:

- the early appointment of a CHMP rapporteur,

- the eligibility criteria, in particular consideration of major public health interest and unmet medical need,

- the monitoring of the development of the scheme and the need for identification of clear criteria for possible withdrawal of medicinal products from the scheme.

[1] Commission Regulation (EC) No 507/2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) N°726/2004 of the European Parliament and of the Council OJ L 92, 30.03.2006, p. 6

There was overall support to apply strict criteria for eligibility to the PRIME scheme, i.e. it should be 'needs driven' and products selected should bring significant benefit over existing therapies.

The points identified by STAMP were transmitted to the EMA for consideration before the launch of the scheme.

c. Adaptive Pathways

- **discussion on policy related issues on the basis of current experience from the EMA's pilot project *and* EMA's proposal for a strategy on registries**

EMA summarised the Adaptive Pathways concept, its development and the experience acquired so far from the submissions and accepted projects within the pilot project. The lessons learned on: real world evidence and registries, the iterative development process and other stakeholder involvement, in particular health technology assessment (HTA) bodies, were presented.

The following points were raised or clarified during discussions:

- extension of indications based on real world data and potential risk of off-label use,
- the use of registries, legal and operational issues,
- opportunities and limitations in participation of HTA bodies in the adaptive pathways pilot projects mainly due to resource limitations.

A questionnaire will be prepared by EMA and circulated to the STAMP members, EUNetHTA and the Network of Competent Authorities on Pricing and Reimbursement to collect their views on the feasibility aspects of adaptive pathways that emerged during the adaptive pathways pilot and the STAMP discussions.

The need of further discussion on compassionate use and its more effective use was raised.

- **"Prescrire" position paper on Adaptive Pathways**

"Prescrire", is a non-profit educational, independent journal about medicines. *Ms Teresa Leonardo Alves, International Policy Advisor* at Prescrire and *Mr Pierre Chirac, Section Editor* at Prescrire, explained their concerns on adaptive pathways, inter alia, lowering the evidence requirements, shifting the burden of evidence from pre-marketing to post-marketing, the lack of transparency and information publicly available. Prescrire explained that their position paper[2] and the presented statistics were based on the limited information on the adaptive pathways pilot that is available on the EMA website.

EMA explained that this scheme is not of universal applicability but is designed for a restricted group of products with specific characteristics.

Prescrire underlined that in their opinion the main role of the regulatory authority is to protect the public health. If the same authorities are responsible for developing innovative medicines and at the same time are evaluating them, in their opinion, it is a potential conflict of interest. Prescrire suggested that the EMA should do comparative assessment against existing medicines.

[2] http://ec.europa.eu/health/files/committee/stamp/2015-10_stamp3/3c_prescrire_position_paper.pdf

The Commission service clarified that the adaptive pathways scheme follows the existing framework. It was stressed that none of the products can get marketing authorisation if the requirements of the legislation are not fulfilled; notably if the benefits do not outweigh the risks for the given patient population for which the medicines will be used.

d. Cross-cutting issues for all tools

EMA gave an overview of the anticipated communication activities related to regulatory tools for early access. The communication document on early access tools will be shared with the STAMP ahead of publication.

It was mentioned that information on early access tools should also be communicated to patients in lay language.

Any ideas from the STAMP members for effective communication about the use of PRIME and all other early authorisation tools are welcome.

4. Update on other EU initiatives relevant for timely patient access to innovative medicines

The Innovative Medicines Initiative (IMI), a public private partnership for Health, was presented. Within the on-going projects related to the timely access, ADAPT SMART is an action to coordinate and support activities for Medicines Adaptive Pathways to Patients (MAPPs) in IMI.

ACTION POINTS AND POINTS TO CONSIDER FOR THE NEXT MEETINGS:

- Following the EMA presentation on their communication strategy on their initiatives (PRIME, CHMP guidelines) – MSs are invited to submit their comments by 11 January 2016
- EMA questionnaire on the feasibility aspects of adaptive pathways - MS are invited to reply by 4 January 2016
- PRIME – EMA public consultation – MSs are invited to submit their comments
- Compassionate use
- Off-label use
- Personalised medicines
- National schemes for early access
- STAMP member to prepare a background note on repurposing of established medicines
- Current discussions on conditional marketing authorisation and adaptive pathways will continue as necessary.

The next meeting of the STAMP Expert Group is planned for **10 March 2016.**

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