EUROPEAN COMMISSION
HEALTH AND CONSUMERS DIRECTORATE-GENERAL

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

PHARM 671

PHARMACEUTICAL COMMITTEE
22 October 2014

Subject: **Relation between pharmaceuticals regulatory framework and timely access of patients to medicines: Reflection on difficulties and opportunities – Follow up from the 72nd meeting and next steps**

Agenda item 2a

The Commission initiated a reflection process with the Member States to discuss the link between the pharmaceuticals regulatory framework and timely access of patients to medicines.

Over the years flexibility has been built in the existing authorisation system in order to facilitate early access of patients to medicines with mechanisms such as the accelerated assessment procedure, conditional authorisations and authorisation under exceptional circumstances on the basis of less complete data, in order to address unmet medical needs. In addition the pharmaceutical legislation provides the possibility to make medicines available to patients before a marketing authorisation is granted, on grounds of compassionate use and treatment on a 'named-patient basis'.

Despite the measures already in place the issue of earlier access to innovative and affordable medicines for patients continues to be raised. In particular adaptive pathways to licensing of medicines are discussed and EMA initiated on 19 March 2014 a pilot project on adaptive licensing (AL). Adaptive licensing is defined as a prospectively planned process, starting with the early authorisation of a medicine in a restricted patient population, followed by iterative phases of evidence gathering and adaptations of the marketing authorisation to expand access to the medicine to broader patient populations[1]. As of September 2014, the Agency had received and assessed 26 applications as part of the pilot project, seven of which had been selected for a discussion with the applicant.

[1] EMA's communication on adaptive licensing including Q&As following the initial experience of the Adaptive Licensing Pilot project : http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000601.jsp&mid=WC0b01ac05807d58ce

The Agency will review the findings of the adaptive licensing pilot project at the end of 2014 and will then decide on the next steps.

In the last meeting of the Pharmaceutical Committee the Commission services asked the members of the Pharmaceutical Committee to give their views on the following points:

1. Analyse the perceived problem of early access of patients to innovative medicines and the reasons for it and to what extent they are related to marketing authorisation procedures or other policy areas.
2. Examine whether current approaches to marketing authorisation meet the objective to ensure timely access of patients to new medicines.
3. Study if there are ways to improve the situation within the current legal framework.
4. Analyse the perceived merits and weaknesses of an adaptive licensing approach from the regulatory/policy point of view, including the acceptable levels of uncertainty, possible change of paradigm and the consequences of shifting evidence gathering to the post-authorisation phase.
5. Examine how AL fits within the current legal framework and principles of legislation.
6. Consider whether any action would be useful or necessary.

The members of the Committee were also asked to inform of any studies carried out on this topic or actions taken at national level to ensure timely access of patients to medicines.

Thirteen Member States kindly responded to the request and provided very interesting feedback. A summary of the comments received per topic is provided as Annex to this note.

Actions to be taken:

- To be informed by EMA on the Adaptive licensing pilot project and progress so far.
- To discuss the feedback received from the Member States.
- To discuss next steps and the possibility of creating a group of experts on "Safe and Timely Access of Medicines to Patients" (STAMP) with the aim to:
    - identify and analyse issues related to the implementation of the EU Pharmaceutical legislation,
    - exchange views and information about the experience of Member States,
    - examine national initiatives and identify ways, including a more effective use of existing EU regulatory tools, to further improve safe and timely access and availability of medicines for patients.