EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Health systems, medical products and innovation
Medicines: policy, authorisation and monitoring

STAMP 8/38 corr
Summary record

STAMP Commission Expert Group
8 December 2017
8th meeting

Summary Record

The Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) held its 8th meeting on 8 December 2017, in Brussels, chaired by Unit B5 - *Medicines: policy, authorisation and monitoring* of Directorate General Health and Food Safety. Representatives from 22 Member States and the European Medicines Agency (EMA) participated in the meeting. Invited representatives of organisations or associations were present for selected agenda items (see attached list).

1. ADOPTION OF THE AGENDA

The draft agenda (STAMP 8/36) was adopted without changes[1].

2. APPROVAL OF PREVIOUS MINUTES

The record of the 7th STAMP meeting (STAMP 7/35) was approved without changes.

http://ec.europa.eu/health/files/committee/stamp/stamp_stamp_record_draft_published_en.pdf

[1] The agenda and copies of the presentations are available on the STAMP webpage: https://ec.europa.eu/health/documents/pharmaceutical-committee/stamp_en

3. EU ACTIVITIES RELEVANT TO TIMELY PATIENT ACCESS TO INNOVATIVE MEDICINES

a. Expert Panel on Effective Ways of Investing in Health - Opinion on Innovative payment models for high-cost innovative medicines

Professor Jan De Maeseneer, Chair of the European Commission Expert Panel on Effective Ways of Investing in Health (EXPH), presented the work of the Panel and the draft Opinion on Innovative payment models for high-cost innovative medicines[2]. There had been a public consultation on the draft report which had also been presented to an open meeting on 25 October 2017. The opinion was being finalised and was expected to be published early in 2018[3].

During the discussion the members of the STAMP asked about: the interchange of value and innovation; and, the modelling which had been used as background to the opinion. Professor De Maeseneer explained that the modelling for the draft opinion had been based on prices that would potentially decrease over time. Regarding innovation he made reference to the Expert Panel opinion on disruptive innovation[4]. The Chair thanked Professor De Maeseneer for the interesting presentation.

b. EURIPID - EURopean Integrated Price Information Database project

Dr Gergely Németh, EURIPID/NEAK National Institute of Health Insurance Fund Management, gave a presentation on the EURIPID database project. The database had been initiated in 2008 and in 2017 included information on reimbursed medicinal products and their list prices in 26 European countries. Dr Primožič, the STAMP member from Slovenia who is the Chair of the Board of Participants for the EURIPID project, explained that investigations were ongoing into the possibilities for creating synergies with other data collection systems. The Chair thanked Dr Gergely Németh and Dr Primožič for the interesting presentation and information about the project.

c. OECD access to medicines project

The Commission (Directorate General Health and Food Safety Unit B1) informed the STAMP about the recently agree project on Sustainable Access to Innovative Therapies which is part of the SANTE-OECD cooperation. As part of the project input regarding Member States experience would be sought and the Member States representatives might be contacted via the STAMP.

---

[2] https://ec.europa.eu/health/expert_panel/sites/expertpanel/files/019_innovative_payment_models_en.pdf

[3] Post meeting note – the final report was published on 9 February 2018: https://ec.europa.eu/health/expert_panel/sites/expertpanel/files/docsdir/opinion_innovative_medicines_en.pdf

[4] https://ec.europa.eu/health/expert_panel/sites/expertpanel/files/012_disruptive_innovation_en.pdf

d. Multistakeholder meeting on Pharmaceuticals

The Commission (Directorate General Health and Food Safety Unit B5) informed the STAMP on the update to the 12 September 2017 Multistakeholder meeting on Pharmaceuticals[5] where the STAMP activities that had relevance to the activities of pricing and reimbursement bodies had been presented. The stakeholders represented in the meeting had noted their continuing interest in the activities of STAMP.

The members of STAMP were asked to consider exchanging information and liaising with their colleagues who deal with pricing and reimbursement issues on topics discussed in the STAMP which are of relevance to them. Bringing their views into the STAMP would also be useful.

4. *AD HOC* SYNERGY GROUP

The *ad hoc* Synergy Group includes the STAMP members from Greece and Denmark as representatives of STAMP. The Synergy Group is mapping the current activities in the areas of potential action identified in the Health Technology Assessment Network refection paper of November 2016. The STAMP member from Greece gave a presentation on the activity of the Synergy Group and the input that had been provided about the STAMP activities.

A representative of the EMA joined the meeting via a teleconference link to present the EMA-EUnetHTA work plan 2017-2020 on behalf of EMA and EUnetHTA. The Synergy Group had been informed about relevant activities for the mapping exercise.

5. REPURPOSING OF ESTABLISHED MEDICINES/ACTIVE SUBSTANCES

During the 7th STAMP meeting on 27 June 2017 it was agreed that the small group led by the UK would analyse case studies of the experience of repurposing of established medicines and report back to the STAMP. The outcome of the analysis of the case studies submitted by the national competent authorities, EMA and a not-for-profit organisation were presented by the STAMP member from the United Kingdom. Members of the STAMP were invited to consider the summary of the cases and the highlighted discussion points.

It was explained that without a business case it is unlikely that a marketing authorisation holder would vary the marketing authorisation to include a new indication. The impact of off-label use was again mentioned. This can have an influence as it can discourage submission of an application either because it is already part of the clinical practice or when a new indication is included off-label use of other medicines with the same active substance can also occur.

The investment required for the inclusion of a new indication will depend on whether it is the result of following changes to medicines already on the market or whether the inclusion of a new indication is on the basis of new research and development activities. The economic cost of the changes will vary depending on the existing evidence, whether there is a need to generate data on the dose, administration route, efficacy and safety, and the required update of the product information. The role of the marketing authorisation

[5] http://ec.europa.eu/transparency/regexpert/index.cfm?do=groupDetail.groupDetailDoc&id=35559&no=2

holder is key for the inclusion of new indications for an active substance because of the need for the production and manufacturing of the medicine.

The following procedures were suggested as a means of updating marketing authorisations: variations requested by the marketing authorisation holder; scientific advice from a regulatory authority; a regulatory procedure to impose the inclusion of a new indication. The question of how to bridge from the available evidence to the inclusion of new indications without encouraging off-label use was mentioned. The question of liability for the product information update was again mentioned. It was noted that the obligations regarding pharmacovigilance related activities meant that new evidence on the safety or efficacy of a medicine should already be monitored and regularly reported on.

It was consider that the framework could be divided into two basic streams. When data is available to the marketing authorisation holder, they could use the normal regulatory procedures to have the new indication assessed. If the data was not available, further research would be required. For the data generation aspects, the regulatory authorities can give guidance.

The need for case-by-case assessment was stressed by some participants. They identified the following factors as influencing the process: the assessment process with the potential need to submit additional data; the safety of the product in the new indications; liability; and, the distribution.

It was suggested by some participants that the creation of a consortium of interested parties (e.g. industry, academia, not-for-profit organisations) could be a way to overcome some of the barriers to repurposing of medicines.

The issue of access to the relevant data and whether there is a need for a handbook or training to support such activities was discussed. It was noted that the EC's Research and Innovation DG (DG RTD) had launched a call for a consortium with the aim to strengthen regulatory knowledge, particularly for academia. It was mentioned that the focus of academia was the publication of the results of research. Also the evidentiary standards was mentioned, in particular which data sources could be used. With respect to the use of data from registries the activities in other groups, for example the Heads of Medicines Agencies, was noted.

It was mentioned that possible incentives, such as the transferable voucher scheme operated in the United States, might not work in the EU regulatory landscape.

The representatives of the industry organisations offered to develop a paper outlining how industry can engage in repurposing of medicines activities and what a repurposing framework might look like. This was welcomed by the STAMP. The issue of repurposing would be discussed in the next STAMP meeting with a view to summarising the output of the STAMP discussions.

6. HEADS OF MEDICINES AGENCIES SUBGROUP ON TIMELY ACCESS

The STAMP member from Spain gave a presentation on the activities of the Heads of Medicines Agencies' Subgroup on Timely Access. It was suggested that publication of the information about the Subgroup's activities related to early access could be a useful way to raise awareness of action in Member States. It was asked that the challenges of

early access be considered, such as the truly innovative products, the balance of knowledge on the efficacy and safety of a medicinal product and early access, the cost of reimbursement and the impact on the healthcare system. It was acknowledged that there is a need to find solutions to the challenges related to early access to medicines. One member explained that when medicines have been authorised there is a demand from patient representative organisations and patients to have access to the product but the cost of reimbursement can be a challenge for the healthcare system. It was noted that patient representatives are involved in the scientific discussions during the assessment of medical products. Some members indicated that in their experience it was useful to have the patient perspective. There are opportunities through EU or national initiatives for training of patient representatives so that they are informed on research methodology, interpretation of the evidence and the regulatory framework. One member indicated that increased interaction between the different players involved in bring a medicine to the market and patient access is important for the understanding of product development and assessment and potential impact on the healthcare system. If resources allowed, the HMA Subgroup was asked to give feedback on the Member States' experience of early access to medicines in the past years.

ACTION POINTS AND POINTS TO CONSIDER FOR THE NEXT MEETINGS:

- Industry representatives to prepare a document on how industry can engage in repurposing activities and what a repurposing framework might look like.

The next meeting of the STAMP Expert Group is planned for **8 June 2018 (tbc).**

*****

8 DECEMBER 2017 STAMP EXPERT GROUP - EXTERNAL PARTICIPANTS

Name	Affiliation	Agenda items
Jan De Maeseneer	Ghent University	3 a
Gergely Németh	EURIPID/NEAK National Institute of Health Insurance Fund Management	3 b
Lydie Meheus	Anticancer Fund	5
Ciska Verbannderd	Anticancer Fund	5
Kristine Peers	EFPIA - European Federation of Pharmaceutical Industries and Associations	5
Sheuli Porkess	EFPIA	5
Beata Stepniewska	Medicines for Europe	5
Catarina Pereira	Medicines for Europe	5
Olga Kozhaeva	SIOPE – the European Society for Paediatric Oncology	5