



STAMP Commission Expert Group
15 March 2019
11th meeting

Summary Record

The Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) held its 11th meeting on 15 March 2019, in Brussels, chaired by Unit B5 - Medicines: policy, authorisation and monitoring of Directorate-General for Health and Food Safety. Representatives from 21 Member States and the European Medicines Agency (EMA) participated in the meeting. Invited representatives of organisations or associations were present for agenda items 1 - 4 (see attached list).

1. ADOPTION OF THE AGENDA

The draft agenda (STAMP 11/46) was adopted with the change that the presentations by the Organisation for Economic Co-operation and Development (item 4) would be taken before item 3¹.

2. APPROVAL OF PREVIOUS MINUTES

The record of the 10th STAMP meeting (STAMP 10/45) was approved without changes.

https://ec.europa.eu/health/sites/health/files/files/committee/stamp/stamp_10_final_record_en.pdf

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

3. REPURPOSING OF ESTABLISHED MEDICINES/ACTIVE SUBSTANCES

The Commission introduced the item referring to the documents linked to STAMP 11/47. During the 10th meeting in December 2018 STAMP had agreed that the proposal for a framework for repurposing of medicines should continue to be developed through the working group. The Chair thanked the working group for their preparation of the documents, noting that the model of working on the topic of repurposing, including involvement of invited stakeholders, had been beneficial as a means to engage interested parties and develop a framework that could be piloted.

The STAMP 11/47 background documents had been prepared by the working group. ‘STAMP 11/47’ introduced the topic and included questions. ‘STAMP 11/47 repurposing proposal framework’ outlined the proposal for a repurposing framework, the suggested pilot project, the suggestion for a group to oversee the pilot, as well as an annex of possible resources. ‘STAMP 11/47 candidate molecules’ included ideas for candidate molecules collated by the Anticancer Fund. ‘STAMP 11/47 STARS overview’ included details of planned activities of the ‘EU Coordination and Support Action – STARS: Strengthening training of academia in regulatory sciences and supporting regulatory scientific advice’ that could be relevant to repurposing activities.

Repurposing framework

There was a presentation on the first part of ‘STAMP 11/47 repurposing proposal framework’ (pages 1-7). During the discussion the following points were raised.

A question was raised about specifying ‘not-for-profit’ in the title. It was concluded that ‘not-for-profit organisations’ specifically in the title could help raise awareness for the type of organisations that could be Champions. With respect to scope of ‘not-for-profit’ it was clarified that academia can be considered within the scope and that a Champion can be a group of partners that collaborates on a specific project.

It was noted that it would be a voluntary framework involving scientific and regulatory advice based on existing mechanisms and procedures. The purpose of the framework was to cover medicines or active substances where there could be more than one marketing authorisation holder. It was mentioned that data and market protection could be difficult to determine and that these are important considerations at the time of submission of an application of the new indication.

Repurposing could be linked to other initiatives/existing structures such as the PRIME (Priority Medicines) scheme, orphan designation. It was suggested to explore whether the Innovation Network could be a possible entry point for Champions prior to scientific advice. The importance of coordination between the regulatory authorities for requests for regulatory or scientific advice would be important. It was mentioned that not all Member States have regulatory incentives (e.g. fee waiver) that could support repurposing process.

The industry representatives explained that the Section 3.3. on industry engagement was intended as a guide on what additional issues may be considered by the companies prior to taking a decision to submit an application for a new indication of a medicine. It was agreed to review the wording to support the engagement of companies with Champions. The ultimate goal of the framework is to bring an indication supported by relevant

evidence on-label by a company, in particular in cases where no direct commercial interest exists.

The possibility that Champions could come back to regulators if companies are not interested in pursuing the new indication or if the evidence does not support the indication was identified as a gap in the paper. This would be considered during the pilot phase.

It was noted that the aim of the framework is to encourage and support not-for-profit organisations, including academic organisations and research groups, to be made aware of the regulatory process and the possibility to receive scientific advice.

The Commission explained that the draft document should be seen as a basic document, jointly developed with the Member States and other participants, it is not a legal document. The overall aim is that it would form the basis to start a pilot. The document will be reviewed during the experience of repurposing activities and after running the pilot(s).

Pilot project(s)

The discussion continued on the second part of the document (pages 8 - 12) where the main point of discussion was the proposal for an oversight group for the pilot project (described as the 'Repurposing Monitoring Board'). The working group had suggested that an oversight group for the pilot project (drawn from the STAMP participants) should be established to give guidance on the framework, monitor the pilot project and report back to the STAMP.

The main points of discussion was the role of the board, its interaction with the Champions and whether there could be a conflict of interest between the representatives on the board and national advisory or regulatory procedures.

It was concluded that the board's main role should be monitoring of the progress of the pilot, identifying if there are aspects of the framework which should be adapted, and reporting on the pilot project overall. It could have two 'levels' with different participants: one to monitor the pilot and report back on the overall experience of the pilot and the other to provide guidance to the participants in the pilot on the practical application of the framework. Any interaction that could create a conflict of interest between representatives on the board and the pilot projects would be avoided. The board would have a guiding and coordinating role but not a decision-making role with respect to the submissions to the regulatory authorities. It should monitor the pilot and report on the experience of the Champions seeking scientific and/or regulatory advice through national or centralised procedures and other parties involved in the process.

In the short-term the objectives of the pilot would be to include candidate molecules, possible Champions, and the process of the provision of scientific and/or regulatory advice. In the long-term the 'uptake' of a repurposing candidate and the submission of a marketing authorisation application could be monitored.

Spain volunteered to lead the board which would be a small group with representatives drawn from the existing working group.

Candidate molecules

The Anticancer Fund presented their analysis of the potential candidates for pilot(s) by different characteristics, namely: entry at an early stage of the development versus late stage entry; ‘hard’ repurposing (from one disease area to another) versus ‘soft’ repurposing (within the same disease area); potentially nationally authorised products versus centrally authorised; combination versus monotherapy products. A list of candidate molecules which they had identified was presented.

In addition to the molecules which the Anticancer Fund may decide to follow up, the candidate molecules for the pilot project could come through the applications for the scientific advice. Also the participants in the STAMP could identify potential candidates through their networks. It is expected that the number of molecules included in the pilot would be limited to so that the monitoring of the pilot would be manageable. The aim should be to have one or two candidate molecules that can be followed in the pilot, such as a monotherapy with a good evidence base and, if possible, a candidate in the ‘early’ development phase. It was noted that it would be good to have the results in a short time frame to allow the assessment of the pilot as soon as possible.

In conclusion, it was agreed that the document provided a starting point for consideration of a framework which could be developed in the light of the pilot and the input of the stakeholders during the process. The working group was reconvened to take account of the comments of the STAMP to finalise the framework, the testing through a pilot and the mandate for the oversight group.

4. PRESENTATIONS BY ORGANISATION FOR ECONOMIC CO-OPERATION AND DEVELOPMENT OF REPORTS RELEVANT TO PHARMACEUTICALS

Representatives from the Organisation for Economic Co-operation and Development (OECD) gave presentations on the following reports: ‘Using routinely collected data to inform pharmaceutical policies’ and ‘Pharmaceutical innovation and access to medicines’.

The report ‘Using routinely collected data to inform pharmaceutical policies’ had been published in February 2019 as an analytical report with accompanying country notes². The European Commission contributed to the funding of the report. The OECD had explored countries' routine collection of data on prescribed and dispensed medicines to identify best practices, and to assess the potential impact on health and pharmaceutical policies. The countries surveyed considered that the routinely collected data could be used more but there were certain barriers to their use. The report includes recommendations on how to improve the use of routinely collected data. In reply to a question whether the European Reference Network (ERN) are covered in the report, it was explained that the focus was on country practices. The EU level projects on health technology assessment had been considered but not the ERN.

The report on ‘Pharmaceutical innovation and access to medicines’ had been published in November 2018³. The work had been initiated by France and was then taken forward

² <http://www.oecd.org/els/health-systems/routinely-collected-data-to-inform-pharmaceutical-policies.htm>

³ <http://www.oecd.org/health/pharmaceutical-innovation-and-access-to-medicines-9789264307391-en.htm>

through OECD. The presentation highlighted the main challenges that governments are facing in appropriate access to innovative medicines to all those in need. The report considered the impact of prices on access to medicines. Drawing from the available evidence, the report includes policy options, outlining their expected benefits and potential costs.

In the discussion the OECD noted that for orphan medicines there no easy solution had been identified to find a balance between the cost of research and development and the price charged for the medicines. One participant highlighted the problem of smaller countries to have access to medicines, in many cases the medicines are not launched on their markets and there was not transparency on price paid in the countries where the medicine has been launched. The OECD agreed that it was difficult to have information about the price paid as compared to the list price as well as when the medicine is place on individual markets. One participant mentioned that in cases where there is only one supplier of a medicine there could be risk of supply. There was a question about the transparency about the evidence for a marketing authorisation and whether this could be improved. EMA explained that pre-authorisation evidence is treated in confidence but following the granting of an authorisation and changes in the authorisation, the evidence which forms the basis of the authorisation is made available.

The Chair thanked the colleagues from OECD for their interesting presentations and further explanations about their reports.

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

There were no specific points raised under the agenda item.

6. FUTURE ACTIVITIES OF STAMP

The Chair noted that the STAMP was an expert group of the Pharmaceutical Committee and has worked on topics agreed by the committee. The Chair explained that in future the focus of the agenda of the Pharmaceutical Committee would be on policy issues and it would be structured to allow time for detailed discussions in the meetings. This would lead to agendas with a limited number of discussion items instead of more extensive agendas which include updates or items for information. The role of STAMP might develop along side the Pharmaceutical Committee, with individual topics being referred from the committee for expert discussion in the group.

Members noted that the value of STAMP is that the participants can be close to the issues under discussion and can give their input based on practical experience. It was mentioned that there are various groups and different configurations in the area of pharmaceuticals and the benefit of the STAMP had been bring together the representatives for the different activities in the lifecycle of a medicine together to discuss specific issues.

Members identified the following topics that might be relevant to the Pharmaceutical Committee or STAMP: follow up of studies on orphan and paediatric medicines and incentives; the use of data collected by wearable monitors; the combination of and borderline between medicines and medical devices

ACTION POINTS AND POINTS TO CONSIDER FOR THE NEXT MEETINGS:

- Working group participants and STAMP members to indicate if they wish to participate in the oversight group.
- The working group to update the proposed repurposing framework.

The next STAMP meeting is planned for 17 December 2019 (tbc).

15 MARCH 2019 - STAMP EXPERT GROUP - EXTERNAL ORGANISATIONS PRESENT

Organisation represented	Agenda items
Anticancer Fund	1 - 4
Belgian Healthcare Knowledge Centre (KCE)	1 - 4
European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)	1 - 4
European Federation of Pharmaceutical Industries and Associations (EFPIA)	1 - 4
European Patients' Forum (EPF)	1 - 4
European Social Insurance Platform (ESIP)	1 - 4
EURORDIS - Plateforme Maladies Rares	1 - 4
International Association of Mutual Benefit Societies (AIM)	1 - 4
Medicines for Europe (MfE)	1 - 4
Pharmaceutical Group of the European Union (PGEU)	1 - 4
Standing Committee of European Doctors (CPME)	1 - 4
Organisation for Economic Co-operation and Development (OECD)	4 (via video link)