



PHARMACEUTICAL COMMITTEE
11 July 2019

Subject: Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) activities – Proposal for a framework for the repurposing of established medicines¹

Agenda item 4

1. Process for the development of the proposed framework

The issue of repurposing of established medicines has been a major point of discussion in the Safe and Timely Access to Medicines for Patients (STAMP) Expert Group since March 2016².

In 2017 there was a brainstorming session with invited representatives of industry, patient, healthcare professionals, health technology assessment bodies, payers and not-for-profit organisations which has been followed up and has led to the development of a proposal for a framework for the repurposing of established medicines. The work on proposed framework was finalised through a working group of the STAMP which included representatives from Member States and stakeholders who had participated in the brainstorming session. The following Member States and stakeholder groups were represented - Belgium, the Netherlands, Norway, Spain, Sweden, the United Kingdom, European Medicines Agency, Anticancer Fund, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), European Federation of Pharmaceutical Industries and Associations (EFPIA), European Organisation for Rare Diseases (EURORDIS), European Patients' Forum (EPF), European Society of Paediatric Oncology (SIOPE), International Association of Mutual Benefit Societies (AIM). The group worked through exchange of emails and regular teleconferences.

¹ This document has not been adopted by the European Commission and, therefore, it does not reflect an official position of the European Commission.

² Repurposing of established medicines/known active substances has been on the agenda of the 4th to 10th meetings of STAMP. The documents related to STAMP are available on the following webpage: https://ec.europa.eu/health/documents/pharmaceutical-committee/stamp_en.

Following the March 2019 STAMP meeting the working group completed the document entitled *Proposal for a framework to support not-for-profit organisations and academia (institutions and individuals) in drug repurposing* which is presented to the Committee for its endorsement.

2. Proposed framework for the repurposing of established medicines

The document *Proposal for a framework to support not-for-profit organisations and academia (institutions and individuals) in drug repurposing* is divided into 3 main sections: the proposal for a repurposing framework; a suggested pilot of the STAMP repurposing framework; the role of the 'Repurposing Observatory Group' during the pilot phase. There is also an annex listing useful resources.

The aim is to provide a visible supportive framework to a not-for-profit organisations and academia (described as Champions), who have evidence and scientific rationale for a new indication. The basis of the framework is within the existing procedures such as scientific and regulatory advice. The document outlines the criteria to be fulfilled by the organisations, the key features of the framework and core components of the targeted repurposing projects. The potential engagement with regulatory authorities for scientific or regulatory advice as well as industry engagement are outlined.

The summary of the proposed framework is:

- A Champion can be a person/entity from a charity or patient group/academic unit/learned society/research fund/payer, with a particular interest in repurposing an authorised medicinal product for a new indication and who has data evidence/scientific rationale to do so.
- A Champion puts forward sufficient supporting data for a new indication to an unprotected off-patent medicinal product to be discussed in a repurposing regulatory scientific advice meeting.
- The repurposing regulatory scientific advice provides comments and feedback on the presented data package components, and the requirements of any future data generation (if required).
- On the basis of the advice, the Champion conducts further development and/or consolidation of the available data.
- The Champion seeks an immediate or future partnership with marketing authorisation holder(s) (MAH) depending on the stage of the development.
- For the purpose of filing the data to support a new indication, the Champion / MAH confirms that the available data are in compliance with the advice given by the regulatory authority (or is expected to provide justification for any deviation).
- The MAH/applicant seek an extension or variation or a marketing authorisation using the existing regulatory pathways if the data package is considered sufficient.

It is suggested that a pilot should be conducted to test the proposed framework, learn from the practical applications of candidates within the framework and build on the concepts identified. The objectives and expected deliverables of the pilot are outlined in the document.

In order to learn from the process and conclude on the operational aspects of the repurposing framework, the STAMP working group considered that it would be important to create a voluntary virtual observatory group (called Repurposing Observatory Group). This group, composed of Champion interest groups, industry and

regulatory representatives (and other stakeholders as appropriate), would report to STAMP and/or the Pharmaceutical Committee. The document outlines the expected way of working of the group and its main activities. Spain volunteered to lead the group.

The Repurposing Observatory Group would not be involved in selecting Champions or medicines for the pilot nor any individual decision making role for the individual pilot projects. The specific decisions and support would be provided through existing mechanisms of the regulatory authorities running the pilot projects.

3. Next steps

- The Committee is invited to endorse the document *Proposal for a framework to support not-for-profit organisations and academia (institutions and individuals) in drug repurposing*, and the associated pilot of the proposed framework with oversight from the Repurposing Observatory Group led by Spain.
- The Repurposing Observatory Group will conclude on the practical aspects of the implementation of the pilot and report on the challenges, successes and opportunities of the framework.
- The Repurposing Observatory Group will report back to Pharmaceutical Committee and/or STAMP and suggest revision of the framework as appropriate.

Action to be taken:

Endorsement of the proposed framework and next steps.