



**STAMP Commission Expert Group
13 March 2017**

Repurposing of established medicines/active substances

Background: The STAMP expert group discussed the issue of repurposing of established medicines/active substances on the 10th March and 28th June 2016¹.

The discussion in STAMP noted the following issues:

- Potential incentives and disincentives
- Drug development- evidence supporting repurposing
- Involvement of academia
- Marketing authorisation
- Summary of feedback from Member States captured from specific questionnaires
- Off-label use

The STAMP discussions mainly focused on drug repurposing for new indications for well established (off-patent) medicines in areas of unmet medical need that could offer additional therapeutic options to patients.

It was considered helpful to broaden the discussion to involve stakeholders who have relevant experience of repurposing established medicines/active substances. An *ad hoc* meeting of stakeholders including representatives from research organisations, patient organisations, industry and some representatives of downstream decision making bodies has been arranged to help extend the discussion, and benefit from attendees experience to help identify opportunities and barriers to repurposing of established medicines.

The aim is to have an open discussion to explore the issues and have a brainstorming around possible options and solutions to support the introduction of:

1. New indications for Off-patent medicines in new marketing authorisations
2. Extension of indications for existing marketing authorisations (variation applications).

We would like an open exchange of views without commitments. We hope that innovative solutions will emerge for further consideration.

¹ Background documents and the notes of the meetings are available via the following webpage:
http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp_en

Repurposing is a broad subject and it would be difficult to cover all issues so the focus of the discussion would be around the process of inclusion of new indications for established medicines/active substances.

SUGGESTED DISCUSSION POINTS TO INITIATE REFLECTION (OTHERS WELCOME):

Are there significant regulatory barriers for new marketing authorisation holders to obtain a marketing authorisation for a new indication for a known active substance?

What has been the experience of researchers/companies seeking a marketing authorisation for a new indication for a known active substance as a new marketing authorisation?

Are researchers/companies aware of the regulatory requirements?

Are researchers/companies aware of regulatory incentives and support e.g. orphan designation, paediatric-use marketing authorisation (PUMA), scientific advice, small and SME office?

Was support available for preparing a marketing authorisation application? Was it used? And which sources of support did you find most useful?

Do you consider that other regulatory support would be beneficial to facilitate the application process?

Were applications for marketing authorisations successful? If not, why not?

Are there opportunities for including new indications in the marketing authorisation for existing approved medicinal products?

What is the experience of researchers/companies seeking to include new indications in the marketing authorisation of an established medicine?

Are researchers/companies aware of regulatory incentives and support available e.g. additional one year of data exclusivity as per Paragraph 5 of Article 10 of Directive 2001/83/EC?

Are the current level of data requirements considered appropriate for applications for a new indication for an existing approved medicine?

Have marketing authorisation holders extended their marketing authorisation based on evidence generated by third parties?

If not, would evidence from third parties be considered an option for extending the indication of a medicine?

What are the barriers to using evidence generated by third parties?

Are there significant barriers for making such marketing authorisations with a new indication for a known active substance (either new marketing authorisations or extensions) accessible for patients?

What has been the experience of marketing authorisation holders in assessments by down-stream decision makers with regard to added value compared to other technologies?

Are there any experiences concerning challenges in national pricing and reimbursement discussions, e.g. reference pricing, that impact translation of added value into economic value?

Was the clinical evidence generated for regulatory decision making adequate for subsequent decision making? Have there been opportunities to discuss and agree such evidence generation plans for repurposing across all relevant decision makers?

What is the experience for the healthcare professionals /patients regarding repurposing of medicines?

Other points for discussion. What are your ideas? Let's think out of the box!

In terms of priority setting and data generation for repurposing, are there possibilities for public/private partnerships?

Are there suggestions for alternative or augmented regulatory aspects / procedures that could impact on repurposing activities?

What type of incentives could facilitate development and authorisation of new therapeutic indications for a known active substance?

What are opportunities and challenges in bringing different decision makers together for discussion of concrete products for repurposing, e.g. to guide on evidence plans or to facilitate added value discussions?