Medicines & Healthcare products Regulatory Agency

MHRA
Regulating Medicines and Medical Devices

Safe and Timely Access to Medicines for Patients

Summary of responses to questionnaire

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Repurposing medicines

- Drug repurposing is the process of identifying a new use for an existing drug in an indication outside the scope of the original indication

- Repurposing includes:
  - **New therapeutic uses for already known drugs**
  - Developing different formulations for the same drug
  - Creating new combinations of drugs previously used as separate products
  - Creating new combinations of drugs with medical devices

- After discussion of the STAMP meeting item on repurposing of established medicines, the Group considered that off-label use of medicines for patient access should be explored further and agreed that a questionnaire should be circulated to seek more information on 'important' authorised medicines widely used off-label

  - Five questions were posed in the questionnaire to the Member States

STAMP Member State questionnaire

- Comments were received 19 Member States (MS)
- Question 1: Do you consider that there are significant regulatory barriers for including new indications in the MA for existing approved medicinal products?
- Question 2: Do you have any specific national provision(s) for supporting off-label use of existing medicines?
- Question 3: Please list the important drugs that your national provision currently or has recently supported
- Question 4: Do you any national incentives for supporting registration of ‘off-label’ indications for existing medicines in marketing authorisations?
- Question 5: Do you have any other comments regarding off-label use and/ or re-purposing of medicines?

STAMP Member State questionnaire

Question 1: Do you consider that there are significant regulatory barriers for including new indications in the marketing authorisation for existing approved medicinal products?

- The majority of MS who responded did not consider that there are significant regulatory barriers to including new indications in existing MA
  - 6 MS considered that there were significant regulatory barriers whilst 13 did not.

- One common theme expressed by several MS was that a new indication could only be sought by the MAH, and that the clinical trial requirements for new indications could be considered to be burdensome

- Comments from 'yes' respondents include that 'there is a need to discuss the option/feasibility of alternative designs for clinical trials, alternative end points and for the increased use of real world data/information from patient registries‘

- From a regulatory point of view, requirements are sometimes far beyond of what would be reasonable, especially with medicines already being used off-label for a long time’

STAMP Member State questionnaire

Question 2: Do you have any specific national provision(s) for supporting off-label use of existing medicines?

- 11 MS have specific national provision(s) for supporting off-label use of existing medicines and 7 MS do not
- These provision(s) have a variety of structures and frameworks, with some very well established mechanisms of supporting off-label use in some MS
- One MS has introduced a new policy, the temporary recommendations for use (RTU), others put the responsibility in a internal committee at each hospital
- In at least one MS, the Agency can establish therapeutic protocols and/or recommendations for the use (or not use) of medicines for conditions
- One MS was exploring how collaboration can be fostered between generics manufacturers & academics with a view to repurposing new indications for off-label

STAMP Member State questionnaire

Question 3: Please list the important drugs that your national provision currently or has recently supported. For each drug, please include the active substance, name, indication, framework of assessing benefit: risk and patient access, and reimbursement availability.

- MS presented a variety of drugs and frameworks where available

- Frameworks included a searchable database for the National formulary reference to the use of medicines in some non-approved indications (off-label) and recommendations given by the off-label expert commissions

STAMP Member State questionnaire

Question 4: Do you have any national incentives for supporting registration of 'off-label' indications for existing medicines in marketing authorisations?

- Only 3 MS have national incentives for supporting registration of 'off-label' indications for existing medicines with 12 MS answering no

- One MS has a specific drug rediscovery policy.

STAMP Member State questionnaire

Question 5: Do you have any other comments regarding off-label use and/ or re-purposing of medicines?

- Additional comments included concerns that any waiver of regulatory requirements might increase the risks

- For smaller MS, a central registry for doctors/health care professionals to log in and compile data regarding their off-label use of medicines might be useful

- Suggestions also included that better use of referral procedure, that might be a means of harmonization of information (Efficacy and Safety) for off patent medicinal products.

Thank You

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