Off-label use of medicinal products in the EU

STAMP Expert Group
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Health and Food Safety

Study on off-label use of medicinal products in the European Union

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OUTLINE

- Why the study on off-label use, and what for?
- What are the main findings of the study?
- Focus on the next steps

Rationale of the study

- Need to understand the range of different issues related to the off-label use of medicines in the EU
  - ✓ **Variety of national frameworks** (regulatory or not) and changes of some national legislation (e.g. France, Italy)
  - ✓ **Drivers** and **prevalence** of off-label use in different therapeutic areas
- **Patient safety** (call from the EP in Resolutions of 2013 and 2015, stakeholders' positions)

Scope of the study

- Scientific: **patient safety**

- **Legal:** regulatory framework for the off-label use of medicinal products in the EU

Main objectives of the study

- ✓ **Drivers** of off-label use
- ✓ Comprehensive information on **national frameworks** (legislative or recommendations/guidelines)
- ✓ Existing and foreseen **practices** of off-label use across Member States **to ensure patient safety**
- ✓ Factual analysis of **stakeholders',positions** on existing measures and possible tools related to the off-label use of medicines

From consultation to publication

- 28 June 2016**: STAMP members** comments on draft study report followed by written comments
- May and September 2016: **EMACOLEX** consulation on legal part (national frameworks and case-law)
- 28/02/2017: publication of final study report

Main findings of the study (1)

- Physician-centred approach towards off-label use
- Drivers and prevalence of off-label use
- Pros and cons of off-label use
- National frameworks
- Identified options to strengthen patients' safety

Findings (2)- Physician-centred approach

- Off-label use is not directly regulated in EU pharmaceutical law:
  - EU provisions regulate the **placing on the market** of products and **NOT the way the products <u>are ultimately used in medical practice</u>**
  - **The** marketing authorisation defines the approved indications and **any departure from the terms of authorisation will remain** the **responsibility of the prescribing physician.**
- Limited EU competences in field of public health (Article 168 (7) of TFEU)

Findings (3) – Main drivers

- **Absence of an authorisation for the therapeutic indication**
- **Lack of treatment in case of shortages or withdrawals from the market of authorised products**
- **Better patient adherence to the off-label treatment**
- **Economic considerations such as cost containment measures/control of pharmaceuticals expenditures.**

Findings (4) - Pros and cons

- **Clear distinction between 2 following scenarios:**

1. **No other treatment options : agreement amongst all stakeholders that off-label use can be beneficial to patients (industry, HCP, patients)**

2. **Alternative authorised: strong disagreement among stakeholders despite same objective of patient safety**

- **Liability in case of health problems related to the off-label use of medicinal products : concern for many stakeholders, including health care professionals and marketing authorisation holders.**

Findings (5) - National frameworks

- 21 participating Member States (MS)
- 10 MS have measures in place (4 with legal frameworks)/ 11 MS without any policy tools (issue to be dealt with at level of prescribers)
- Off-label use addressed in a variety of ways: legislation or soft law (recommendations, guidelines…), authorising expressly the off-label use or recognising tacitly the off-label use, providing expressly or not the reimbursement of medicinal products used off-label, or by not acting (considering that the issue is exclusively the prescribers' competence).

Proposed options for discussion

- Need for **treatment guidelines at EU level** – common ground for national treatment guidelines in individual MS
- Moving **from "off" to "on" label**: repurposing, incentives to register new indications for existing medicinal products.
- Explore **possibilities for action at national level** on off-label prescription/use and reimbursement measures (taking into account stakeholders' various positions, the impact of such measures, recent and ongoing court cases, etc.)
- Development of **treatment** guidelines by professional bodies; **awareness campaigns** for patients and health care professionals.

European Commission

Thank you for your attention !