Off-label use of medicinal products in the EU

STAMP Expert Group
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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 consultation 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 are ultimately used in medical practice**
  - **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.
Thank you for your attention!