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

Summary

Background

The European Commission (EC), the European Medicines Agency (EMA) and the 28 Member States are working closely together to assure that all medicinal products for humans that are introduced to the European market meet the EU standards on quality, safety and efficacy. For medicinal products to become available on the market, the first step is getting a marketing authorisation. During the marketing authorisation procedure, the EMA or the national competent authorities of the Member states, establish the terms under which the product can be used safely and effectively. These terms are described in the Summary of Product Characteristics (SmPC) and are the basis of information for healthcare professionals on how to use the medicine. Off-label use refers to any intentional use of an authorised product not covered by the terms of its marketing authorisation and therewith not in accordance with the SmPC. This may for example concern prescribing the medicinal product for a different indication or to another target patient group as laid down in the SmPC. Off-label prescribing has cons: there is no evidence-based practice and there is lack of knowledge of its effects. Yet, off-label prescribing also has pros as it sometimes represents a medical need that is not fulfilled by pharmaceutical companies. Given this, it is not surprising that the off-label use of medicinal products is leading to an increasing number of questions by Member States and stakeholders towards the Commission.

General objective

The general objective of this study is to provide the European Commission with a clear description of existing and foreseen practices of off-label use across Member States and a factual analysis of all parties' positions towards the existing measures and possible envisaged tools to regulate the off-label medicine use.

This will be provided through the following specific objectives:

1. Providing information on the variety of situations in EU Member States in relation to off-label use of medicinal products, as to how authorities have addressed the issue and different ways stakeholders (patients, health care professionals and industry) react depending on the situation;
2. Providing information on the drivers for off-label medicine use, to be divided in therapy related drivers, patient related drivers, professional related drivers and health care system related drivers;
3. Providing a factual analysis of the outcomes of WP 1 by analysing the off-label use and practices in the EU Member States against the EU legal framework (for example by analysing court cases) to identify particular aspects and/or therapeutic areas of off-label use that would deserve specific attention at EU level.