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Health systems, medical products and innovation
Medicines: policy, authorisation and monitoring

STAMP 7/34

STAMP Commission Expert Group
27 June 2017

Subject: Compassionate use programmes

Compassionate use programmes (CUPs) and early access programmes were discussed during the 4th STAMP meeting. In that meeting the legislative framework for compassionate use was outlined [1]. The discussion highlighted the diversity in approach at Member State level to compassionate use, named patient or other means of early access to medicines and the understanding of which uses should be notified to the European medicines Agency (EMA).

The conclusion of the previous STAMP discussion was that there is a need to clarify the application of compassionate use for products in development and the notification of such schemes to the EMA. It was proposed that there should be further consideration whether there is need for more information on the compassionate use framework at EU level and how to facilitate requests for CHMP opinions on compassionate use.

In April 2016 the Heads of Medicines Agencies (HMA) published a list of the national competent authorities that publish guidance on their compassionate use programmes. The list includes links to the compassionate use guidance in 17 Member States[2]. Of the Member States included in the list, 2 indicated that they do not have a CUP as described in Article 83 of Regulation (EC) No 726/2004. The HMA Subgroup on timely access intends to prepare an overview of the situation regarding compassionate use programmes in the Member States.

More recently, in November 2016, an overview of compassionate use programmes in the EU Member States has been published. This is based on a literature review in which the authors identified that compassionate use programmes were in place in 20 Member States [3].

1 STAMP 4_22 (https://ec.europa.eu/health/sites/health/files/files/committee/stamp/2016-03_stamp4/6_compassionate_use_background_paper.pdf)
2 http://www.hma.eu/fileadmin/dateien/HMA_joint/02-_HMA_Strategy_Annual_Reports/08_HMA_Publications/2016_05_HMA_Compassionate_use_program.pdf
3 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5116859/

Following the previous discussion in STAMP in the next meeting we would like to have a better understanding of the experience of Member States of compassionate use programmes that are subject to Regulation (EC) No 726/2004 as mentioned in Article 83 of the Regulation, details of which are given below. We are aware that there are also early access schemes under Article 5 of Directive 2001/83/EC.

> **Article 83 of Regulation (EC) No 726/2004** provides the possibility for a Member State to make a medicinal product for human use belonging to the categories referred to in Article 3 paragraphs 1 and 2 of Regulation (EC) No 726/2004 available for compassionate use to a group of patients and for the Committee for Medicinal Products for Human Use (CHMP) to adopt opinions on the conditions for use, conditions for distribution and the patients targeted.
>
> Article 83 is applicable when:
>
> - The medicinal product belonging to the categories referred to in Article 3 paragraphs 1 and 2 of Regulation (EC) No 726/2004 is to be made available to "a group of patients with a chronically or seriously debilitating disease, or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily by an authorised medicinal product"
> - The compassionate use programme is intended for a "group of patients",
> - The medicinal product is either "the subject of an application for a centralised marketing authorisation in accordance with Article 6 of Regulation (EC) No 726/2004 or is undergoing clinical trials".
>
> If a Member State makes use of the possibility for compassionate use for product subject to the Regulation (EC) No 726/2004, it is requested to notify the European Medicines Agency (EMA).
>
> Member States may ask the Committee for Medicinal Products for Human Use (CHMP) to provide an opinion on the conditions for use, conditions for distribution, as well as the target patient population.

The following questions are intended to facilitate the further discussion in the meeting on the following:

1) A reflection about whether CUPs can benefit more patients in the EU through knowledge and data sharing, benchmarking etc.
2) How CUPs are currently operating and whether it would be useful to make adjustment to the mechanism of informing the EMA and of seeking a CHMP opinion foreseen under Article 83 of Regulation (EC) No 726/2004.

It will be considered after the discussion whether it would be helpful to have written replies to a questionnaire.

List of questions to frame the discussion on 27 June 2017 – no need to submit in writing prior to the meeting

Q 1: Does your Member State have a regulatory framework to authorise use of medicinal products for cohort (group) of patients compassionate use programmes (CUP)?

Q 2: Are there alternative early access programmes in your Member State?

Q 3: Do you Member State CUPs cover only medicinal products which would be subject to a central authorisation?

Q 4: Are there any regulatory constraints implementing CUP?

Q 5: Do you consider that CUPs facilitate the availability of non-registered medicines for patients in your Member State?

Q 6: Does your regulatory framework for compassionate use allow the collection of more systematic data and information on safety and efficacy?

Q 7: Do you consider that a CHMP opinion on compassionate use facilitates implementation at national level? [

Q 8: When would you consider requesting a CHMP opinion for a compassionate use programme?

Q 9: Would you consider the availability of standardised application formats (templates, notifications) related to compassionate use programmes useful?

Q 10: Do you have any other comments or suggestions to optimise the use of compassionate use in the EU and/or your Member State?