EUROPEAN COMMISSION
HEALTH AND FOOD SAFETY DIRECTORATE-GENERAL

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

PHARM 775

PHARMACEUTICAL COMMITTEE

11 July 2019
Brussels
83rd meeting

SUMMARY RECORD

Meeting chaired by Olga Solomon, Head of Unit B5 – *Medicines: policy, authorisation and monitoring* - Directorate-General Health and Food Safety. Present: representatives from 28 EU Member States, EFTA (Norway, Lichtenstein), European Medicines Agency (EMA), Council of Europe.

1. ADOPTION OF THE AGENDA

Draft agenda (PHARM 768) was adopted without changes[1].

2. PHARMACEUTICAL LEGISLATION: CHALLENGES AND OPPORTUNITIES

a. ORPHAN AND PAEDIATRIC MEDICINES: EVALUATION OF THE CURRENT LEGISLATION

The Commission provided feedback on its 17 June conference titled 'Medicines for rare diseases and children: learning from the past, looking to the future' and presented the main ideas that came out of its five breakout sessions: unmet medical need, incentives, medicines for children, from R&D to patient and scientific developments.

The Commission also informed the Pharmaceutical Committee ('Committee') about the state-of-play of the ongoing evaluation of the orphan and paediatric regulations. The evaluation will help the Commission learn about the functioning of the two regulations and assess their actual performance compared to initial expectations. It will take the form

[1] The agenda and copies of relevant documents are available on the webpage of the Pharmaceutical Committee: https://ec.europa.eu/health/documents/pharmaceutical-committee/human-meeting_en

of a Staff Working Document which will be a factual presentation of the findings of the evaluation process and should be finalised before the end of 2019.

Based on the update of the Conference and the ongoing evaluation of the Orphan and Paediatric regulations, the Committee discussed the issue of "unmet medical needs" for which multiple meanings exist for different stakeholders. It was suggested that a mapping could aid in better targeting research and funding in this area. It was also mentioned that it is important that the recognition of unmet medical needs is underpinned by evidence.

The Committee also discussed the need to have the right incentives for the right products to ensure that certain therapeutic areas are not neglected. The availability of medicines (including medicines for orphan diseases) remains a concern especially for small Member States. The Commission reminded that, while this cannot be overlooked in the context of the regulation, other aspects such as HTA, pricing and reimbursement policies also affect access and availability of medicinal products.

b. SCENARIO-BASED DISCUSSION

Member States exchanged views on the preparedness of the current pharmaceutical framework with regards to tackling future and ongoing challenges on the basis of a hypothetical scenario. The discussion was pertinent in view of scientific and technological developments, notably in the area of information technology (e.g. Artificial Intelligence, big data, 3D printing) but also in genomics and other areas. Member States noted that regulators need to be ready to respond to those changes in a coordinated manner, as they are extremely complex.

The meeting participants agreed that there is a need to reflect on the challenges within the current framework and map existing issues while allowing more flexibility, traceability and trust in the system. We see a situation where innovative medicinal and combination products are becoming increasingly complex and may not fit under existing definitions. Regulators, with the cooperation of relevant bodies in the EU, may need to strengthen or revisit certain aspects of the system and its operation and enhance coordination.

Ethical, proprietary and security concerns such as ownership and access to data were also discussed. Participants stressed the importance of ensuring that personal information is collected, stored and analysed in a secure and unbiased manner. In addition, they recognised the importance of the post-marketing authorisation phase and discussed the possible use of real word evidence in the authorisation system along with enhanced monitoring throughout the product's lifecycle.

Developing (internal) specialised capacity would particularly focus on gaining expertise in data science, new clinical designs, modelling, medical devices, IVD and combination products. The system should also ensure that the EU is not overly dependent on active pharmaceutical ingredients originating from third countries for medicines that present a high unmet need so that the quantity and quality of supply in the EU can be guaranteed at all times. A possible first step is to develop a list of "strategic" medicines (such as antibiotics). Reflecting on the need for incentives to keep or increase manufacturing in the EEA was also suggested. Issues such as affordability and accessibility to medicines are ongoing problems that require our continued attention. Any future system should ensure equitable access to both new as well as existing substances and products.

Participants examined the possibility for a two-step strategy: Short-term actions addressing the most urgent issues (e.g. developments in the area of personalised medicines, medical devices/combination products) which could be addressed more readily and long-term actions within a more holistic approach. There is a need for horizon scanning capability and ability to build flexible platforms that bring relevant parties to discuss what is needed.

c. MARKET LAUNCH OF CENTRALLY AUTHORISED PRODUCTS

The Commission updated the Committee on the activities of the ad hoc Working Group on Market Launch of Centrally Authorised Products (WG). It informed that the next step would be to hold specific meetings on the 3 working papers prepared by the WG (namely: the sunset clause, improvement of knowledge on market planning of the marketing authorisation holder (MAH), and increase of transparency regarding the roll-out of centrally authorised products) so as to discuss each aspect in more depth.

The Committee also formally adopted the mandate of the Ad-hoc WG, which is tasked to:

- exchange views about the market launch of centrally authorised medicinal products;
- identify ways, to contribute to a quicker and more sustainable market launch with the aim to support the goal of centrally authorised products being widely available in the EU; and
- consider non-legislative action that could be taken to improve the situation.

The group may develop specific project proposals and should report in regularly to the Pharmaceutical Committee. It should avoid duplication of work with the HMA/EMA Task Force on availability.
While the primary focus of the group lies on short-term actions, where the group identifies limitations of the current regulatory framework, it may reflect on how these limitations could be addressed and report back to the Pharmaceutical Committee.

The Committee adopted the above mandate with the understanding that the members of the WG will also analyse the concept of “single market” and its interrelation with the provisions laid down in the sunset clause.

The members of the Committee exchanged views on the scope of the discussion and recognised that certain aspects of market access may extend to areas such as reimbursement and other aspects of EU legislation, however it clarified that the WG is only tasked to examine the aspects of the issue that are relevant to the remit of this Committee.

The ad-hoc WG is currently composed of 10 Member States and EMA. The Commission invited other members to join the ad-hoc WG.

3. ATMPS

a. HOSPITAL EXEMPTION

The Commission presented the updated version of the document on hospital exemption reflecting comments received from the Pharmaceutical Committee after the 1 April 2019 meeting. The development of a document on hospital exemption was considered useful. However, some delegations considered that additional reflection is necessary and a second round of comments (until 15 September) was agreed.

The Commission clarified that the document reflects the discussions among national experts in the context of a reflection process on the application of the hospital exemption in an *ad hoc* group. It was stressed that the document is not a Commission document, nor an interpretation, but it is a good practice that Members States may decide (or not) to follow when they apply Article 3(7) of Directive 2001/83/EC.

b. Guidelines ON GCP FOR ATMPS

The Commission presented an overview of comments received from the consultations of the Committee for Advanced Therapies, Good Clinical Practice inspectors and the Expert Group on Clinical Trials and how they had been reflected in the document submitted to the Pharmaceutical Committee.

The document was endorsed by the Committee.

4. REPURPOSING OF MEDICINES FOLLOWING STAMP

The Commission presented the final documents on a framework for repurposing of medicines and proposed pilot to test the framework which were endorsed by the Committee. The aim is to provide a visible supportive framework to not-for-profit stakeholders including academia ("Champions") who have the data and scientific rationale for providing evidence for a new indication of already authorised medicines. The Champions can engage with regulatory authorities/agencies, including EMA, to receive scientific and/or regulatory advice and with industry with a view to extend a marketing authorisation to new approved indications. A pilot to test the framework will begin in the autumn.

The Repurposing Observatory Group led by Spain will facilitate the process, oversee and monitor the pilot and report on the experience. The group would not make regulatory decisions regarding the candidate molecules considered within the pilot project. Member States interested in participating in the Repurposing Observatory group were asked to inform the Commission.

The Committee endorsed the framework, the pilot project and the mandate of the Repurposing Observatory Group.

5. A.O.B.

Belgium presented questions related to the regulatory framework on bacteriophages in advance of the next Pharmaceutical Committee meeting tentatively scheduled on 7 November 2019 where the issue will be discussed in the framework of a general discussion on antimicrobial resistance (AMR).

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