EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Stakeholders and Communication Division

Highlights of EMA - Thalassaemia International Federation (TIF) bilateral meeting

29 September 2023 – moderated by Juan Garcia Burgos

1. Welcome and introductions

The Executive Director welcomed all Thalassaemia International Federation (TIF) participants and round table introductions of EMA and TIF participants took place. TIF has been an EMA eligible organisation since 2007 and has collaborated with EMA on many activities, including being member of the PCWP and having representatives in EMA scientific committees.

The meeting was intended to provide an opportunity to discuss topics of mutual interest to TIF and EMA, and to all patient organisations and will be followed up with the PCWP.

2. Patient participation at the EMA in the coming years

The changes that will be brought by the new pharmaceutical legislation were raised and TIF highlighted their commitment to their continued participation and support to EMA.

EMA reassured TIF that patient engagement is an important activity at the Agency, which will continue to be a priority, as it prepares to implement the proposed revision of the pharmaceutical legislation.

3. Definition of unmet medical needs

TIF expressed concern around the terms "unmet medical needs" and "high unmet medical needs" and wanted to discuss how these will be defined as well as the incentives for developers to continue research into orphan medicines.

The Agency explained that the European Commission has proposed a criteria-based definition in the draft legislation that will help to objectively define the medical need and to associate specific tools that would incentivise the development and authorisation of medicinal products in therapeutic areas that are currently underserved. Under the draft legislative proposal, the CHMP will be in charge of preparing scientific opinions on medicinal products. This will include the assessment of the unmet medical need and high unmet medical need will be under the responsibility of the CHMP. It is noted that patients will be members in the CHMP, fully contributing to the decision-making process.

EMA welcomes contributions on this subject from TIF in context of their membership of the PCWP, where the guidance of the definition of unmet needs will be discussed in due course.

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4. EMA/FDA collaboration

TIF provided their views regarding collaboration between the EMA and the FDA as some differences in recommendations have been observed. EMA explained that FDA, DG SANTE and EMA have a confidentiality arrangement that allows for close collaboration and discussion on different areas of medicines regulation. Regular interactions are held on topics or issues where scientific collaboration and intensified exchange of information are necessary in the context of clusters meetings.

Thalassaemia could be covered as part of the blood Cluster that meets three times per year with participation of EMA, FDA and Health Canada. In this cluster, there are product related discussions, guideline discussions, policy discussions among others. Additionally, ad-hoc bilateral meeting with FDA can take place to discuss product related issues whenever is needed.

5. Conclusions and next steps

The meeting was an opportunity to have a discussion on these topics of high interest to patient organisations. The importance of patient engagement for the Agency was reiterated. EMA will continue to strengthen this dialogue to address issues of common interest to both regulators and the patient community.