Open Academy Meetup - September 2023

Everything you wanted to know about the reform of the EU Pharmaceutical Legislation

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Pharmaceutical Framework Reform: Increasing Accessibility of Transformative Therapies

Simone emphasized the need for pharmaceutical framework reform to increase the availability of transformative therapies, highlighting the problem of accessibility in many countries and the goal to develop 1000 therapies within 10 years. He also mentioned Eurordis's engagement with the European Commission to revise legislation and ensure strategic autonomy in research and development. Kaja presented the current status and future changes regarding EU policy on orphan medicines and paediatric medicines, discussing the ongoing pharmaceutical reform and its building blocks initiated by the pharmaceutical strategy. She also talked about the review of the regulation on orphan medicines, which started in 2017, and the subsequent evaluation results that led to future plans to improve the situation for patients with rare diseases, including children.

EU Pharmaceutical Strategy and COVID-19: Reform and Incentives

Kaja discussed the evolution of the European Union's pharmaceutical strategy in the context of the COVID-19 pandemic and its connection to the revision of the Orphan and Paediatric Legislation. She highlighted the key political objectives driving the recent pharmaceutical reform, including improving access, availability, and affordability of medicines, maintaining a competitive framework, ensuring environmental sustainability, and addressing the needs of patients with rare diseases. Kaja also explained the incentives provided to developers of orphan medicines, including market exclusivity and scientific advice, and outlined the criteria for a product to receive orphan designation.

Paediatric Regulation: Increased Clinical Trials and New Plans

Kaja discussed the Paediatrics topic, highlighting the increase in clinical trials involving children and more authorized medicines since the implementation of the paediatric regulation in 2017. Kaja clarified that, unlike the Orphan regulation, the Paediatric regulation is a legal obligation for companies to study their products in children, with rewards once the study is done. She also noted that companies were requesting deferrals for their Paediatric Investigation plans, with a new stepwise adopted Paediatric Investigation Plan to facilitate the process. Additionally, Kaja mentioned NGOs and academics could now submit clinical

data for the repurposing of a medicine and the abolition of the 2-year market exclusivity for orphans.

Transparency and Patient-Centered Reform in Discontinued Medicines

Kaja presented on the importance of transparency in discontinued medicines and the establishment of a multi-stakeholder expert group for pediatric research. There was a focus on ensuring the European Parliament's pharmaceutical reform was patient-centered, with the aim of introducing innovative products for patients. Questions were raised regarding how significant benefits are defined and who gets to decide, as well as how to ensure patients are seen as equal partners in the design and conduct of clinical trials.

Patient Involvement in Research Prioritization

Kaja discussed the criteria for prioritizing research and product development, highlighting the importance of patient involvement in the decision-making process. She explained that the 'high-end needs' concept has been introduced to guide the process, but acknowledged that it would be impossible to be prescriptive in a legal act. Kaja emphasized the need for discussions and decisions on what constitutes a 'meaningful reduction in morbidity and mortality', which is both disease-based and product-based. She stressed that patients' needs must be considered in these discussions, as they play a crucial role in defining the research program's priorities.

Simplifying EU Medical Product Authorization: Patient-Centric Focus

Kaja and Dominique Sturz discussed the simplification of the authorization process for medical products in the EU. They focused on the need to involve patients early in the process and to make it more efficient, with the aim of bringing products to market quicker. Dominique raised concerns about the disappearance of orphan drugs from pipelines and questioned whether these decisions were sometimes driven by business rather than scientific considerations. Both agreed on the importance of ensuring patient-centricity throughout the drug development cycle. Kaja also explained changes to the orphan designation process, setting a limit of seven years to push developers to move more quickly.

Pharmaceutical Reforms and Children's Therapies

Simone and Kaja discussed plans for pharmaceutical reforms, with a focus on the potential for new therapies for children's diseases. Kaja was hesitant to provide specific numbers for the potential new therapies, but emphasized the importance of the mechanism of action and the introduction of criteria for unmet needs. Simone expressed concern about patient involvement in the regulatory process and asked if there was a need for further patient representation in the regulations. Kaja agreed that patient involvement would be crucial, particularly in the main committee and in the development of scientific guidelines.

Regulation Impact on R&D Spending Discussed

Simone and Kaja discussed concerns raised by industry stakeholders about a proposed regulation potentially reducing spending on research and development. Kaja acknowledged the reduction in incentives for data protection and market exclusivity, but argued that the reform also provides significant market exclusivity for orphan conditions, encourages more developments for molecules, and allows rolling review for promising medicines. Simone

raised concerns about the limit of 13 years of market exclusivity and the potential impact on developers, both commercial and non-commercial.

Next Steps:

• Organize a follow-up meeting with the Parliament rapporteurs.

• Send any additional questions to Marta and Rachel via email.

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