European Health Forum Gastein 2021

A BETTER TOMORROW FOR RARE DISEASE CARE AND RESEARCH

Rethinking investment and access in light of the EU OMP Regulation review

EVENT REPORT

29 September 2021, 09:00-10:30

EUCOPE, in partnership with Sanofi, participated in the European Health Forum Gastein on 29 September in a session on rare diseases to discuss what is needed to stimulate basic research, clinical development, and access to care across the EU in light of the EU Orphan Medicinal Product (OMP) Regulation Review.

Speakers:

1st panel: "Looking back"

- **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe
- **Kaya Kantorska**, Policy Officer, Pharmaceuticals, Directorate-General for Health and Food Safety, DG SANTE, European Commission
- **Manuela Buxo**, Head of Region Europe, Sanofi Genzyme

2nd panel: "Looking forward"

- **Michela Gabaldo**, Head of Translational Projects, Management & Regulatory Affairs, Fondazione Telethon
- **Alexander Natz**, Secretary General, EUCOPE (European Confederation of Pharmaceutical Entrepreneurs)
- **César Hernández García**, Head of the Department of Medicines for Human Use, Spanish Medicines Agency

Moderator:

- **Christian Jervelund** Health Care & Life Sciences Partner, Copenhagen Economics

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The current OMP Regulation review represents a unique opportunity for Europe to tackle remaining gaps in rare disease research, investment and care. It is essential that the fight against rare diseases remain at the heart of Europe's recovery.

The **first panel** looked at the achievement of the EU OMP Regulation to date and the broader context this review is set into, including the ambitious plan of the European Commission with its Pharmaceutical Strategy for Europe.

Kaja Kantorska, policy officer at the European Commission working on the OMP Regulation review, highlighted the success of the legislation since its adoption in 2000. Today, there are about 200 authorised treatments which have transformed the lives of many patients in Europe. However, she also noted that 95% of rare diseases remain without authorised treatment. The current lack of treatment and accurate diagnosis for the majority of rare diseases represents an enormous unmet medical need (UMN). It is, therefore, the perfect moment to look at possible solutions that will foster development addressing those unmet needs.

To complement the Commission view on the topic, representative of industry and patient group also contributed to the discussion and expressed their views on the important changes the OMP Regulation brought to the rare disease framework.

Different challenges were identified such as the need to strike a balance between needed change and a system that continue to attract companies to invest in rare diseases. **Manuela Buxo,** Head of Europe Region, Sanofi Genzyme, pointed out that the EU cannot afford to lose ground to other regions of the world such as the US or Japan in terms of patient care and research and development efforts. Going forward, the EU should provide incentives to develop medicines addressing all rare – not just ultra-rare diseases. She added that the real challenge lies in creating access conditions that reward innovation and ensure rare disease patients get access to the medicines they need.

While **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe, outlined the Regulation had been able to save and transform the lives of many patients in Europe, but it must now adapt to the new standards of science and technology. The revised OMP framework should acknowledge the importance of gene and cell therapies and modulate incentives in order to attract investment in areas of UMN. He also stressed the importance of upstream dialogue and coordination among regulators, patients, HTA bodies and developers to pave to way to faster access to rare diseases therapies.

In the **second panel**, experts focused on solutions to tackle challenges discussed in the first session and fleshed out actionable ideas to strengthen the OMP development environment in Europe. The panel built on the recommendations of the European Orphan Drug Expert Group (OD Expert Group) presented earlier this year [Link]. The OD Expert Group is a multidisciplinary group of rare disease community experts bringing a multi-stakeholder perspective on how to address the unmet needs of rare disease patients in the evolving European orphan drugs landscape.

Michela Gabaldo, Head of Translational Projects, Management & Regulatory Affairs, Fondazione Telethon believed the main concern for the future is to improve access for orphan drugs, which is even more challenging in the case of orphan drugs for Advanced Therapies for Medicinal Products (ATMPs), as they require highly specialised clinical expertise, which do not exist in all EU counties. In this context, crossing countries can often be the only solution for patients. It is, therefore, essential to build a functioning cross-border healthcare framework that is able provide patients with access to those therapies.

From the national perspective**, César Hernández García,** Head of the Department of Medicines for Human Use at the Spanish Medicines Agency highlighted of the importance of adopting a multistakeholder approach when addressing the future of rare diseases and the OMP legislative framework. All stakeholders, including regulators, researchers, payers, industry, patients and policy makers, should come together to reach closer collaboration and create a strong OMP development framework that actually reach the patients.

Finally, **Alexander Natz,** Secretary General at the European Confederation of Pharmaceutical Entrepreneurs, (EUCOPE) shed light on the importance to think broadly of ways to ensure access to rare disease treatments in Europe and to cooperate and coordinate better at EU level. He pointed out we should build on existing infrastructures such as the European Reference Networks (ERNs) in order to create a clear governance structure for rare disease data collection and care coordination across the EU.

In a final call to action, speakers agreed that creating 'pilots' of multistakeholder cooperation from research to access would be key to tackle rare diseases' challenges. They reiterated the need to continue working together and develop a new companionship in the coming years in order to improve patient access to treatments across the EU and to keep rare diseases a public health priority.

For all inquiries on this session please contact

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