Roundtable 'Revision of the European Orphan & Paediatric Regulation and impact for the Nordic rare disease community'

EVENT REPORT

7 September 2021, 12:00 – 13:30

The following document summarises the key points discussed during the roundtable 'Revision of the European Orphan & Paediatric Regulation and impact for the Nordic rare disease community'.

Panellists:

- **Birthe Byskov Holm**, President, Sjældne Diagnoser (Rare Diseases Denmark)
- **Pernille Weiss**, Member of European Parliament (EPP)
- **Lina Nordquist**, Member of Swedish Parliament (Liberalerna), spokesperson on health policy
- **Jonas Vikman**, Director Government and International Affairs, LIF Sweden

Keynote speech:

- **Terkel Andersen**, President of the EURORDIS Board; Co-founder of Rare Diseases Denmark

Moderated by:

- **Line Friis Frederiksen**, journalist, biologist and experienced moderator

Welcome address:

- **Vittoria Carraro,** EUCOPE and **Johan Järte**, IML

Organised in partnership with the European Confederation for Pharmaceutical Entrepreneurs (EUCOPE) and the Swedish Industry Association for Small and Medium-Sized Life Sciences Companies (IML).

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The European Regulation for Orphan Medicine Products (OMP) is currently under review and the European Commission is expected to publish its legislative proposal in late 2022. Find below the main themes discussed during the roundtable.

OMP Regulation: successes and areas for development

- The OMP Regulation was the first attempt at regulating the rare disease (RD) space. Over the last 20 years, it has successfully managed to incentivise R&D for orphan drugs, with more than 200 products receiving Marketing Authorisation (MA) and more than 6 million patients accessing treatments across the EU. This is also demonstrated by the fact that, currently, 41% of all drugs in clinical trials aim to obtain an orphan designation, while 20 years ago most drugs were 'blockbusters'.
- However, this did not translate into equal access and development across disease areas. Most OMPs are concentrated in certain disease areas (especially antineoplastic and alimentary tract diseases), and 55% of MAs have been granted to RDs with a prevalence of 1-5 in 10,000 (thus, the most prevalent),

- The rarest RDs account for 89% of all RDs and have benefitted little of the treatments developed since the introduction of the regulation. Overall, it is clear that there is still a significant unmet need.
- A key reason for the delay in the development of OMPs is the small patient population. It should be possible to accelerate treatments by allowing quicker access to treatments in development and enable the collection of Real-World Evidence (RWE). European Reference Networks (ERN)s should have a key role in collecting this data and ensuring it is captured and shared.
- Even when a new orphan drug has obtained a MA, there is no guarantee that patients will be able to access the treatment. The average delay between MA and availability can be 3-5 months in some countries, but also 3-6 years in others. Each Member State (MS) has its own Health Technology Assessment (HTA) procedures in place, which result in delays to access, or even no access at all.
- The European legislation should therefore be seen as a foundation to stimulate investments in the areas of OMPs but should be complemented by national legislations in order to ensure patient access and sustainability.

Role of patient groups

- Patient groups are already occasionally involved in the development of RDs but speakers agree that more cooperation should be considered, for example to include the patient experience in a scientific and transparent manner.
- Patient groups have expressed their willingness to be involved in the development of RD medicines and help drive the innovation to treat their conditions.

Potential of cross-border cooperation

- During the discussion, the need to have a common infrastructure for data collection across EU MSs was pointed out. Given the small patient populations of many RDs, sharing clinical data can be valuable to improve research & innovation as well as care provision.
- Another EU-wide action that can be considered is the development of centres of excellence not only sharing knowledge, but also treating each other's patients when needed. Speakers raised the point that it shouldn't matter where patients live for them to be able to get access to the best care.
- The key to ensuring access and sustainability is being attentive to the situation of patients and their relatives, to the needs of the pharmaceutical industry and to what other countries are doing. Sharing best practices is indeed essential to improve access across the EU, especially when it comes to ultra-rare diseases, as countries alone might not have the right knowledge and resources to deal with them.
- Patient groups are delivering very valuable contributions to the development of RDs but given the limited patient populations, cross-border data sharing should be considered. This can include EU-coordinated programmes based on RWE.

Importance of R&D investment

- The revision should focus on improving incentives, tools and strategies in order to tackle RDs and driving innovation, as this will profile the EU as an attractive market for investors, the pharmaceutical industry and researchers to address unmet needs. Attracting these investors and entrepreneurs would be beneficial for the healthcare profile of the EU.
- The central reason many RDs do not have a treatment yet is a lack of basic research and knowledge of the diseases. This means that driving this R&D investment is especially critical for OMP's because the EU needs to attract the researchers who make it possible to create medicines that are not on the market yet.
- Access and availability are important parts of the equation, but without the R&D investment in creating medicines RD patients need, their needs will not be met.
- While 3% of national budgets should be spent on research, as per a long-term European Council objective, most countries are nowhere close to this threshold. Agreements reached at the EU level are not often reflected at the national level. In Eastern Europe especially, investments in R&D lag behind. In the long term, this can jeopardise the EU's competitive muscle and healthcare profile on a global scale.
- The UK was a clear leader in medical research in Europe but after Brexit, the EU will need to ensure a global leadership position in the field of research and development.

Next steps to make the revised legislation a success

- Investments in R&D should be targeted at those areas of unmet need, which also encompass diseases for which treatment(s) might be available but without treating all unmet needs.
- It is essential to take stock of what we have learnt in the past 20 years, thus looking at the gaps in the legislation. In this context, contributions from patient organisations, based on real patients' experiences, should be taken into consideration in the revision.
- The revision should not only aim at maintaining, but even boosting competitiveness, in order to make the EU an attractive market for R&D. This requires a new vision for incentives, tools, and strategies to drive R&D.
- The legislation is key to improve the availability of OMPs, but there are other factors influencing the access and sustainability side of these drugs. A multi-stakeholder dialogue should be started to look into the root causes of unequal access across the EU and unequal R&D efforts across RD areas in order to come up with possible solutions and a model for collaboration across MSs.
- Clear indicators and goals should be agreed on, in order for EU policies and initiatives to align to achieve them.

Rewatch the roundtable

The recording of the roundtable is available on the EUCOPE YouTube channel and here.

Resources

Some further background readings and proposals for the EU Orphan Medicinal Products legislation revision are available here.