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Outlook on European market access strategies for rare diseases

World Orphan Drug Congress USA 2021 Brief




Moderated by Anna Bucsics, Project Advisor, Mechanism of Coordinated Access to orphan medicinal products (MoCA); the panel comprising Reda Guiha, Regional President of International Developed Markets, Rare Disease Division, Pfizer; Marcus Guardian, Chief Operating Officer, EUnetHTA; and Simone Boselli, Director, Public Affairs, EURORDIS, discussed considerations for legislative changes for orphan products, updates to clinical assessments, and continued hurdles for rare disease patients and drug developers.

Panelists were not consulted in the development of this summary

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We identified 3 main themes from the lively and insightful discussion as summarized below:

Urgency

The unmet need in rare diseases remains very high. According to the panel, only 5% of patients have access to curative or transformative therapies and 40% do not have access to any treatment at all. Clearly, we need better treatments as the currently licensed or off-label drugs are not effective enough. There can also be long delays from regulatory approval to reimbursement, sometimes for more than 4 years, that impact patients' timely access to much needed therapies.

That's why Rare Diseases Europe (EURORDIS) has set an industry objective to make 1000 new rare disease therapies available by 2030. However, the panel also noted that patient access is a journey that starts in the laboratory and involves many complex steps to get patients access to the treatment. So how can this be sped up?

The panel thinks there are important lessons to be learned from the COVID-19 pandemic, which has led to end-to-end innovation at an unprecedented pace. Usually, it takes 10 to 14 years for a company to get a vaccine to market, but it has happened in less than 1 year. The scale of investment was a major factor in the vaccine race because there was such an urgent need.

But there is a similarly immediate need for treatments for rare diseases, and the funds are not there. And so, the panel urges us to capitalize on this crisis with a courageous and risk-embracing mentality from all stakeholders to redress the deficit.

Opportunity

Europe is facing regulatory changes on several fronts. First, the European Commission is looking at its orphan drug legislation. Introduced in 2000, the research and development incentives and support that this provides to manufacturers have led to the approval of more than 160 drugs for rare diseases in the past 20 years.

Industrial representatives on the panel caution against making the criteria for orphan drug designation more stringent or tampering with the 10-year marketing exclusivity bonus awarded to these products. The panelist notes that approximately half of the approved rare disease products would not have been commercially viable without these incentives.

So, whereas some aspects should not be changed, what can be changed and how will the change impact rare diseases? Although the reforms have been in the works for a while, the pandemic provides a unique opportunity to "shake things up" in a meaningful way. The European Commission now has the opportunity to look at the whole ecosystem of legislation. As the panel says, "more can be done."

Second, the European Commission is putting its "stake in the ground" on health technology assessment (HTA), often the bottleneck to timely and equal patient access. This European HTA regulation would manifest as a Europe-wide law that supersedes national law. It is an attempt to bring cohesion and efficiency to the European HTA processes, which are currently decentralized with national, regional, and, occasionally, local HTA.

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Built on the work of the European Network for Health Technology Assessment (EUnetHTA), which has identified commonalities across countries, the main part of the proposal is for the joint clinical assessment (JCA) of pharmaceuticals and medical technologies but will also include horizon scanning and early dialog opportunities as part of a framework where centralized reports can be used across the EU.

The panel's consensus is that for rare disease in particular, the JCA could be a big advantage that should allow for faster, better, and more equal access across the EU. But the panel also warns that "the devil is in the details," and because the current text provides flexibility for nations to use or not use the JCA, there still might be some duplication of efforts, especially because individual nations will maintain sovereignty of economic and pricing assessments.

Overall, the reform was viewed as an opportunity for the EU to place itself at the forefront of innovation, hence, the call for more weight to be given to rare diseases and advanced therapy medicinal products in the legislation to keep it firmly on top of the agenda and maintain innovation.

Collaboration

The pandemic has also highlighted to the panel the importance of working together. After achieving so much in such a short time for COVID-19, the panel asked why not for rare diseases? After all, one panelist noted that the world has now seen first-hand in COVID-19 what having a rare disease is like, with various symptoms, uncertain diagnosis, and the lack of treatments.

What was encouraging to the panel was that the pandemic collaboration primarily took precedence over competition. Examples include increased interaction between regulatory and HTA bodies, even without a legal framework, joint procurement of vaccines, which was previously thought too administratively burdensome, and genetic testing, which is also a key component of the rare disease patient journey. This proves that the capabilities exist. The next step is to bring the approach from crisis mode to "normal" conditions.

To do so, collaboration is needed across the whole product life cycle and must include all stakeholders–manufacturers, regulators, payers, and patients too. Here, transparency is key as is a clear framework, with the EUnetHTA panelist highlighting a role for European regulatory-access roadmap to support innovators from early on in drug development to postlaunch real-world evidence (RWE) generation.

The industry panelist was vocal on the latter point regarding the alignment of RWE strategy with regulators and payers, which is especially important for gene therapies that treat rare diseases because their transformative long-term benefits are not sufficiently captured in conventional clinical trials. This point relates to another request to introduce more flexibility in the regulatory process for rare diseases to allow rolling review of data that are generated over time, as occurred with the COVID-19 vaccines.

All panelists agreed that strong collaboration between HTA partners and the wider community will be critical to coordinate processes and ultimately provide faster patient access.

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