EN VOZ RARA

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REVISION OF THE EUROPEAN REGULATION ON ORPHAN DRUGS.
CONCLUSIONS.

Impact on and contribution to the quality of care, quality of life, and efficiency of management of rare diseases and orphan drugs.

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In the year 2000, the European Union adopted a regulation on orphan drugs that represented a turning point, establishing incentives for the research and development of relevant treatments. This was made possible thanks to dialogue between authorities and the pharmaceutical industry, and strongly stimulated the work of companies in this field.

Without doubt, the current regulation has been a success, and Europe has greatly benefited from its Orphan Drug Regulation.

The current system of incentives for the research, development, and registration of these drugs enabled more therapies to reach the market, thereby transforming the lives of patients and their families, improving health outcomes, and contributing to the economy of the entire European Union. However, despite these achievements, 95% of rare diseases still lack treatment options. Children account for 50% of those affected.

The current regulation has helped provide access to around 200 medicines in EU countries and has benefited more than 20 million patients throughout the region, facilitated clinical research (AEMPS: more than 4,000 trials underway in 2019, 837 focused on rare diseases), and expanded knowledge of rare diseases, which is essential for the development of new treatments: 24 reference networks have been created in Europe; 23 member states have launched national plans for rare diseases, including Spain.

Now, the aptly named initiative ***EN VOZ RARA***, has given a voice to the different stakeholders within the health ecosystem involved in improving the lives of those affected by so-called rare or infrequent diseases, starting with the patients themselves and the associations that represent them.

This project could not be more timely: an interesting debate is currently underway within the European Union, with a view to remodelling the framework that regulates orphan drugs, about which Dr. Alvaro Lavandeira has enlightened us. A new environment that will undoubtedly allow us to move towards a more hopeful future for affected patients and their families.

En Voz Rara, anticipating changes to the current EU regulatory framework, has transparently debated that which has been achieved with the current regulations, as well as key aspects to improve that should be considered as part of the proposed changes.
It is important to continue guaranteeing a stable and predictable regulatory framework, with incentives for the pharmaceutical industry, which in turn supports the research and development of new treatments for the 95% of rare diseases for which there are no therapeutic options.

Predictability is a key requirement because the decisions are made by the sector at least 10 years before a drug reaches the market.

Currently, the regulatory framework for orphan drugs in the European Union provides for 10 years of market exclusivity along with protocol assistance and fee waivers. We remain convinced that the existing framework is a success and that a substantial part of the observed inequality of access stems from national policies and decision-making processes.

This is not the time to jeopardize the continuity of the achievements made with a proven and effective regulation, but rather to further encourage research into new treatments for patients with unmet medical needs, especially when one of the deficiencies identified by the Commission in its evaluation report is the absence of any treatment for 95% of rare diseases.

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In the current context of dialogue and debate on regulation, we welcome the efforts of the European Commission to explore the creation of additional incentives to stimulate innovation and the development of orphan drugs in areas with unmet medical needs. In this regard, an open and constructive dialogue should be promoted with all interested parties regarding the definition of what should be considered an unmet medical need.

On the other hand, the necessary balance between innovation, access, and sustainability should not be jeopardized.

Together with health authorities, it is necessary to further delve into the definition of drug entry agreements that are novel (payment by results, payment deferred over time, etc.) and facilitate rapid patient access to new treatments, allowing payers to better manage clinical uncertainty and budgetary impacts, and thereby contributing to the sustainability of healthcare systems.

We are convinced that there is still a long way to go. The creation of patient registries, data sharing, and greater public-private collaboration would also help in this journey that the European Orphan Drug Regulation began 20 years ago, which requires the global cooperation of all agents to continue investigating and offering hope to patients for decades to come.

There are multiple avenues yet to be explored:

- Accelerated financing procedures.
- Early dialogue.
- Criteria for the evaluation and financing of orphan drugs that, in addition to considering budgetary impact and cost-effectiveness, take into account the severity of the disease, the needs of affected groups, and the social value of the drug.
- Greater involvement of experts and patients in decision making.

The following are some of the main considerations addressed and challenges posed:

- Patients with rare diseases should benefit from the same quality of treatment as anyone else.
- The current orphan drug regulation has promoted research and development of treatments and has encouraged biotechnological entrepreneurship.
- Accelerate access to innovation, reducing the times between drug approval and its commercialization in Spain, avoiding feelings of abandonment on the part of the patient and of impotence on the part of health professionals.
- Increase equity and coordination between Spanish autonomous communities and even between hospitals.
- Spain must close the gap relative to other European countries in terms of the number of treatments available and financing terms, favouring patients.
- Effective participation of patients and professionals must be implemented at all levels of the approval process.
- We must ensure that the criteria for drug access are mainly centred on the health needs of the patient.
- Research into rare diseases must be promoted in the European Union, guaranteeing competitiveness against other countries (e.g. the United States, Japan).
- The future Spanish presidency of the European Commission in 2023 is an ideal opportunity for the Spanish health administration to consolidate its commitment to rare diseases.
- Any modifications to the regulation must continue to promote research while solving existing problems of access and improving patient quality of life.

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Although we have made progress, we need to maintain incentives for the development of Orphan Drugs. The current difficulties in access cannot be addressed within the European regulatory environment. Rather, this is the responsibility of each of the member states.

It is critical to provide a regulatory environment that allows a response from the promotion of science. We can optimize access in a sustainable environment without needing to change the legislation: new access models, digitization, etc.

In conclusion:

1º This is the right time to calmly assess the role played by the Orphan Drug Regulation. Twenty years have passed, and we have the data. It is time both to build and to conserve.

2º Given the vastness of the field of rare diseases it is unsurprising that a huge number of unmet medical needs remain. It should be emphasized that the reason for this lies not with inadequate regulation, but with the enormity of the challenge posed by so many different diseases.

3º It is clear that the incentive system is fundamental, although there is room for improvement.

4º The two main problems are the number of unmet medical needs and deterioration of access (difficulty and inequity).