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Rare diseases: Protecting and perpetuating the incentive character of the European regulation to ensure continuity of innovation

Rare diseases affect around three million people in France, of whom 75% are children. Despite being a major public health issue, they have serious effects on the lives of patients and unfortunately go untreated in the majority of cases.

The committed pioneering role played by France

The launch of the First National Rare Diseases Plan in 2005 made France a European pioneer in orphan diseases. This pioneering role has since led to considerable progress being made in improving and structuring research, diagnosis and treatment at national level

Furthermore, France also initiated the December 1999 adoption of a European regulation on orphan medicinal products (REGULATION (EC) No. 141/2000), which provided an effective incentive for the development and marketing of medicinal products designed to prevent or diagnose rare diseases.

In practical terms, this regulation defines orphan medicinal products on the basis of harmonised criteria, introduces a centralised marketing authorisation (MA) procedure for products intended to treat rare diseases, and ensures market exclusivity (10 years, extended to 12 years in the context of a Paediatric Investigation Plan (PIP)).

However, the reopening of the European regulation by the European Commission could potentially threaten these innovation-friendly provisions.

A European regulation whose incentive framework must be protected and perpetuated...

The results obtained since the introduction of the regulation have been positive. And **the number of treatments approved by the European Medicines Agency increased from 8 in 2000 to 190 in 2020**.

Trials related to clinical research into rare diseases rose by 88% between 2006 and 2016.

Clinical trials of treatments for rare diseases now account for 22% of all trials initiated by the pharmaceutical industry. Some 2,800 clinical trials are now underway. A significant proportion of those are at an advanced stage, and likely to provide new therapeutic responses going forward.

After the regulation came into force, 220 new SMEs were formed, and these businesses are now responsible for the development of 51% of orphan drugs in Europe.

... to enable the pharmaceutical company innovation required to provide the therapeutic solutions of tomorrow

Despite the fact that some limitations, including unmet medical needs and unequal access from country to country, remain, it is nevertheless strategically important for Europe to retain this incentive-driven framework. Without it, our continent would be deprived of an essential innovation research and development resource that benefits European patients, as well as the competitive edge enjoyed by its pharmaceutical industry.

Estimates suggest that without it, 44% of the orphan products approved since its introduction would probably not have been developed at all, and around 2 million European patients would not have had access to treatment.

"For all these reasons, the pharmaceutical companies are asking for the retention and perpetuation of this incentive-based, predictable and understandable system in order to continue driving forward the innovation essential for providing patients with the best-possible treatments and to address medical needs that currently remain unmet. More than at any time in the past, pharmaceutical companies now want to be closely involved in European policies designed to structure research for the benefit of rare disease patients," concludes LEEM Chairman Frédéric Collet.

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