

Tackling rare diseases a 'European success story'

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News

Tackling rare diseases, said to affect up to 30 million people in the EU, has been hailed as a "European success story."



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Speaking in Brussels, Yann Le Cam, CEO of Eurordis, the European organisation for rare diseases, said, "We have gone from near ignorance to the recognition of rare diseases as a public health priority in Europe."

He added, "The landscape has changed drastically; expertise and innovative technologies that can potentially benefit people living with a rare disease have been developed."

He said, most EU Member States now have national rare disease strategies and connected specialised centres of care which was thanks in part to the establishment of European Reference Networks (ERNs), which were launched two years ago.

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He was speaking at an event, "Equal access to care for rare diseases," organised by Friends of Europe, a leading Brussels-based think tank.

The event was told that rare diseases, often of genetic origin, affect small to extremely small populations of patients, usually fewer than 1 in 2,000 individuals.

Most rare diseases are chronic, progressive, degenerative, disabling and frequently life-threatening, it was said.

Each of the over 6,000 identified rare diseases affects a very small population, but altogether they affect 25 to 30 million of people in the EU - around 5 percent of the EU population - or the population of Belgium and the Netherlands combined.

Le Cam said this is without taking into account carers, families and friends.

Such diseases are 'rare' because their prevalence in the general population is extremely low, and because they are all too often 'forgotten' precisely because of the scarcity of medical knowledge about them, he told the debate.

"The lack of knowledge means that many rare diseases are not diagnosed, that disabilities and other symptoms are not recognised, and that in turn persons with rare diseases do not receive the same compensatory support as those with more common and well-known conditions."

"The landscape has changed drastically; expertise and innovative technologies that can potentially benefit people living with a rare disease have been developed" **Yann Le Cam, CEO of Eurordis**

He added, "Individual countries cannot face alone issues related to rare diseases."

Rare diseases, he said, represent an area where "European action brings true added value."

"A strong track record of achievements on rare diseases has been established through cross-country collaboration and the support of the European Union."

A number of countries, he said, have "led the way" on rare diseases, with European legislative proposals such as the Orphan Medicinal Product Regulation in 1999 and the Directive on Patients' Rights in Cross Border Healthcare (2011).

Despite such progress, he told the event, “Still there is a lot left to do.”

“A significant challenge for patients, professionals, and health and social systems in Europe is the absence of streamlined, integrated pathways to allow people living with rare diseases to navigate health and social care systems.”

“This is particularly problematic in view of the complexity of many of these 6,000 conditions, and the lack of awareness and understanding in all sectors of society regarding their full impact.”

“Despite successful progress, the original ambitions remain far from being fully achieved, especially when considering patient access to approved therapies across Europe.”

Le Cam said that new scientific developments, the promise of many new therapies and the new diagnostic landscape may raise hope “but new challenges in pricing and access of therapies show how existing models are not sustainable and an area where increased European cooperation could bring concrete solutions.”

Another speaker, Rute Fernandes, head of rare diseases for Europe and Canada at Takeda, highlighted another issue of concern: accessing treatments and medicines for patients with rare diseases, saying, “It still takes too long for a patient to be diagnosed which is one reason why Member States need to adopt national action plans and establish centres of excellence.”

About the author

Martin Banks is a senior reporter at The Parliament Magazine

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