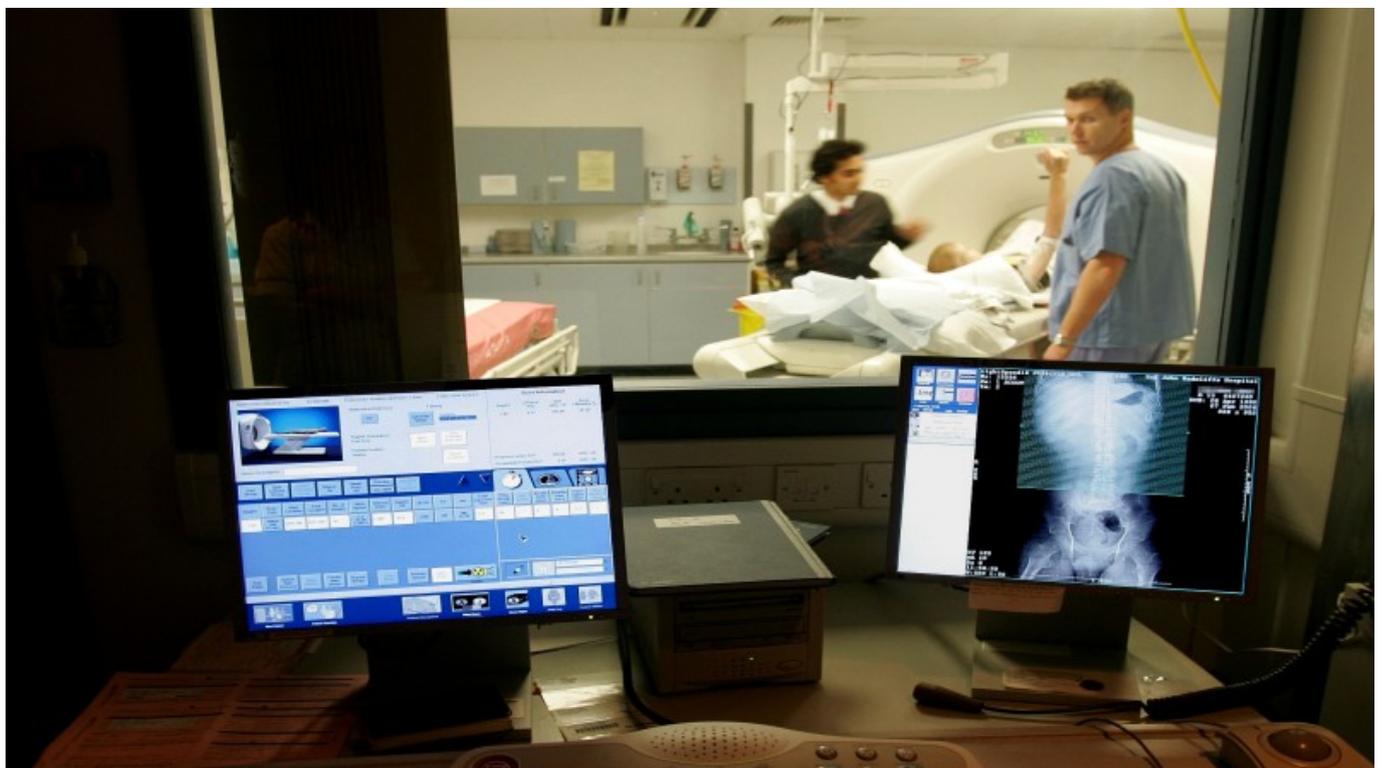


Rare disease patients in EU and US share policy priorities

Written by Pamela Gavin on 4 November 2014 in Opinion
Opinion

There is still a lot of legislative work to be done for rare disease patients, but EU and US organisations are stronger when they work together, writes Pamela Gavin.



Recent advances in the scientific understanding of rare diseases have underscored the need for patient advocates in the EU and US to work together to promote public policies that are important to patients on both sides of the Atlantic. That is why the two leading rare disease patient organisations - the national organisation for rare disorders (NORD) in the US and the European organisation for rare diseases (EURORDIS) in the EU - joined forces a few years ago to collaborate on shared objectives.

As I look over the agenda for the upcoming world orphan drug congress and reflect upon topics discussed at NORD's recent rare diseases and orphan products breakthrough summit, which took place last October, I am struck by the overlapping nature of patient priorities in the EU and US. Both sides want strong research and regulatory programmes. The national institutes of health (NIH), the chief governmental source of research funding in the US, has actually lost purchasing power in recent years as a result of stagnant funding. Similarly, the food and drug administration (FDA), which

regulates products that represent 25 per cent of all dollars spent by American consumers, is increasingly squeezed to provide expanded service with shrinking resources. NORD supports strong federal funding for these agencies, and our colleagues at EURORDIS do the same with the corresponding organisations in Europe.

Expanded newborn screening is another priority we share with our European partners. The newborn screening saves lives act, which is up for reauthorisation in the US congress this year, has been stalled in the senate even though the value of newborn screening - both to families and to society, since it reduces the cost of care in the long run - has been well established. NORD supports this legislation.

Patient access to diagnostics and therapies remains a priority for patient advocates on both continents. Of the approximately 7000 diseases classified as rare in the US and a similar number in Europe, only a few hundred have treatments approved by the FDA or the European medicines agency (EMA). For many patients, access to off-label treatment is an issue. Similarly, an accurate diagnosis for a rare disease takes far too long, and many patients don't have access to the testing they need.

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Patient advocates in the US and Europe work closely with academic and industry researchers, and are becoming increasingly sophisticated about how to contribute to the innovation process. These efforts include developing new tools to support research, and EURORDIS, NORD and the Canadian organisation for rare disorders (CORD) have published joint principles regarding patient registries. NORD recently launched a patient registry and natural history platform, described in a blog by Janet Woodcock, director of the FDA centre for drug evaluation and research. Our first international databank is with the von Hippel Lindau alliance, and we are working with other patient organisations to help them implement longitudinal natural history studies on the platform.

Maintaining the integrity of orphan drug legislation is another priority that is shared by EU and US advocates. At NORD's breakthrough summit, congressman Henry Waxman, the primary author of the US orphan drug act (ODA), gave one of the final speeches of his congressional career. Representative Waxman spoke about the importance of protecting the ODA's incentives - and with good reason, since a tax reform proposal floated in congress last year would eliminate the orphan drug tax credit, an important ODA incentive. Patients in the US and EU must remain vigilant to protect the legislation we've worked so hard to bring to fruition.

About the author

Pamela Gavin is chief operating officer of the US national organisation for rare disorders (NORD).

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