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O'Donnell **Subject:** EU struggles to meet ambitions for rare disease research

EU struggles to meet ambitions for rare disease research

-- By Peter O'Donnell 2/22/16, 6:00 AM CET

An ambitious plan to make better use of Europe's scarce facilities to research and treat rare diseases is taking longer than anticipated to get off the ground, as political sensitivities threaten to get in the way of science.

EU advocates of the scheme are redoubling their efforts to reassure nervous patients, physicians and member countries that their individual interests will not be ignored. Disputes include those over criteria for joining networks, concerns from some patients about neglect, and tension between state institutions over who will dominate governance of the scheme.

Clustering specialized research centers to pool talent has been under discussion for more than 10 years and the EU's directive on cross-border health care formally proposed creating [European Research Networks](#) back in 2011.

Mid-2016 was initially foreseen as the date for patients with rare diseases to start benefiting from the scheme, which should ease their access to treatment, at the same time as sharpening specialized EU research by pooling expertise from around Europe.

Now invitations to research centers to apply to join the scheme are scheduled to go out only in mid-March, and the earliest kick-off for has been consequently pushed back towards the end of this year.

Above all, concerns about exclusion are apparent at the national level in countries with less developed medical research structures. So a delicate balance has to be found between requiring scientific excellence from network participants and ensuring wide buy-in. ERNs are "the most ambitious health care project of recent decades," said Arimantas Tamasauskas, director of the Neuroscience Institute at Lithuania's University of Health Sciences. "It might be difficult to reach consensus at EU level because of different expectations, different levels of preparedness, and different legal aspects of health care systems across the member states."

He warned that ways must be found to attract smaller countries to participate actively or they may not make the cut when decisions are made on who can be a network member.

"Otherwise there is a danger that nine countries, almost one third of the member states, could be eliminated," he said.

Ruth Ladenstein, a pediatric cancer doctor in Vienna, is familiar with the sensitivities about being either in or out.

She runs a pilot European network in rarer aspects of child oncology, and sees ERNs as "a brilliant initiative" that could help solve "the big problem of where to find medical experience and therapeutic options" for patients suffering from

rare diseases.

But she knows that in groups that have been working together for decades, some doctors fear loss of recognition of their expertise if they — or their particular areas of specialization — are not selected as one of the new networks.

Securing the engagement of member states and the broader research community is central to the plan, because the essential objective is to provide better care to patients across Europe.

So a delicate balance has to be found between requiring scientific excellence from network participants and ensuring wide buy-in. That means breaking with traditions of inclusiveness, something that can easily provoke discontent.

Estimates of the set-up and initial running costs are around the €10 million mark.

Small firms fret

While many leading European medical societies are enthusiastic, the diversity of this field — there are 6,000-8,000 rare diseases identified and as many as 30 million sufferers in Europe — makes smaller research centers nervous that the drive to consolidate will lock them out.

The ERN approach of pulling together expertise from across previously separate disciplines and countries will require a new degree of trust among clinicians and hospitals accustomed to familiar work patterns in research and care. Patients with extremely rare diseases fear that attention to their concerns may suffer if already-scarce resources are refocused. Privately, many specialists admit that they work in silos, and are comfortable doing so.

The threat to the old way of doing things exists within as well as across borders. One advocate of ERNs argues, “Spain has 40 hospitals treating children with cancer — but only seven are needed.”

Patients: care v. research

Support groups for patients with rare diseases are also nervous, in part because they fear the new scheme may give more priority to research that is useful in the long term, over care that patients need right now.

And some of them see risks in streamlining centers into just a few networks, each with a major theme such as cancer or heart disease. Patients with extremely rare diseases fear that attention to their concerns may suffer if already-scarce resources are refocused at European level on these broader disease groupings.

Colin Pavelin, Head of Regenerative Medicine and Rare Diseases Policy in the U.K. Department of Health, has publicly raised warnings about inattention to innovation.

But he told POLITICO this week that pressure has been exerted to ensure research is clearly included in ERN objectives, “and I am a lot more confident that the balance is now about right.”

Practical issues

Many of the reasons for the delay are technical.

Obvious questions are whether patients should travel to see doctors in another country, or doctors move to patients, which has big implications for organization

and funding, because cross-border reimbursement of non-emergency care is still in its infancy in Europe.

Member countries even differ over the way they classify diseases, making cooperation still more complicated. An outline agreement has been reached on a list of about 20 rare disease themes. Sophisticated computer-based communications will also be needed if virtual clinical care and high definition images are to cut the need to travel, and if e-health tools are to exchange and consolidate scarce patient data, again implying high costs.

Some money may be forthcoming under the EU's Connecting Europe Facility for building the IT infrastructure, and from the amended EU health program for operations — with official announcements expected within weeks.

But strong national engagement will still be necessary for purely financial reasons, since there is little new EU funding for the networks, and their sustainability will therefore depend largely on support from national research resources.

Other questions present further political challenges: who should judge applications to set up or to join a network, and how to guarantee impartiality in those decisions? Or what sort of associated participation in a network can be offered without creating a sense of first- and second-class membership? Even agreeing the format and mechanics for setting up a network triggers divergences of view: is it better to start small with a narrow focus and scale up, or to begin with a broad umbrella overarching several sub-networks? Will trying to cover all aspects of a broad disease group (such as cancers) in a single network prove too diverse and cumbersome?

As part of its bid to overcome these concerns, the board that has been set up to manage the ERN initiative is working on clearer criteria and conditions that any network must meet.

“Quality and mutual recognition of all member states is of utmost importance,” the board said in a position agreed last month, and “the process must be equal, fair and robust in order to encourage applications from all levels of interest.”

PwC, the management consultancy, has also been brought in to review key aspects of the plan through a consultation process. Starting from the premise that ERNs could be of value across a broad range of services — from patient recruitment and referral to treatment monitoring, and from defining research areas to training and development of clinical guidelines — it is assessing which activities stakeholders consider as priorities.

An outline agreement has been reached on a list of about 20 rare disease themes, including immunology, bones, cancers, heart, eye, gastrointestinal, lungs, or liver.

Yann Le Cam, chief executive officer of the European Organisation for Rare Diseases, Eurordis, believes that this grouping into broad themes is necessary, and Eurordis is currently restructuring its own wide membership into similar thematic groupings in response.

Meanwhile, the logic of creating ERNs remains implacable, according to

Karleen De Rijcke, president of Cystic Fibrosis Europe.

For example, she says that at present cystic fibrosis patients in Romania are seriously under-served with specialist care. The few centers that exist are only for children, not for adult patients, and the treatment team is in many cases just a pediatrician – with no support from the gastroenterologists, physiotherapists, psychologists, or nutritionists that patients need.

To view online: <http://www.politico.eu/pro/eu-struggles-to-get-ambitious-rare-disease-research-project-off-the-ground/>

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