

Greater EU collaboration needed to combat rare diseases

Written by Cristian-Silviu Buşoi on 3 November 2014 in Opinion
Opinion

Clinical trials need to be carried out at European level to maximise their effectiveness, argues Cristian-Silviu Buşoi.



Rare diseases, characterised by their low prevalence, affect between six and eight per cent of the population in the EU, representing between 27 and 36 million people. The challenges posed by rare diseases, including a limited number of patients, scarcity of relevant knowledge and expertise, and the lack of specific health policies for rare diseases translates into delayed diagnosis and difficult access to treatments and care - despite the fact that some rare diseases are compatible with a normal life if diagnosed early enough and properly managed.

Research on rare diseases has focused on understanding the mechanisms of common conditions such as obesity and diabetes, as they often represent a model of dysfunction of a single biological pathway.

Given the fact that many rare diseases affect only a few thousand or even fewer than one hundred patients in the EU, one of the biggest challenges in treating rare diseases are clinical trials. Under

current circumstances it can be difficult to effectively conduct, analyse, and interpret studies enrolling several hundred patients with rare conditions.

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At EU level, the commission has already taken concrete steps to address the issue of rare diseases, to encourage more clinical trials and to make it easier to introduce new medicines, without compromising the safety of patients. Since the 1990s at both EU and member state level, national strategies and different initiatives have emerged.

There are currently a few key policy documents establishing a political framework for action in the field of rare diseases and orphan medicinal products at European level; the orphan medicinal product regulation adopted in 1999, the commission communication on rare diseases: Europe's challenge (2008), the council recommendation on an action in the field of rare diseases (2009), the application of patients' rights in a cross-border healthcare directive, and the clinical trials regulation adopted this spring, on which I was rapporteur for the internal market and consumer protection committee.

Despite all these efforts, there is still much to be done. For example, not all member states currently ensure full access to each authorised orphan medicinal product that has been approved, or if a particular treatment is not reimbursed in the patient's home country, it will not be reimbursed if accessed in another member state, even if this treatment could cure them.

The new clinical trials regulation, which is currently being enforced by the EU and member states, has at its core the need to improve conditions for carrying out clinical trials for rare diseases. This involves streamlining the approval of multi-country trials, while safeguarding the safety of patients and the robustness of scientific data.

"To tackle these barriers I strongly support a multi-centre collaboration at EU level to encourage partnerships with patient organisations and making use of funding research through programmes like Horizon 2020, with dedicated calls for rare diseases."

Even though steps were made, there are still challenges that rare disease clinical trials face. These include an incomplete understanding of natural history to inform trial design, the limited number of patients available can make it difficult to achieve statistical relevance, the need for more sensitive outcome measures to quantify the disease, and limited access to resources required to mount a clinical trial - patients and their families often need to travel to trial sites and stay there for weeks and months.

To tackle these barriers I strongly support a multi-centre collaboration at EU level to encourage partnerships with patient organisations and making use of funding research through programmes like Horizon 2020, with dedicated calls for rare diseases. Therefore, EU initiatives, such as the European reference networks, should further help with the creation of pan-European collaboration between member states.

The focus should be on making information available to patients and their families, supporting patients and their families, and ensuring that both public and private resources are used effectively.

About the author

Cristian-Silviu Buşoi was parliament's internal market and consumer protection committee opinion rapporteur on clinical trials on medicinal products for human use.

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