



The creation of a network of Reference Centres is a prerequisite for improving the diagnosis and treatment of rare diseases

PharmaMar and FEDER organise a panel of experts entitled "Reference Centres and Care Quality in Rare Diseases"

Madrid, 24 February 2016.- It is vital to create a network of Reference Centres for rare diseases, at national and European level, in order to address the main problems facing persons who suffer from these pathologies—three million people in Spain alone. This was one of the main conclusions of the panel of experts entitled **"Reference Centres and Care Quality in Rare Diseases"** which was organised by PharmaMar in cooperation with FEDER, the Spanish Federation of Rare Diseases.

Participants included **Ms. Maravillas Izquierdo**, Deputy General Manager of the Basic Healthcare Portfolio at the Spain's National Health System and the Cohesion Fund under the Ministry of Health, Social Services and Equality; **Dr. Ricardo Cubedo**, an oncologist at Hospital Universitario Puerta de Hierro in Madrid; **Mr. Santiago de la Riva**, member of the Board of FEDER, and **Ms. Sara Ortiz**, Head of Projects at PharmaMar's Regulatory Affairs Department.

The specialists in attendance agreed that delays in diagnosis and the lack of access to care and treatment facilities are the main obstacles in managing these pathologies. This is the conclusion of the ENSERio¹ survey, which indicates that one out of five persons affected by rare diseases experienced a delay of 10 or more years in the diagnosis, and that 62.28% experienced a delay of over one year. The survey also revealed that 42.68% of patients with rare diseases state that they are not receiving treatment or that the treatment is inappropriate.

¹ https://www.enfermedades-raras.org/images/stories/documentos/Estudio_ENSERio.pdf

Santiago de la Riva said that FEDER sees networks as fundamental for overcoming these problems with access to diagnosis and treatment. For that reason, he said, the campaign to mark World Rare Disease Day, on 29 February, will have the slogan "Creating Networks of Hope". *"Coordination is an urgent need and 2016 will be a key year in this connection, since the European Commission plans to call on member states to present candidates for European Reference Networks. This initiative seeks to establish networks of centres of excellence which, through advanced knowledge, techniques and multidisciplinary teams, share existing knowledge about rare diseases and provide healthcare to patients,"* he added.

In this line, Dr. Ricardo Cubedo, whose talk focused on functional units in oncology, with sarcoma as an example, said that reference centres are useful for ensuring proper treatment once patients are referred, but they do nothing to guarantee early referral. *"I don't believe there are many effective routes for early diagnosis of a rare disease. In Spain, it would appear that the most backlogs build up not at the level of the patients themselves or their primary care physicians but at the medical specialists. For example, sarcoma patients are generally referred to surgeons, who are not familiar with the disease. That delays the diagnosis,"* he emphasised.

He added that, apart from Italy and Scandinavia, European countries share the same difficulties with regard to the initial diagnosis of sarcoma. This situation is aggravated in Spain by the fragmentation between autonomous regions and the barriers to access to treatment. *"In Spain, the administrative barriers between treatment in different autonomous regions greatly hampers referrals to specialised centres and, in some cases, the access to treatment,"* he said.

In this respect, the FEDER representative noted that the European Commission's Group of Experts establishes 21 groups around which European rare disease networks should be organised. He said that Spain has sufficient accredited groups to participate in at least 50% of them. *"Europe has stated that these Rare Disease Reference Centres should coordinate the resources available in all the autonomous regions,"* he added. *"But that does not occur because most of the centres are located in Madrid and Catalonia and there is no coordination between those two regions, with the result that 80% of Spain is excluded."*



Coordination between all parties involved

The panel discussion also highlighted that coordination should not be confined to the care network but should also extend to all sectors involved in combating rare diseases: healthcare professionals, scientific associations, researchers and patient associations. *"We call on scientific associations, professionals, researchers and specialists to share knowledge and to work to ensure that experience can transcend the borders between autonomous regions,"* said Santiago de la Riva.

Dr. Cubedo concurred, stating that access to diagnosis and treatment of rare diseases also requires an enhanced role for patients' associations with a well structured presence in the internet and social media.

Access to the orphan drug market

During her presentation, in which she focused on the research and development of new drugs, Sara Ortiz said that although the spirit of European legislation has favoured research into rare diseases and, therefore, the development of orphan drugs, much work remains to be done. *"More knowledge of these diseases is essential, through creating patient data bases, in order to allow more specific guides to be developed by the regulatory authorities. Greater understanding of these pathologies will make it easier to design and target clinical research in the development of new orphan drugs,"* she stated.

Ortiz added that, although a single orphan drug designation is valid for the entire EU, the main obstacle is in accessing the markets, each of which is subject to the individual member state's regulations. *"In Spain, accessing the market requires meeting the requirements not just of the central government but also those of the autonomous regions. For that reason, it is important that the Administration takes steps to reduce barriers to market access,"* she concluded.

About PharmaMar

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