Maria Jose Sanchez Losada – President, AELMHU, Spain

The main challenge for orphan drugs in Spain is access, which has become highly complex in recent years

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The Spanish Association of Orphan and Ultra Orphan Medicinal Products Laboratories (AELMHU) is a non-profit organisation working to improve awareness about rare diseases and access of Orphan Drugs. Maria Jose Sanchez Losada, president of the association, comments on the current status of diagnosis, access and reimbursement of rare diseases and orphan drugs in Spain.

With over two decades of experience in the pharmaceutical industry, how different is it to work with rare diseases and how did you get involved with AELMHU?

Working in a highly innovative and specialized therapeutic field with a focus on rare diseases requires you to have an entrepreneurial spirit, thinking outside the box to bring different perspectives; it is more trailblazing than following a familiar path, which is, of course challenging in a highly regulated market. It brings also a personal and professional additional reward as you see very closely the impact of your job improving people's lives.

The Spanish Association of Orphan and Ultra Orphan Medicinal Products Laboratories (AELMHU) was started precisely because we needed to share our issues, looking for different ways of doing things, supporting each other at a moment when European countries started having a big interest in supporting and regulating orphan drugs.

I first interacted with AELMHU after joining CSL Behring five years ago and I am part of the board now for over a year and a half. We have defined a clear strategy and objectives for AELMHU,

working very hard to build a community of companies with shared values. We can say that AELMHU has become the reference organization in the Pharmaceutical Industry in Spain for Orphan Drugs. Our members face similar issues but, above all, a commitment to help patients and improve timely access to our innovative therapies for them. All the companies that are part of AELMHU are mostly dedicated to the research, development, and commercialisation of Orphan Drugs. Of course, that last step – commercialisation – is where we now focus because the effort does not pay off unless patients see the benefit.

According to recent data, there are 175 products with orphan drug designation in Europe, of which 125 have received marketing authorization. In Spain, approximately eight orphan drugs have received reimbursement approval this year. Can you comment on the status of orphan drugs in Spain?

The main challenge for orphan drugs in Spain is access, which has become highly complex in recent years. Today, about 60 percent of orphan drugs with a commercial authorisation are not reimbursed in Spain. Moreover, those reimbursed took an average of 27 months. That means that, in addition to the many years it takes to develop, research and receive EMA approval, orphan drugs in Spain must wait over two additional years on average for patients to gain access.

We understand that the COVID-19 pandemic has made things difficult, and the Ministry of Health (MoH) had different priorities, but compared to other European countries, Spain could do much more. That is what AELMHU is trying to communicate and explain to different stakeholders.

Our association is working to create spaces of discussion, serving as a bridge between innovators and different authorities so they can find common ground and improve access for patients in need. We have shared with the MoH several concrete areas where we can improve the reimbursement process; and of course, we believe the pharmaceutical industry must be a key stakeholder in the process.

There must be a transparent process in which the authorities agree with the industry on a clear pathway, fixing a set of requirements and timelines upfront that companies and administration must meet during the development and reimbursement process. Open dialogue is very important because the development of orphan drugs is rarely a straight line.

According to recent figures, there are approximately 3 million patients and families affected by rare diseases in Spain. How big of a challenge are we talking about?

The 3 million figure is a statistical projection since many patients have not been diagnosed. Nevertheless, it is a high number and a big challenge; there is plenty of work to be done, starting from improving diagnosis, of course. We must keep in mind that once patients have access to the treatment, it is often a game-changing personal solution, a therapy that greatly improves their quality of life and their families'.

As you stated, diagnosis is often one of the main challenges for rare disease patients. However, the situation has improved significantly around the world with the advancement of genomic technology since many rare diseases are due to gene mutations. What is the diagnosis situation in Spain?

Spain's public health system is at a very high level within Europe, offering a great quality of care to the population. Spain's physicians, pharmacists, nurses, and overall personnel have a high level of experience, training and resources.

Our system is ready to keep improving in the diagnosis and treatment of rare diseases. Fortunately, there is already a deep understanding of many rare diseases and, the more we continue learning, the better diagnosis will be.

Tony Zbeidy from Recordati Rare Diseases in the Middle East and North Africa recently told PharmaBoardroom that Saudi Arabia is currently screening newborns for more than 16 rare disorders whereas European countries are doing it for only three or four. Why is that the case and how can Spain improve screening for newborns?

Our Ministry of Health has recommendations for screening newborns for different diseases. There are now patient associations requesting to include further tests for rare diseases as the earlier you can diagnose you can prevent further disabling consequences or even disease development. I believe that we should continue pushing in that direction.

Are there any projects or activities being conducted by AELMHU that you would like to highlight?

At the moment, AELMHU is focusing on putting data on the table regarding access. We are trying to communicate the value of orphan drugs, which bring key innovation to patients and it comes as a cost since they are commercialized for a small group of people, and there is a long and arduous process behind all of them, not to mention the invaluable benefit that they provide to patients and families.

There must always be a balance when deicing on ways to finance these therapies, understanding how innovation in orphan drugs can be supported.

On a personal note, is there a specific disease or situation that has moved you after many years of working in rare diseases?

I started working in rare diseases with spinal muscular atrophy (SMA), for which there were no treatments available at the time, but today there are at least three therapies in the market. Without naming brands or companies, it is a great example of how innovation from the pharmaceutical industry has been able to provide advanced treatments for patients. I remember discussing with families about the disease and how much we all learned from them on the disease affecting their loved ones but had not treatment options. They fought hard to provide something for their kids that could at least stop the disease from progressing.

Orphan drugs change lives in a profound way, it is why we work hard every day, why we work so many hours in the day.

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