Interview: Alba Ancochea â?? Director, FEDER, Spain

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Alba Ancochea of FEDER, the association that groups together Spainâ??s 337 rare disease patient organizations, explains how it interacts with both government and industry to the benefit of rare disease patients, the evolving situation for these patients in Spain, and her strategy for the future.

Could you please start by introducing FEDER?

FEDER is made up of 337 rare disease patient organizations here in Spain and was born 20 years ago, although we still feel that we are relatively young in that we try to maintain a vibrant, start-up style, resourceful mentality. The organization has developed very much in line with the European Alliance of Rare Diseases (EURORDIS) where we have always been represented on the board of directors. We are really proud of that fact and make great use of the guidance delivered to member states and national alliances. There is a council of national alliances within EURORDIS and we all work together to achieve the same goals in different countries, adapting our strategies to fit the local context accordingly.

There is a European Commission directive on national strategies for rare diseases, and we try to implement all those recommendations and measure them against the European project. Every couple years we also participate in a Europlan conference (A tool to measure the fulfilling of EU recommendations on RDs).

Right now, we are invited to participate by the Ministry of Health in different working groups and committees. Our input is also taken into consideration in the national strategy monitoring committee. Also, we sit on a working group with the Spanish Medicine Agency.

How would you characterize your relationship with the Spanish authorities?

It depends very much on the specific area. For example, we were admitted as a member to the working group relating to the royal decree of the registry, which signified a big step forward. Nowadays we have a seat at the table as a matter of course and we have established what I think can be described as a productive working relationship with the Ministry of Health.

When it comes to pricing however, we are not present. A multi-departmental committee has been established with a view to fixing prices, but we have not been invited to attend those sessions and the voice of patients is not heard at all. We believe this is a mistake. Our feeling is that patients, industry and practitioners should all be consulted on such an important matter. By including these types of stakeholders would surely lead to a much more transparent and efficient pricing decisions.

There are other ways in which we can potentially influence the policy agenda. Presently, we have been approved by the Inter-territorial Committee, which is a task force approved to assist in the study of the impact of rare disease pharmaceutical products. The plan was to establish a committee built by different stakeholders, and the patients were going to be represented by FEDER. We hope this initiative is going to go ahead, but because there are changes in the government, we are not completely sure at this moment in time.

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How would you describe your relationship and interactions with industry?

Some of the big companies sponsor FEDER for the roll out of specific projects and activities, but they also work as an assessor for FEDER. We manage our relationship with industry primarily through the *AsociaciÃ³n EspaÃ±ola de Laboratorios de Medicamentos HuÃ©rfanos y Ultra HuÃ©rfanos* (AELMHU). That way we ensure that there are no potential conflicts of interest and that we can guarantee transparency and good government within FEDER. That is necessary because we do not represent one disease, or one specific treatment. There are no compliance issues in FEDER because we represent 7,000 diseases. We also have a kind of ethical code that all the industry sponsors have collaborated in following. All of them signed this ethical agreement. As a result, there are two ways to work with the industry: as assessors, through AELMHU, and also as sponsors of our different projects. Every donation goes with the project, and with the agreement, everything is clear.

Can you tell us about your capabilities?

We distribute our action across three pillars: our actions for patients, for patient organizations, and for society. It is because we pursue a healthcare system ready to diagnose and treat rare diseases, to give healthcare attention to rare diseases. But we also think that through this process to get an ideal system, we should offer services.

We have established a Help Line through which we have a huge database of more than 30,000 patients. We connect patients, so they can exchange their knowledge, not just to help manage the disease, but also experts with expertise and resources. It is through this help line we have two lawyers who can give legal advice and also five psychologists who deliver mental support. Our main job is with social workers, from whom we can deliver information about resources and social benefits.

Also, we support getting a diagnosis through our expert committee, which comprises 28 experts from different health fields. They support us, and at the same time our patients, to get a diagnosis. We have an agreement with the main research institutions in Spain â?? Rare Diseases Research Institute and the Biomedical Consortium Network. We are part of their external scientific advisoty committee, where we represent patients. We also have an agreement in place with them for referring patients to their non-diagnosis program because they invest some funds from research to support the diagnosis.

Concerning our service to patients, we work, above all, for patients that have no personal organization in reference. For the 337 associations, we empower their services by giving them economic support. Also training. Because we want them to be empowered to represent their needs

in the different areas with the authorities, but also with industry and for research.

Where does your funding come from and how does it get spent?

50 percent is from the government, through call of projects, with 70 percent at the national level and 30 percent at the regional level. That is because there are different goals at the national and regional levels, regarding social concerns rather than department. Then 50 percent is private funding â?? more or less 10 percent is individual donations, 30 percent industry, and the other is different such as with banks, etc. More or less 25 percent of our budget then gets channeled towards supporting our patient organizational programs and our associations. Also, we support research projects through our associations. This year we have EUR 100,000 funding for research. We are going to give five grants.

Are you able to tap into any European Union funds?

Not now. But we will certainly be looking at that sourcing channel in the future.

Tell us about your organizational structure. We understand you possess a federation, a foundation and an observatory.

The foundation is the mechanism we use for research funding. So the funds we refer to the foundation go straight to funding research projects. There are no other expenses in the foundation. The observatory involves social research in the form of surveys. We had 1,500 respond to our most recent survey on access to diagnostics and treatment with interesting results. We discovered some 50 percent of known rare disease patients didnâ??t get diagnosed for more than ten years. While a further 20 percent required between four and eight years before they received a proper diagnosis. The average in getting a diagnosis is about 4 years. That demonstrates that there are still shortfalls in the way that Spain is caring for its patients.

It wasnâ??t easy to get definitive conclusions, but we are now contrasting these figures with clinical data so as to get better insights. From 100 clinic histories, we found that it took, on average five years to achieve a proper diagnosis. We want to increase the sample to 1,000, but to date it has just been 100 in Madrid. Now we are going to replicate this same test in different regions, so we can demonstrate that it is not just our perception, but hardcore clinical data that demonstrates five years is a standard average time for getting a rare disease diagnosis.

How many people suffer from rare diseases in Spain?

Around three million, although that is just an estimation, as it is supposed to be seven percent of the population. There is no definitive data. We are still setting up the registry of National System of Health, but it is not concluded yet. The main difficulties we have right now with the registry is that there are 17 registries. We are trying to collect all the data into a single registry. That is not easy because the source of the data and the evidences are different depending on the regions. The diagnosis criteria are not the same and the data sources vary. Therefore, the Registry Committee is working right now on just ten of the most widespread rare diseases.

You have a wide visibility at the European level, how is Spain doing relative to other countries?

In terms of access, just 56 percent of the treatment and drugs approved at the European level are marketed in Spain. That is at the national level, but then at the regional level, not all people get access to the treatment. In fact, our main challenge is the equity among the regions and again standardization across member states in Europe. That is why we trust in common regulation at the

European level in terms of prices and marketing. Right now, just because a drug is authorized at the EMA, that doesnâ??t mean it is going to be marketed in Spain. And there is an average of one and a half years between it getting authorized at the EMA and it being marketed in Spain. That is because negotiations are very hard because of budgets and the economic situation in Spain is not the same as Germany, for example.

Also, the Ministry of Health is not confident fixing the price at the national level because then they have to scale the price at the regional level. And it is not the same for a hospital in Madrid and a huge hospital to get an agreement with industry, because bigger hospitals buy more products and can get a better price.

We thought that a good initiative could be to not just purchase products at the national level but also align through the Centers of Expertise. Because they could know much better the potential candidates and the potential users of the treatment. In terms of the Centers of Expertise, Spain is in a good position because, as with France, we already have a real decree that identifies and certifies Centers of Expertise and we have good criteria. But our participation in the European Reference Network was low. Unfortunately, some countries endorse every hospital with quality criteria already set. It is not fair the way they endorse hospitals to be in the European reference Network.

The way regions work with a Center of Expertise is that the hospital gives the funds in advance to cover the treatment but then when the state reimburses the money it is not to the hospital, it is to the region. So sometimes hospitals are not interested in being Centers of Expertise because it is a deficit financially. Sometimes Centers of Expertise are recognized not so much in terms of quality or expertise but in terms of political interests.

You alluded to the discrepancy between approvals at the EMA level and nationally. What do you put that discrepancy down to?

Personally, I think it is because of the decentralized system. It is so difficult to make a decision because they have to take into consideration 17 different opinions and they want to avoid discussions. It is very difficult in terms of rare diseases, in the minority, to fund the treatment of 17 different health systems. With a common global system, it would be much easier.

What is the way out of that? And how optimistic are you with the new government that things are going to get better?

We think the solution is with a regulatory framework at the European level. We think building a regulatory framework, not a directive, not a recommendation. And we should be optimistic with the new government.

What is the record of Carmen MontÃ³n, Spainâ??s new minister of health, on rare diseases?

We hold Carmen MontÃ³n in high esteem because of our positive experiences of working with her in the past. At the Valencia administrative level, she distinguished herself by promoting a network on research on rare diseases. It was with a legal framework and official networking research for rare diseases. She also created a committee for monitoring the main action of the strategy for rare diseases at regional level. Because we have a national strategy and then seven regional strategies. Sometimes it is not enough with a national strategy and they need to adapt to their own characteristics. She also supported us by coming to our events. We had a couple of interviews with her and she was supportive.

She is a doctor and that is also very meaningful because she is closer to patients. When the minister is a doctor it is a real advantage for us as they understand our needs better. At least now we

donâ??t have to start from the beginning again, because each time the minister changes we have to explain to them what a rare disease is.

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The change we need is that we cannot work with 17 budgets to face rare diseases issues. We need a common budget for funding a center of expertise, we need to be referred with no boundaries, and to have collaboration among the 17 regions because the main hospitals are in five regions. We also need a genetic and genomic plan. There was a commitment to build that plan, but it has not been implemented yet. But we also know that the new government is interested in that initiative because they were one of the encouragers of it.

Last year a kind of pilot genetic program was released for diagnosing rare diseases, but it failed because the funds were supposed to go to three main hospitals, so we could join efforts on diagnosis but at the end it was distributed to 17 projects in 17 regions. It was impossible. We need to work in one centralized system like the French, in rare diseases at least. We also know it is not possible unless there is an international or a European initiative.

What is your vision for FEDER and rare diseases in the next three to five years?

We should work at a much more international level and try to be a stronger part of the rare diseases international movement. Right now, we are in the advocacy group, the advocacy committee of Rare Diseases International. We thought Spain could be a good facilitator in including rare diseases in the World health organization program. In fact, we got the commitment of the government before to support us in that challenge. Spain has good practices to show other countries and we could be an interesting country to lead this initiative.

Here in Spain we should consolidate our network because many of the patient organizations serve the same diseases, at regional level and at national level. By working together, we think we can pool our resources and create a better network. FEDER aims to achieve Patient Organization shared goals by increasing the support to its membersâ?? advocacy and strengthening the cohesion of the rare diseases community.

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