David López García – Country Manager, BioMarin Mexico

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David López García, Mexico country manager of global rare genetic therapy player Biomarin, outlines the challenging situation around patient access to orphan drugs in Mexico and the importance of building awareness of rare diseases.

David, you have been the GM of BioMarin Mexico since 2012 but in 2020 we see a new situation in the public healthcare sector. Could you share your perspective on the ongoing healthcare reforms?

When you have a new government in the country – globally – there will be questions about the new political environment and policies. This is also the case in Mexico, which is natural. Understandably, there have been some concerns because radical changes have occurred not only in our government but also in our healthcare system.

One of the main concerns relates to the new federal healthcare institution, INSABI (*Instituto de Salud para el Bienestar*), specifically how efficient it would be compared to the previous institution, Seguro Popular. The government wants to centralize some procurement decisions relating to medicines and medical supplies to reduce or prevent the misallocation of federal healthcare funds. In theory, this does make sense.

I have been working in the healthcare industry in Mexico for 20 years, so I have witnessed the establishment and end of Seguro Popular in 2003 and 2019 respectively. While the idea behind

Seguro Popular to cover the healthcare expenses of Mexicans, particularly relating to catastrophic diseases, was good, there were problems with the day-to-day implementation. There was a significant lack of resources so eventually, some thresholds and limitations were put in place to limit healthcare spending, effectively prioritizing some patient groups over others.

The purpose of INSABI seems to be to rectify this and provide universal healthcare coverage to Mexicans, which is mandated by our country's Constitution. My impression is that this would remove the limits and thresholds previously put in place, which is good for both patients and industry.

The problem with INSABI is that it was still not ready when it was launched on 1 January 2020. There were no rules, plans or resourcing procedures put in place for its operations. As a result, there has been – and still is – a lot of uncertainty regarding INSABI. This has had some negative repercussions for patients. For instance, for one of our products, patients need to receive weekly infusions. Due to the transition, there has been some confusion surrounding the procurement and payment of therapies in hospitals and as a result, roughly half of our patients we have on this therapy have missed up to eight or nine infusions. Other companies have similar stories. This is a rather complex situation.

How do you expect these changes to affect BioMarin's operations in Mexico in the near future?

As a global leader in rare genetic therapies, we typically work with public payers in each country since orphan drugs typically require special funding. This is also the case in Mexico, where we are only present in the public sector. In Mexico, we currently have two products available on the market and included in the national formulary (previously called the *Cuadro Básico* and now called the *Compendio*). Two more products are in the process of regulatory approval.

The challenge for orphan drugs is the price. It is not always easy to explain to the regulatory authorities that the value of such therapies should not be assessed in the short term but rather evaluated over the long term. This applies not only to orphan drugs but also to the new innovations and technology being developed today, like gene therapies, where BioMarin is also one of the leaders. Without a longer-term perspective of the therapeutic benefits of such therapies, it is true that there is often a sort of 'sticker shock' when healthcare authorities first encounter these new therapies and their associated costs.

However, the mindsets of key decision-makers in Mexico are slowly changing. We have worked with various authorities including the National Health Council (*Consejo del Salubridad General CSG*), which specializes in health technology assessments (HTAs). Products usually have to be approved by the CSG in order to be included in the national formulary. With the new *Compendio*, I believe that there would be more opportunities for BioMarin to communicate the value of our products. Actually, in terms of HTA, Mexico has always been one step ahead of the rest of Latin America in the past decade. Our assessment regime used to be very close to the UK NICE Institute.

The National Health Council has also shown an openness to work on risk-sharing agreements, for instance. In addition, I believe that after a transitional learning period, INSABI will also be able to understand the best way to evaluate such therapies. Globally for BioMarin, we have collaborated on risk-sharing agreements in countries like Canada, the UK and Australia so we are keen to bring these models to Mexico as well. Last year, the Mexican innovative pharmaceutical association, AMIIF, has been very active to discuss such models with different public payers in Mexico but we are still waiting to see concrete action from the payers. But I think this is the only way to continue to receive new innovations and therapies.

That said, the environment in 2020 will be difficult because of the transition. There are newcomers and new actors within the system, and there is a learning curve. However, I believe that in a year or two, the system will work much better and all the stakeholders – including patients, doctors and industry players – will benefit.

Rare diseases can be a challenge for any healthcare system. How has the rare diseases environment in Mexico evolved over the past few years, especially in terms of disease awareness and access?

The awareness of rare diseases in Mexico has grown significantly. To illustrate, 2008 was the first year that the World Rare Diseases Day (29 February – or 28 February if it is not a leap year) was celebrated internationally. That year, there were only two events commemorating it in Mexico: in Mexico City and in Guadalajara. This year, there are already over 50 planned events across the country. Over the past three weeks, I had the opportunity to visit some legislators around the country and most of them told me that they were aware of rare diseases and in particular, mucopolysaccharidosis (MPS), which is an area of focus for BioMarin. This is progress.

However, awareness is not enough. The next step is access, which is still a key issue. I have already highlighted the challenges with the transition of Seguro Popular to INSABI. But a perennial

concern is the length of the regulatory timelines in Mexico. Research from IQVIA has found that from the day a company submits its dossier to COFEPRIS for approval to the day that the approved drug is available to all patients in the public system is around 4.5 years – for a normal drug. The process for an orphan drug is even longer. This is a big challenge for patients with rare diseases in Mexico.

Then we have the challenge of identifying and treating patients with rare diseases. Rare diseases are more commonly found in smaller communities rather than in big cities. At the same time, doctors do not receive enough information about rare diseases during their medical training. BioMarin actually invests in organizing around 75 medical meetings across the country with different hospitals and associations to educate healthcare professionals about rare diseases. In conjunction with patient associations and patient programs, we also provide the agreements with the testing laboratories to test patients for these diseases. We want to help increase the awareness of these diseases in the medical community in Mexico so that doctors can improve their abilities to diagnose these diseases.

Unfortunately, even after the patients have been identified, the pathways for them to access these therapies can be very long. For instance, for a new patient to access an approved product through IMSS (*Instituto Mexicano del Seguro Social*, one of the public payers), there are 154 steps for them to complete!

Another challenge is to keep the patients on therapy. This is partly due to the centralization of our healthcare services. Across the 32 states of Mexico, we only have 21 hospitals able to provide our therapy for Morquio syndrome (also known as MPS Type IV). We currently have 30-35 in therapy, and roughly half of them have to travel four to five hours each week to receive treatment. This can be difficult for them.

To treat rare diseases truly requires an orchestra of players and efforts: doctors and other healthcare professionals, patient support groups and patient associations, patients themselves and of course industry players like BioMarin.

On a final note, you have worked in the rare diseases area for two decades, including a few years with Genzyme before BioMarin. What excites you most?

I have had the full start-up experience with BioMarin in Mexico. I was the first official employee of BioMarin in Mexico and I have seen the affiliate grown from zero, receiving the approvals for two

products and building relationships with different authorities. Soon we will be moving to a larger office to accommodate our growth, which is another milestone for us.

At the same time, I have also seen the growth of BioMarin from a company initially focused on MPS to the company it is today, with a number of innovative therapies for other rare diseases like phenylketonuria (PKU) and CLN2, an ultra-rare and rapidly progressing pediatric brain disorder, as well as an emerging leadership in gene therapy. It has been a truly exciting journey and I continue to be very excited about leading BioMarin Mexico!

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