Interview: Carla Benedito – General Manager, Shire Portugal

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Carla Benedito, general manager of Shire Portugal, provides useful insight into the rare diseases landscape in Portugal. Carla discusses market access models for innovative and rare treatments, encouraging dialogue between industry and government, and the touching nature of working for a pharmaceutical company that knows all of its patients by name.

How is the Shire global portfolio reflected in Portugal?

Shire global has a portfolio spanning several areas: neuroscience, genetic diseases, internal medicine, hemophilia, immunology and in Portugal we cover genetic disorders and hemophilia. We look forward to launching products in oncology, where we enter the final stages of negotiations with INFARMED. In immunology, we have a significant offering in IV medicines, which we have already made available in some hospitals in Portugal country. Revestive®, a critically important medicine for patients suffering from short bowel syndrome should be made available by this summer. Revestive® is a life-changing drug. It enables patients to lead more or less normal lives, because it puts the small bowel which barely functioned previously, into an ultra-absorption mode.

Finally, although we do not have neuroscience scope in Portugal, Shire has struck up a strategic partnership with Bial, who will be responsible for the commercialization and the distribution of our product in Portugal. The launch is imminent, as they, like us approach the final bottleneck of approvals from INFARMED. This product will be fantastic for patients, as Portugal is of the few

markets in Europe that does not have access to this medicine.

Rare diseases affect up to 30 million EU citizens. What is the current situation in Portugal?

We estimate that roughly 600,000 patients in Portugal live with a rare disease and unfortunately much of these patients live for a long time without diagnosis—this is where Shire's efforts are critical, to facilitate more diagnosis. As we are aware, as high as 85 percent of rare diseases, do not have a treatment, which means that for those diseases that do have medications available, every month or year that passes without diagnosis and care is time lost in quality of life. It is urgent that we improve diagnosis of the rare diseases we can treat. Accordingly, Shire works in partnership with physicians, healthcare professionals, and the more comprehensive medical community to support better diagnosis.

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Up to 40 percent of patients are misdiagnosed across Portugal. What more can be done to remedy the situation?

We take our role very seriously as a member of the pharmaceutical industry, in bringing more information to physicians. Some of the diseases we encounter are incredibly rare and therefore outside the scope of knowledge of a regular doctor. A physician may not be aware of a condition that they face once in their lifetime— at which point Shire is on hand to assist. The classic example is the seven patients we see treated for Hunter's Disease in Portugal—an incredibly low number of patients for a disease in a country—which means that most physicians in Portugal will never see a Hunter patient across their entire medical careers. As a result, healthcare professionals may not, therefore, be able to adequately treat such a patient given their limited experience in such a rare area. Our job is to aid physicians in accessing the right information to enable rapid diagnosis and treatment, which accelerates the process of patients living more normal lives.

From a market access standpoint, what challenges do you face?

If we observe the APIFARMA statistics, the average approval time is 22 months. Almost two years is an extremely long time, and this is, therefore, one of our greatest challenges, especially at present when we want to bring as much of our portfolio to market. At Shire, we pride ourselves on innovation that we bring to patients, and therefore these delays in access have a direct impact on patients.

INFARMED has made clear their intention to reduce these times, and the President recently announced a directive to follow a 180-day limit, which maximizes at 210 days for negotiation of more complex innovative medicines. We are cautiously optimistic and hopeful that the new legislation will be a game-changer for Portugal, shifting the paradigm for patient care. However, the result remains to be seen—the statement landed last October, and we have seen few products so far that have met the timelines.

For a smaller company like Shire, delayed timelines make investment decisions more challenging. And above all; sadly, these delays mean that patients miss out on potentially life-changing treatments. This dramatic impact that new medicines can have for patients is the real concern at Shire, and it can be challenging to emphasize the urgency of our patients' needs to stakeholders. Early access programs are encouraging, but evidently, not all patients can take advantage. Ultimately, the delays disadvantage the patients the most.

Should rare disease treatments should be classed differently to innovative disease treatments?

Rare diseases should absolutely be costed differently to innovative products. From the get-go, rare diseases are complicated to diagnose, treat and even research. Clinical trials have a far smaller pool of patients, and by the very nature of being rare, there is less research and evidence to seek treatments. The EMA, however, treats rare diseases differently and although nine out of ten drugs are rejected for treating orphan diseases (because there is too little evidence), the one drug that passes the system makes a positive change.

In a country like Portugal, the rare medicine process is too broad. We have patients who wait for treatments for years and who recognize the drug's approval in Europe and are disheartened to see that they may have to wait up to two more years for the patient to be approved for administration in Portugal. There is no clear pathway for approval for rare diseases here, and we would welcome such a process. This is high on our agenda—to improve this pathway with policymakers, because naturally, the budget impact is smaller given that so few patients are on the list to be treated. We are taking tens of patients for rare disease products, not hundreds or thousands of more common diseases.

How open is the government to this kind of dialogue?

The government is always open to dialogue, but because of the constraints in the economy, there is a chronic underfunding of the healthcare system in Portugal which reflects directly on medicines. The government has several priorities as the economy exits a crisis period. Public sector workers

request salary increases, for example, and there are multiple financial pools the government must fill. For the health system, we know that there are high hospital debts and the budget is restricted.

If we consider the Portuguese healthcare system first hand, we see that we have some inherent issues causing delays. To explain, the UK medical system emphasizes clinical benefit: therefore, the drug must show an acceptable QALY, hence price vs. the quality of data. If the QALY is sufficient, the medicine is generally accepted. Other countries, such as Spain, emphasize budget impact: the government will approve a drug dependent on their finances at the time. In France and Germany, for example, the drug approval is outcome based: if a drug is effective it receives a 1 grade and can demand a higher price if the drug is of lower effectiveness it is graded 5 and has less bargaining power. Portugal, sadly, does all three practices at the same time: clinical assessment, QALY assessment and thirdly budget impact. INFARMED walks each medicine through each bucket of this process. This makes the entire approval procedure far more challenging, and barriers are present at every turn. The system is so squeezed and driven towards savings and obstacles that innovation is stifled.

We, therefore, must find an equilibrium in which the pharma industry can thrive, and the government can be sustainable. Discounting, negotiated caps, risk-sharing agreements and dialogue going forward will help to strike a balance.

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Patient care is at the crux of Shire's culture. How do you build relationships with patients and patient associations?

If there is one passion that Shire holds across the board, it is caring for patients. When wandering through any of our offices, we see photos and notes from patients. Although we do not know our patients physically, we have a level of proximity, even intimacy with our patients because we deal with such a select few, and we deal with every individual on a case by case basis. As part of our work, we engage and support patient associations to go further than merely prescribing medicines. Not only do we work with general associations, but associations specific to a disease type, such as the Hemophilic Association where we have an ongoing dialogue. Ultimately, for Shire what separates us from traditional Big Pharma companies is that patients' outcomes define our success—we have five patients in some cases, so success and failure are tangible.

What is it that inspires and motivates you to lead the Shire affiliate?

In a market as challenging and small as Portugal, being true to your values and being passionate about you do is key. Passion will be the driving force for when you are presented with challenges,

bad luck or delays and this combined with resilience (a vital attribute in Portugal!), will keep your head above ground. A General Manager’s role is purely a title—we lead by example to make a difference in patients’ lives. Personally, when there are so few patients that wait such a long to receive a medicine, and there are so few people defending their interests, the challenge becomes more meaningful. Fighting rare diseases is like leading a long crusade where every day presents challenges, yet being able to visibly take in the impact of our actions on patients’ lives is incredibly rewarding.

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