Stefan Joris â?? Director, Belgian Cystic Fibrosis Association (BCFA)

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Stefan Joris of the Belgian Cystic Fibrosis Association (BCFA) highlights the progress that has been made on cystic fibrosis screening of newborns and reimbursement of innovative modulator therapies in Belgium in the past few years. Joris also explains why the move to better integrate the patient voice into the countryâ??s drug reimbursement negotiations is a welcome one, and outlines his hopes for more interactions between patients, governmental stakeholders, and industry sponsors at all levels of the healthcare value chain.

What brought you to this position as Director of the Belgian Cystic Fibrosis Association (BCFA)?

I do not have a medical background and did not have a personal or emotional link to Cystic Fibrosis (CF) or any rare disease when I took up the position around seven years ago. I was already working in the non-profit sector helping other associations in areas such as management and IT. BCFA wanted someone who could come in and professionalise the association, so I was chosen.

I would say that at the start my emotional connection was lower due to my lack of personal link to the condition, but this has evolved over time and today I definitely feel a strong connection to the patients and families we deal with.

You came in around seven years ago to bring a professional touch to the association. How has the organisation evolved since then?

The association was founded in 1966 and has grown steadily over time. Today we participate in NIHDIâ??s CF convention, have set up a registry, and have seven reference centres across Belgium. We are one of the largest patient organisations in Belgium with 17 employees. We are responsible for communication and fundraising and last year raised over EUR four million for patient support. This is extremely important as we do not receive any public funding. It has been a long road, but we have been successful in running the group like a business and bringing a professional approach to our operations.

In the last few years, we have achieved a lot by bringing in mandatory newborn screening for CF, which commenced in the Flemish-speaking area of Flanders in 2019 and a year later in the French-speaking area of Wallonia. The first CF transmembrane conductance regulator (CFTR) modulator therapies were granted reimbursement in Belgium in 2016 and a number of others have achieved the same status since, with the most recent coming in 2022. Reimbursement was first granted for patients aged 12 and over, and a few months later for those aged between six and 11.

2022 was a great year for us and a tipping point for the life expectancy and quality of life of CF patients in Belgium, even though we know we have a long way still to go.

CF is far less prevalent than conditions such as cancer and cardiovascular disease, but you still represent one of Belgiumâ??s largest patient organisations. What is the reasoning behind this?

The first reason is that CF is a rare disease which the Belgian health service â?? like health services around the world â?? is not well set up for. Rare conditions involve a very small group of patients with multisystemic impacts that require a multidisciplinary approach and integrated care. The healthcare professionals and carers dealing with the patients must be in constant contact so they can adapt their care to each patient. For this interaction, we have been able to set up the aforementioned seven reference centres, and conventions and four times a year have a multidisciplinary patient consultation where the team can discuss the progress of patient treatment.

If you look at the Belgian rare disease association, RaDiOrg, it is made up of 86 patient groups and a lot of these associations are extremely small but must grow to build the voice of the patient and their role is of pivotal importance. In oncology, the main patient associations exist to generate funds and distribute them to different projects, with less need for patient support or direct financial aid than a group like ours.

What is the impact of CF on patients and what is the prevalence of the condition in Belgium?

CF is a genetic metabolic disease that impacts many organs in the body, but most heavily the lungs and digestive system. The transport of chloride in the body is inhibited which results in thick and sticky mucus that cannot be easily evacuated. As a result, it builds up in the lungs and causes inflammation and infections and the lung capacity decreases over time. Furthermore, the same build-up occurs in the digestive system as the pancreas is blocked and they cannot create the necessary enzymes. Therefore, patients must be given enzymes to digest the food, and in fact, the treatment

for a patient is usually between three to four hours a day, a part-time job.

The prevalence is still roughly similar to where it was a few years ago (between 2,500 and 3,500, making it one of the most prevalent rare diseases in the country) but the number of diagnosed CF patients has increased. This is due to longer life expectancy resulting from better care quality. Every year between 25 and 30 babies are born with CF in Belgium.

How would you characterise the reimbursement process in Belgium for cutting-edge CF therapies?

The process in Belgium is quite long, usually between 10 to 12 months, making it one of the longest waits in Europe. I believe this lengthy process comes from the required national-level health technology assessment (HTA) which takes around 6 months but is often merely a reproduction of the work that has already been done at the European level. Leading up to negotiations with the Ministry of Health cabinet on pricing, they require a 30, 60, 120 and 180 day report. The move towards a joint European HTA process to speed things up â?? which looks set to come online in 2025 â?? would be very much welcomed.

The major hurdle at the negotiation stage is nearly always pricing, and this will remain an issue for rare condition treatments, fuelled by a logical desire from pharmaceutical companies to gain a return on investment for a drug that only serves a limited group of patients. This cost by the companies is based on what they call added value, but the judgement on what is considered value is where we have questions.

For the treatment called Orkambi, for example, the process dragged on for many years, and there was a lot of discussion around added value as the clinical outcomes between patients varied heavily. The point of discussion is how companies can ask for such a high price when the added value is not clear and equal for every patient.

Will having better data have a part to play in these pricing discussions?

The negotiations about Orkambi were definitely the tipping point for when the discussion on data began, but nobody was ready to collect that data in a structured way. I am not against the health system here asking for data in the decision-making process of added value considering the high prices. That is logical and I do not think that the healthcare system should be paying for treatments that do not get results. It is important to gather data from the patients and have a well-structured database.

Sweden has a good system in place in which the patient associations and healthcare professionals work together and there is an application for patients to use. We should look towards a similar model in Belgium, informing the patients that we are collecting and storing data and where we are using it. Giving them this information builds trust throughout the healthcare network.

We have built a database that looks at quality of life through a new questionnaire, PRO-CF, that was developed at the European level. We are building a validation study for this system as well as a paediatric version. Furthermore, we are looking to collect data from medical professionals and store it. I would say we have underestimated the importance of patient trust as they have fears about the government having certain information and many are not ready to have their confidential information in the hands of someone they do not know.

A good way to bring innovation is through clinical trials. How well-equipped is Belgium for CF clinical research?

At the European level, we have a CF advisory board that discusses with pharmaceutical companies how to set up clinical trials as well as the administration steps, informed consent procedures, frequency of patient visits, and transfer of data.

As you mentioned, clinical trials are a great way to introduce innovation and patients are behind it, especially due to the lengthy reimbursement period. Furthermore, clinical trials assist in reimbursement discussion as data is built on Belgian patients, which means the Ministry of Health has one less concern when looking at HTA assessments.

Do the reimbursement challenges in Belgium make it difficult to bring trials here?

Yes. Companies are not always looking towards Belgium for clinical research as once the drug is approved at the EMA and Belgian level it does not guarantee a speedy reimbursement of the same therapy. Saying that, we are looking to attract companies through the establishment of a Belgian CF clinical trial network and show we are a great setting with our world-class infrastructure and global experts.

The government has put forward a 52-point roadmap for reforming the reimbursement framework and which includes the creation of a patient council. What is your take on this?

It is a major step forward. In the past, our voice was only heard in the process if we were invited by the pharmaceutical company, which was not an option as we did not want to be perceived as defending their interests. We have been trying to explain that there are three parties that need to be included in any reimbursement decision: the payer, the pharmaceutical company, and the patient.

Another point is that if the government stakeholders making the decision do not have the expertise on the disease, they should go and seek it. Patients are the true experts as the only actors that know what living with a condition is like and the impact of a treatment on how they feel. Doctors are seeing this now and are asking for patientsâ?? opinions, and so should the reimbursement body. Therefore, this move to include the patient voice is a welcome one if it happens, but we are not there yet.

What are your hopes and goals for the future?

We are investing heavily in scientific research and are the largest funders of CF in Belgium, working with the European priority list to understand which studies are best to invest into. We are looking at concepts such as gene and cell therapy to hopefully in the long term correct the mutation and find a complete cure.

In the middle term treatment, CF patients are experiencing more comorbidities, such as renal problems, osteoporosis, diabetes, and particular cancer. This is because the disease in the past was mainly in children, now patients are reaching adulthood and we want the disease to be less of a rare condition but a chronic one. Therefore, we need to make sure all patient groups receive the aforementioned modulator treatments as they are the only therapies that treat the root of the

disease, not just the symptoms. 15 percent of patients still are off the reimbursement list due to rare or ultra-rare mutations, so we need to continue to fight for them.

What is your final message to the international and Belgian audience?

A few years ago, there was a discussion set up in the UK where clinicians and researchers spoke to patients. From what I have heard, it was a complete eye opener for all involved as perspectives completely shifted as to what are the priorities for the patients. We must look at the patientâ??s voice in an upstream manner moving forward and make sure that their voice is shaping the approach and target of therapies and assisting in the decision-making process at the government level. For example, with Orkambi maybe it only makes a small difference clinically, but for the patients involved it gives them a lot more energy to live their lives in a normal way and this must be considered. On the pharmaceutical side, companies should be talking to patients from day one as the patient is their partner and must be involved in the drug development journey.

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