Eva Schoeters – Director, RaDiOrg, Belgium

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RaDiOrg aims to support rare disease patients and patient organisations in Belgium, defend their interests, and raise awareness of what can be devastating and little-understood conditions. Director Eva Schoeters - who joined the patient advocacy field following the diagnosis of her son with a rare condition - outlines some of the current diagnosis and access challenges for rare disease treatments in Belgium, how RaDiOrg taps into European-level expertise and support in its work, and why maintaining Belgium's positioning as a clinical trials hub is so important for the patients and patient groups she represents.

What are the main functions of RaDiOrg?

The association was set up in 2008 by five rare disease patient associations. We now represent more than 85 member associations with a large variation of conditions and group sizes. For example, the Cystic Fibrosis Association (Association Muco) is quite large and runs like a business, while others like the Tuberous Sclerosis Complex (TSC) association that I founded (be-TSC) are run by just a couple of volunteers. RaDiOrg has quite low thresholds for members to join as we want to be very much inclusive, and even have a database of individual members with extremely rare conditions that are not represented by any of the associations currently in Belgium.

RaDiOrg is structured around three pillars. The first is support to the patient and patient organisations. This is not easy due to the large number of rare diseases, the complexity of the conditions and the limited resources that we have. We do have a helpline to direct people in the correct direction as rare diseases exist in such a complex landscape. As well as supporting our members, they equally support us to advocate for important topics, such as neonatal screening programs and market access to innovative therapies.

The second pillar is the defence of interest. For this, we are involved in a broad number of networks, committees and working groups at the national and regional levels, and give a voice to the patients living with the diseases there. This includes being in the working group for rare diseases at the College Genetics. I am also part of the management committees of rare disease-specific actions set up by NIHDI and Sciensano, for instance, the development of a national register for rare diseases. Apart from representing the patient voice in bodies and initiatives by other stakeholders, we are also putting forward our own agendas and being proactive in making positive changes.

The third pillar is raising awareness. We have been putting forward a strong Belgian version of the European Organisation for Rare Diseases (EURORDIS)' annual rare diseases campaign. The EURORDIS campaign did not perform so well in Belgium due to the language barrier as well as featuring testimonies that were less relevant in a Belgian context, so we tailored the program for Belgium. This has really created a strong connection with patients and helped us grow the visibility of rare diseases in the country and become the face of rare conditions.

How did you come to take up the position of Director of RaDiOrg?

I have a son that was born in 2008 and 18 months later was diagnosed with TSC. This was because he was showing symptoms of infantile spasms, a rare and subtle form of epilepsy that when undiagnosed for a long time can have a catastrophic impact on development.

As a result, we established the TSC patient organisation and this is when I got involved in RaDiOrg. I was still working my old job and really began with no knowledge of rare diseases, but over time got more and more involved, moving into a coordinator role and then into my position today as Director.

I joined the patient advocacy field without specific training or medical background. However, thankfully EURORDIS, as a non-governmental, pan-European, patient-driven alliance of patient groups, has been very much involved in training and highlighting the work we need to do to overcome obstacles and address key challenges.

What are the current topics at the top of your agenda?

We have been repeating over and over again the need for our patients to have quick and correct diagnoses, so shortening this diagnosis period is essential. This can be achieved, for example, through organised neonatal screening programs.

Secondly, we have many patients with the correct diagnosis but without the expert care that they require. Sometimes this is not available due to the small size of Belgium, but even when we have dedicated experts here, patients are sometimes not referred to them. We also see that there is inequality in the quality of healthcare for complex rare diseases. For some, there is a multidisciplinary approach where post-diagnosis they interact straight away with a centre and a whole program is set up with dietary, psychological and social support. The majority of rare diseases that are equally complex do not have these structures available, and so we have been advocating to have this kind of integrated care for all conditions. This was at the forefront of our 2022 campaign.

Thirdly, we are beginning to focus more and more on market access and reimbursement of treatments.

Market access for rare disease therapies is quite complex and can be a balancing act when looking at pricing and government budgets. How significant is this area for you and where can it be improved?

It is extremely significant, but a very complex and technical issue that is very diverse. There are many aspects to address, and we want all patients to receive treatments that are recognised as

beneficial and recommended by experts.

NIHDI is developing a roadmap for better access to medication and the reorganisation of the health technology assessment (HTA) procedure. Many measures presented are very promising. For instance, we applaud the introduction of a platform for using real-world data (RWD). On the other hand, however, we have great concerns regarding the fact that the confidential contracts that have so far allowed for increased access to innovation, would be limited to 6 years in the new legislation. Knowing over 450.000 patients in Belgium now benefit from medication that is available to them thanks to a contract that is older than 6 years, and knowing how important confidentiality is in a European context, we feel quite anxious about the impact this will have on our access to innovation.

We hope to see more collaboration at the European level which would mitigate the national differences across Europe on patient access to treatments. EURORDIS equally wants pan-European market access, and we do see this continental approach being proposed for HTA with a structure set to be in place by 2025. We already have the BeNeLuxA program in place for Horizon Scanning on pricing negotiations, so why not take this further for all of Europe in rare diseases?

Sometimes, the question is not whether a therapy will be reimbursed, but whether sufficient infrastructure is in place in the country to administer it. This is especially relevant in areas like gene and cell therapy. We want Belgian patients to have equal access to therapies regardless of whether they can be administered in Belgium or not.

A big piece of the puzzle to introducing innovative therapies is utilising clinical trials. How important is clinical research for rare disease patients in Belgium?

We have been very much involved in this, and we understand the benefits for pharmaceutical companies to conduct clinical trials in Belgium. We need to support the industry and government on this. Obviously, there are economic benefits, but for us, the importance lies in the benefits for patients when they get the opportunity to access innovative medication early via a clinical trial. Access to innovative therapies can have a sizable impact on patients’ lives, considering that oftentimes there doesn’t yet exist any other beneficial treatment.

I have personal experience of this as my son received game-changing TSC treatment through a clinical trial and my aunt will soon be part of a trial for a serious lung condition. We are concerned that Belgium will not remain an attractive country in which to organise clinical trials.

Do you feel that having a relatively slow reimbursement process puts off companies from bringing clinical trials to Belgium?

It is definitely a worry. We know that the reputation for reimbursement in Belgium is poor compared to our neighbouring countries such as Germany and France. There is a concern that this will put off companies bringing clinical research here as they may not receive reimbursement for some time once the drug is approved, and even the patients can be on the therapy during the trial and afterwards have a lengthy period of uncertainty before reimbursement is approved.

We have been told the reimbursement committee is quite conservative in their approach, but we only have an outside perspective. We do not know what the financial constraints of the government in terms of budget are and what pricing the pharmaceutical companies are asking for. All we know is that we want negotiations to be smooth and fast so patients can receive the life-changing medicines as soon as possible

What are you looking to achieve for the patients you advocate for in the future?

I hope that we will be able to develop a system that directs patients to experts in their disease without wasting time so they can be diagnosed and receive expert treatment without missed changes. For patients, it is so important that they feel comfortable and looked after by a professional expert that understands their condition and is switched to global developments to improve their lives and attack the condition in a multidisciplinary way. For many of the diseases cure might not be possible for decades, so making sure they can live with their condition and be treated by an expert is all the more valuable.

Putting in place this multidisciplinary approach currently is only taking up a tiny amount of funds compared to the budget for innovative therapies, but we want to change this and make sure it takes up a bigger slice of the pie. Regarding innovation, we know the process is slow for market access. It is a great concern to us that it becomes faster in the future. Some patients have no treatment and that is hard on them, but it is completely heart-breaking when a therapy is available, but they are not able to receive it. We want to make sure all stakeholders involved know how important their decisions are for the lives of patients and their families.

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