Interview: Mirosław Zieliński – President National Forum for the Therapy of Rare Diseases (ORPHAN), Poland

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10.11.2017

Tags: Poland, ORPHAN, Association, Rare Diseases, Orphan Drugs

Mirosław Zieliński, president of the Polish National Forum for the Therapy of rare diseases (ORPHAN), the representative voice for 35 Polish rare disease associations, discusses the implications of the introduction of a national rare disease plan and the need to improve rare disease diagnostics. Furthermore, he highlights the importance of a separate orphan drug budget that takes into account different factors as well as the future aspirations of the organization.

As the president, could you introduce ORPHAN to our international audience?

We are the Polish umbrella organization for rare diseases that acts as the representative voice for 35 member associations. Our role is to be in constant dialogue with the Ministry of Health, parliamentarians, and national health fund, a well as supporting initiatives such as rare disease day and other actions aimed at improving the entire system for the treatment of rare diseases in Poland.

The main objective over recent years had been the attempt to implement a national plan for rare diseases, in line with the European initiative announced in 2009 that every nation within the EU will develop and implement its own rare diseases strategy by 2013. Today, Poland remains the only nation that has not done so.

We first took over the plan and wrote the first draft from 2009 to 2011, after noticing not enough was being down at the governmental level. This took into account the opinions of 400 individuals

from many areas of the healthcare community. After providing this plan to the Ministry of Health, they declared they would move on this immediately and in 2013 the plan was completed. Unfortunately, despite initial strong interest, the former government did nothing with this national program.

When the government changed hands, the new Minster of Health constructed a new task force, ourselves being members, to develop a revised, more specific, plan. In August this year the Ministry of Health accepted a national plan for rare disease, though we have not seen the final copy. We have heard recently this new strategy will roll out shortly, and we hope this is the case after many dedicated years of work.

What specifically will this national rare disease plan cover?

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The national program has been developed based on the initial model designed by the European project for rare disease plans. The model consists of the chapters; diagnosis and registry, centers of expertise, therapy – including orphan drugs, improving social care and education – split into public education teaching the broader polish population about the special attention rare diseases require and education of the medical community.

How advanced is the system of rare disease diagnosis in Poland?

Diagnosis has improved to some extent over the last decade due to specific national programs, such as new-born screening. On one hand the 19 screenings being conducted, mostly for metabolic diseases, is a good thing – on the other hand – it is not enough. In fact, there is a thought with some; why should we conduct a diagnosis if there are no treatments?

We strongly disagree with this. Patients that are correctly diagnosed and have no direct treatment, can still understand how to live improved lives, and also will not be treated incorrectly in the future; therefore, we should be driving forward more diagnostic procedures as healthcare community.

How can diagnosis be improved?

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Firstly, education at the level of medical personnel and universities. Secondly, registration, centred around the concept that Europe should have an established network that allows the transfer of information. Our vision is that this registry will be the basis for the entire management of the rare

disease sector.

In fact, you could view the required registry system like a Rubik’s cube, in which each square is data on a particular patient. Each group will be able to access certain information depending on their role in the rare disease community. For example, doctors can only view blood type and other physical parameters, while students can only see the symptoms of the condition. This coordinated approach will allow greater leaps in rare disease healthcare, especially in the field of clinical trials.

Healthcare systems during the drug authorization process are looking more into pharmacoecomonics. How does this impact on the market access of orphan drugs?

The polish healthcare system does not recognise the existence of rare diseases. This means that throughout the entire application for product reimbursement not once is there a differentiation of orphan drugs and other products.

Currently, companies provide a health technology assessment (HTA), incorporating data on the product and disease, as well as the influence its introduction will have on the national health fund´s budget and how the Ministry of Health should fund the drug. This process takes 180 days, and at the end of this period the company must negotiate with the economic committee on price. The Minister of Health then makes the reimbursement decision taking into consideration the recommendation of all advisors and institutions involved in the process.

Statistics show that the Ministry of Health had never made a positive decision for expensive rare diseases; why? Our point of view to attack orphan drug reimbursement has been developed by a group of polish experts called “Poland for rare diseases”, We believe that orphan drugs should be financed and have a separate pool of money to the normal national health fund budget.

This is because the treatment of rare diseases has nothing to do with normal health insurance and when the national health fund makes a decision on market access they will always factor in ethical, jealous and competition concerns. Should we treat a single patient with one drug, or treat 100 patients of a more common condition with the same amount of money? In this regard rare disease will always lose out. Although, if we have a separate budget that judges rare disease treatments at the same level, we will have a much fairer system

Additionally, the management of the entire process should be handled by a centralized entity, that will be responsible for treatment, registration and the network of rare disease specialist centers.

How does the proposed reimbursement amendment impact the rare disease community?

Thankfully, the system of Quality Adjusted Life Years (QALY) will be removed. This is an HTA system developed in the UK that estimates the cost for an additional healthy year of life for a patient.

This model functions for ordinary drugs, though when you apply the same system to orphan drugs you lack data; therefore, the indicated value is not correct as rare disease treatments should be approach in a completely different manner. As aforementioned, Polish experts are recommending to remove the QALY system, and hopefully this will allow more orphan drugs to gain market access.

What are goals of the organization moving forward?

We must create a sustainable environment for rare diseases, as a plan without money is useless. We must develop a complete registry and equally, rare disease centers of excellence.

Many people forget that rare diseases effect six to eight percent of people, that is around 2. 5 million polish people. As the government has set up the national HIV center, it is now time to implement the same system for rare diseases. The organization will continue to fight for the entire rare disease community and be an active voice in driving forward the cause to improve the lives of affected patients.

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