Interview: Rudolf Widmann – CEO and Founder, AOP Orphan, Austria

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Rudolf Widmann, CEO and founder of AOP Orphan, the first rare disease-specific European company, discusses the overriding goal to ensure rare disease patients are matched with the correct treatments and the importance of open dialogue with specialists and patient groups. Furthermore, he highlights the need to differentiate health technology assessments between rare disease drugs and normal originators to ensure rapid market access to patients.

As the CEO and founder, could you introduce to our international readers AOP Orphan?

The company's headquarters are located in Vienna and houses around 80 staff, with another 100 employees littered around the globe in various nations. We have a strong presence in the EU, except the southern part, and equally the middle east. Our concept has always been to have a strong pan European rare disease company, and currently have an abundance of products that are going through the registration process.

One of the key steps in the orphan business is to be incredibly close and constantly educate our stakeholders; specialists and patient groups. During my time working in big pharma I realized that individual patients in the rare disease community had difficulties in obtaining the correct drug as doctors had trouble aligning patients with diseases. Our influence ensures that ultimately patients are given the correct treatments, and this was always the original idea all those years ago.

Do you interact closely with stakeholders in every nation you are present?

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More or less, and with our 20 plus years experience we now better understand how to manage this dynamic. At the end of the day, it is important that employees within the organization have the appropriate skills and knowledges to be able to effectively deal with specialists and patient groups in their respective countries. At times it is challenging to work out of a central office; therefore, we are extremely active in educating and accessing patient access schemes.

Nevertheless, it is not an easy task to find staff, though with the opening of the EU's borders to eastern Europe it has helped. AOP orphan must make the rare disease business attractive and exciting for employees so they understand the great role they are playing in patients' lives, and this requires a lot of support. Within this therapeutic care one must have a certain way of thinking and be driven to deliver individual patients with innovative drugs.

For example, when I worked in big pharma I knew there existed certain drugs that saved lives, but there was no focus on this, as administrative procedures hampered the drugs ability to be administered to patients. AOP Orphan has the initiative to change this.

Healthcare budgets in recent years have found it difficult to give complete access to innovative drugs. How does this effect patient's ability to receive rare disease treatments?

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In Austria, we are in a privileged position to be able to offer orphan drugs to our patients as there exists an open mindset when discussing the topic with the reimbursement authorities and dialogue is strong with patient groups. In other markets we encounter a lot more obstacles due to the fact rare diseases are being classified on the same legislative basis as normal mainstream originator drugs. Governments are utilizing the same health technology assessments for both. Instead, rare disease treatments should be looked at in a completely different light and be judged for reimbursement using separate health technology outcomes. Society must decide if they want to give patients access to these life-saving treatments, and is a matter that goes beyond pure pharmaceutical affairs.

Austria has a specific rare disease plan for the future. Do you feel this is a model that can be replicated in other nations?

The plan is based around a system ensuring patient navigate the healthcare system and make their way to the correct centers and are treated accordingly at the best scientific level. Austria

does had incentives to invest in R&D and clinical trials, though there is a still long way to go. In respect to the rare diseases industry many other countries are more advanced, such as the Nordics.

Do you believe that the global trend of personalized medicine has raised awareness for rare diseases?

The therapeutic drive of personalized medicine is definitely something the healthcare industry must learn how to cope with. From my personal experience, personalized medicine is an area that is extremely difficult to gain a common understanding at a regulatory level as you can not conduct the same clinical studies as a normal drug. There are attempts to merge personalized medicine and health technology assessments and this may be the long-term future, but currently we are way off, especially at a regulatory level.

AOP Orphan is driven by true innovation. How do you ensure your partners fit perfectly into the vision of the company?

We are not investing in new molecules or mechanisms of action, as this is the role of basic science and must be discovered in universities and specialist centers. Each year we have four to five projects where we bridge the gap, allowing molecules to move from academia to the clinical research phase.

AOP Orphan’s role is to take molecules that fit a certain pharmaceutical profile that are lost in big pharma and construct an individual program so they can be utilized in the rare disease therapeutic area. This task is undertaken with our partners, and AOP orphan has expertise from pre-clinical to Phase IV and regulatory affairs. Furthermore, digitalization has assisted us to ensure information can be easily communicated and is a game changer in our business.

When you founded AOP Orphan you have a vision to be the voice of the Orphan drug community. Have you achieved this?

Absolutely! It was never as easy as many people think as orphan drugs generally have a significant price and it is a challenge to pass the regulatory steps so patients can access the drugs. We must always educate our staff; therefore, they can give good advice and push our vision.

Nevertheless, like any dream more can be done. We are looking to be concept driven in certain areas of expertise, such as in hematology where we have built our own path and we are bringing products to patients that are changing lives long-term. The real challenge is finding the right combination of drugs for certain patients, and this is our goal as we look ahead.

AOP Orphan is a pioneer in the rare disease industry. What is the final message you would give to community?

We must continue in what we are doing and promote open dialogue with specialists and patient groups to ensure we are the shapers of change in the orphan drug community.

In the future I see a shift in the rare disease ecosystem as the current approaches will not cope in the long-term, both from a legislative perspective and from a health technology assessment point of view. It is critical that orphan drugs are classified and looked at in a different manner to normal originators at a regulatory and reimbursement level. Furthermore, drugs in this area must gain market access at faster rate, and the decision makers at a national and European governmental level must make quicker decisions.

On another point, Europe requires harmonization of clinical studies to allow them to run smoother across the continent so we can take a leading role in driving forward orphan drug innovation. In the US this is the case, with the collective work throughout the states giving them an upper hand.

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