



FIVE KEY THINGS WE CAN ALL LEARN FROM RARE DISEASE COMPANIES



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It can be a huge challenge to help ensure that patients with rare diseases receive an accurate and timely diagnosis and then get access to the right treatments, but the results can be life-changing. This is the first of two articles in which we share five key things we can all learn from the most successful speciality healthcare companies working in rare diseases and how they help make this happen. Watch out for the second article containing five more key learnings, coming soon.

1. What do patients really want?

This is probably the most important question to ask yourself. To be able to answer it properly though, you need to get true insights from patients themselves and not assume that you or healthcare professionals (HCPs) know best. Starting from this point will help you avoid any big surprises later. Alpharmaxim has worked with

clients who have not planned for the fact that patients who have had to live with an untreated rare disease sometimes accept their symptoms as the new 'normal', which can make them resistant to trying a new treatment, even if it could substantially improve their symptoms or ultimately cure their disease.

2. Who cares wins

HCPs are busy people, and getting your voice heard can be difficult – particularly in rare diseases – so you need to provide them with a strong reason to care about your disease and your brand. Whether you are the first or the last market entrant, you need to focus on the unmet patient needs that only your product can address. It's all about putting patients first.

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3. The power of emotions

In the healthcare world, where every decision must be evidence-based, it can be easy to forget the power of emotions. Making an emotional impact has been shown to create more powerful memories and to help motivate us to act. So, to really engage with HCPs, you need to be able to communicate the true disease burden that patients face, both rationally and emotively.

4. Defining a differential diagnosis

Reaching an accurate diagnosis in rare diseases can be a real challenge, so you need to help make it as straightforward as possible for HCPs. Identifying 'red-flag' symptoms and simple, conclusive diagnostic tests, to reach a differential diagnosis, is key. Otherwise, patients with rare diseases can wait years to get the right diagnosis, which can have a devastating impact on them and their families.

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5. We're all in this together

As Donald Rumsfeld might have said, there are a lot of 'unknown unknowns' in rare diseases. Patient advocacy groups and key opinion leaders can give invaluable insights and support here, based on their hard-won knowledge and experience. To make diagnosis and treatment work effectively for patients, it really helps to build a consensus around best treatment options and agree a multidisciplinary team approach with key HCP stakeholders, across different roles and specialisms, so that you are all working together.

At Alpharmaxim, we have extensive experience in helping speciality healthcare companies across the world communicate with HCPs and patients, particularly in rare diseases. We are passionate about helping our clients tell their stories and fulfil their promises, and we aim to make a real difference to patients, families and healthcare professionals.



If you would like to know more, please visit our website www.alpharmaxim.com, or contact Sophie Jones on +44 (0)161 929 0400