A Critical Disconnect in Rare Disease Treatment

"After 2 podiatrists told me I was crazy, the 3rd podiatrist finally sent me to an Endocrinologist who not only did the normal dexa scan (which always came back normal) but did other blood work. He found the needle in the hay stack and diagnosed me in March of 2008." HPP patient, US. www.experienceproject.com

It is common knowledge that the path to diagnosis for a rare disease sufferer can be long. There are often devastating emotional and physical consequences. Once diagnosed, things can become a little easier, but the patient and their carer must relentlessly fight to receive the best care.

In pharma, orphan drugs are the new frontier. Investment is increasingly flowing in this direction. Offering new hope for countless sufferers. But how can we turn the tide fast enough to increase diagnosis rates and treat more patients?

To create a fundamental change in treater behaviour in this space, a deeper understanding of the cognitive and behavioural barriers at play is necessary. So what exactly is holding doctors back from earlier diagnosis and more effective treatment? Three behavioural barriers are particularly influential in forming this road block to diagnosis and treatment; saliency, effort and self-efficacy each play a critical role in maintaining the status quo.

With saliency, or lack thereof, this is evident in the name 'rare/orphan'. A study by Deloitte (2013) showed that only 50% of doctors expect to see a rare disease patient. With these odds, and in the increasingly time pressured environment physicians work in, there is little incentive for physicians to invest in educating themselves, and indeed, keeping up to date. Marketers of orphan drugs are some of the best in the business when it comes to disease awareness campaigns, but how do you motivate doctors to engage in something they don't see as relevant?

Understandably the effort (both mental and practical) involved in each step of diagnosing and treatment of rare diseases is higher than a common condition. Mentally, it is unknown territory. Practically, there can be access hurdles for both testing and treating resulting in paperwork and coordination challenges.

Self-efficacy, however, is perhaps the most influential barrier of the three. We all know that (theoretical) knowledge and logistical ease can help, but don't necessarily translate into desired behaviour. Belief and confidence in one's ability to recognise, diagnose and accurately treat a rare disease is quite rightly the secret ingredient.

With a better understanding of these barriers, we can more effectively develop educational and marketing tactics to change behaviour. Clearly, this isn't something that can happen overnight by waving a magic wand. Nonetheless, there are some relatively simple actions and activities that could begin to nudge behaviours in the right direction.

1) For diagnosis, incentivise the right behaviours, not the right result. Consider: What is the incentive to keep the disease top of mind if it is unlikely to be rewarded? A positive diagnosis is inherently rewarding, however, it is important to reward all testing behaviour, regardless of positive or negative results. Social norms may also have a role to play here too; sharing the number of tests conducted by neighbouring or similar hospitals to create a sense of healthy competition.
2) Leverage emotional buy-in through patient-centred campaigns. We've been in several situations where physicians can play-back pharma messages, and yet their behaviour does not change and no additional effort is taken to keep the disease top of mind. This is not necessarily because the messages are not credible or compelling. Without the experience of a patient, such campaigns are purely theoretical and fail to evoke any sense of emotion or urgency.
3) Streamline diagnosis and prescribing: reduce both the physical and mental effort involved in diagnosis and prescribing wherever possible. Consider: is there a central lab that samples can be easily sent to? Are testing kits simple to use? Is the treatment easy to access?
4) Translate support into confidence: Do not underestimate the importance of the medical rep relationship with the doctors and pathologists in this space. They hold an infinite amount of knowledge that needs to be made available in a clear and simple way. The relationship needs to be rooted in trust, and the HCPs must feel supported in this space at all times. A lack of support can quickly turn into uncertainty around diagnosis and treatment. Ultimately this delays time to diagnosis in the best case and leaves patients undiagnosed and circling the system in the worst.

Clearly, these three behavioural biases and subsequent tactics to overcome them are not isolated from one another. They are interconnected and interdependent. With consideration, time and care, however, pharma holds the key to improving the lives of rare disease patients and the result will be hugely rewarding for global healthcare.

Samantha Martin, Principal, Incite
Dr Pamela Walker, Director, Incite

Disclaimer: The views and opinions expressed in this feature are those of the author and may not reflect the official policy or position of the BHBIA. The BHBIA have not verified any of the information quoted and do not accept any responsibility for its accuracy, or otherwise.