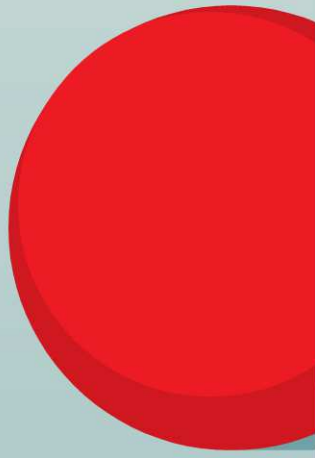
ORPHAN DRUGS

Leading the way in patient-centricity

What lessons about optimal patient engagement can big pharma learn from those operating in rare diseases?



In rare diseases, true patient engagement is critical to every step of a drug's development, from clinical trials and regulatory approval, to patient diagnosis and adherence.

Why is that? Because patients with rare diseases (and their families) are essentially the thought-leaders of their condition. Usually these patients are very few in number, and have high unmet needs. Management of their condition is often fragmented, with knowledgeable healthcare professionals few and far between. Patients can feel isolated which begets active participation in both online and offline networks with other affected patients and families. Many rare diseases specifically affect children, which causes huge distress for parents and family members, who become very actively involved.

So despite often very challenging circumstances, patients with rare diseases and their families and caregivers are typically very well-informed about their condition, very connected and active in the space, and very willing to be involved when it comes to potential advances in the area.

And it is often more than typical patient advocacy - patients are central to decision-making. Patient representatives actively participate in EMA as members of various scientific committees and working parties, and rare disease patients are also solicited as experts on the development of products related to their disease.

Patient engagement throughout the organisation

Putting the patient at the centre is an absolute necessity for pharma companies involved in this space, and several companies have been very successful in doing so, for example Sanofi Genzyme, Novo Nordisk and Novartis, that were ranked the top three companies for corporate reputation according to rare disease patient groups.

So how do companies successfully engage with patients and ensure the orphan drug they have in development will reach the patients who need it? The key is involving patients as early as possible throughout all relevant functions within the organisation.

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R&D: Traditionally, R&D has not been an area for patient engagement, but things are changing. Recently the European Patients' Academy (EUPATI) released a study showing a growing belief among pharmaceutical industry personnel that greater involvement of patients and the public could improve R&D. Pharma companies can help to support patients with tools and knowledge for meaningful involvement.

If patients are involved, they can provide input on identifying health research priorities and participate in the design and undertaking of research projects. As a result, research is conducted in areas patients value; researchers understand the value of patient involvement and patients understand the value of research.

In the rare disease space patient organisations are even setting the research agenda, promoting collaborative research networks, initiating studies and providing financial support for research infrastructures, such as patient-powered registries.

Clinical development: It has been shown that patient involvement in clinical development may contribute to the successful delivery of trials by increasing the likelihood of recruitment to time and target together with improving participant retention.

Many companies investigating orphan drugs are involving patients at all stages of clinical development from providing input into study protocols and the selection of endpoints to the dissemination and implementation of findings.

Patients are the experts in their disease and its impact, and can identify the clinical outcomes that are most meaningful to patients. Due to their often advanced networks, they can also help to enhance communication about research participation opportunities and disseminate research findings in a patient-friendly way.

Approval and market access: In both the US and Europe, a range of schemes to facilitate patient involvement in the regulatory process has been established. The FDA has introduced a Patient Engagement Advisory Committee and the EMA has patient representatives on a range

of its committees, including its management board.

Health technology assessment agencies in several countries have also focused on improving patient involvement and are asking patients to engage at the time of reimbursement decisions for payer decision-making.

For rare diseases, physicians may not see a sufficient volume of patients to be able to correctly characterise the full range of symptoms and disease manifestations across different patient segments. A recent paper concluded that: 'In practice, this implies a need for more systematic and meaningful involvement of patients and HCPs into value assessment processes for orphan medicines.'

Orphan drug companies collaborate with patient advocates for example to demonstrate the burden of the disease, add it to the policy agenda and help to incorporate the rare patients' benefit-risk preferences into a structured evaluation process.

Commercialisation and life cycle management: In orphan diseases, the role patients and patient groups have at this stage can be significant, whether it is patient group dissemination of disease information or trial results in patient-friendly ways, patient group engagement in guideline shaping, or involvement or co-development of beyond-the-pill services.

Lessons in patient-centricity

Patient engagement is not only suitable for rare diseases. And with the patient now moving towards the centre of the value equation, pharma companies have little choice but to embrace a more patient-centric approach.

Although patient engagement fits very naturally with rare diseases, there are lessons that big pharma can apply to their more 'mainstream' disease areas.

The following four steps are integral to success:

1. Adopt a (compliant) strategy of listening and learning

Although it is particularly true in the rare disease space, all patients are experts on their own condition. They know how it makes them feel. They know what improvements would be most meaningful to them. This is invaluable information for pharma companies.

Companies should adopt a culture of respectful listening and learning and be open to incorporating the patient perspective into their strategy. Also by relinquishing some control and empowering patients, a genuine partnership can be formed.

While it is important that companies work with their legal and compliance colleagues to ensure any activities adhere to guidelines, compliance should not be an excuse for not engaging with patients. Enough companies are doing it successfully to show it is possible.

2. Be inclusive and engaged and be in it for the long haul

In all disease areas, patient organisations and networks can be very passionate and vocal in their space; it is important that the commitment the company makes is genuine, long term and meaningful. There is often a willingness on the part of patients ➔

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and caregivers to collaborate in the hope of driving research and progress forward for the community.

There's more that companies can do beyond the standard market research and patient advocacy organisation liaison. Establish transparent two-way communications. Find out where the unmet needs are. In this context, pharma companies have a real opportunity to add genuine value, providing support for patients and caregivers in the long term.

"We want to know that you care about our children, that you care what happens to them as people without respect to whether you are able to sell them a product or not… We want to know that if the research is more difficult, that if the science takes longer, if the competition gets heavier, that you are committed", says Melissa Hogan, whose son suffers from a rare disease called mucopolysaccharidosis (MPS).

3. Provide support beyond the pill

Providing solutions that make a meaningful difference means taking a holistic view of the patient journey, identifying the gaps, and working directly with patients to develop 'beyond-the-pill' solutions, even if they do not relate directly to your product.

Can you help to increase awareness for earlier diagnosis? Are there unmet needs with emotional and interpersonal aspects, such as patient-doctor interactions? What are the underlying reasons that make patients stop or pause their treatment? How can patients be supported in daily tasks so that they can lead a normal life? Plotting the unmet needs along the patient journey will demonstrate where you can add the most value.

4. Organise around your stakeholders

In rare diseases there are often a limited number of experts globally, meaning that successful strategies are less country focused and more focused on targeting well-developed networks that typically span regions, and increasingly also countries.

While the traditional affiliate model may still be more suitable for more common disease areas, it is worth considering how you can structure your approach around your stakeholders. There may be clusters of HCPs or patients/patient groups that operate on a more supranational level.

By structuring your approach to them accordingly, it may result in improved customer interaction and satisfaction, increased speed of organisational evolution, cross-border best practice sharing and ultimately, improved efficiency and profitability.

Rare diseases leading the way

Greater patient engagement is essential for pharma as the patient moves towards the centre of the value equation. While patient-centricity in the rare disease space has been borne out of necessity, it has now become an example of best practice for others to follow. At the heart of its success is genuine, transparent two-way communication with patients and patient organisations, enabling pharma companies to provide solutions that will make a meaningful difference.

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