The Planning Shop

RARE DISEASE MARKET RESEARCH NEEDS A MULTIFACETED APPROACH

Caroline Mathie discusses some of the challenges in researching rare diseases, what the broader market can learn from observing these approaches, and the role of online research in particular.

Earlier this year, England's chief medical officer called for the 'genomic dream' of genome sequencing for all cancer and rare disease patients. Accelerated use of genome sequencing is allowing the identification of ever rarer diseases – a recent case in point being six children who were identified through an international database of genes and disease characteristics as having the same ASXL2 gene mutation. This answers two fundamental needs experienced by those with rare diseases; (1) knowing the cause of their problem and (2) the ability to share their experiences with others with the same condition. It also presents the industry with an opportunity to address the root cause of the disease through targeted therapies.

Background

By March 2017, 49.7% of the world's population had access to the internet (88% in North America and 77% in Europe) - figures that are growing rapidly. Unsurprisingly, this access is a valuable tool for researching rare diseases. In Germany alone, the Journal of Medical Internet Research (January 2017) identified 693 websites containing information on rare diseases, many of them provided by support groups/patient organisations, and showed that these are extremely valuable sources of information for patients and their families.

A high proportion of internet users engage with social media, including Facebook and Twitter, and there are many social media support groups for a wide range of rare and ultra-rare medical conditions. As genome-wide analyses, such as exome or whole-genome sequencing, become more commonplace, it is likely that the number of patient groups for rare diseases on social media will increase substantially.

Patient isolation:
Problem and opportunity

Having a rare disease can feel lonely, and rare disease patients are already fighting their isolation through social media, and by linking to others with the same condition. However, internet access also offers new opportunities in terms of specialist consultation, and patient education.

As new diseases are identified, ever more patients are forced to travel long distances for lengthy periods of time to specialised treatment centres. Each new disease may require a multi-disciplinary team, sparking the need for information and education for patients, carers and often other treating healthcare professionals (HCPs).

The use of the internet, to share information and for discussion, has become critical to both reduce the burden on health services and give patients the information they want at the time they want or need it. Forward-looking specialists are now looking at technological advances to enable at least some consultations with patients via video conferencing. However, though they may relieve logistical and time burdens for patients, fears have been expressed that they may be clinically risky and associated with significant technical, logistical and regulatory challenges.

Forums:
Opportunity and challenge

Patient forums are having a dramatic effect on the world of market research. They often emerge spontaneously after a small number of patients set up networks or communities to address their needs for information and to share experiences. These types of forums – and other, more established, groups – have been great tools for market researchers to gather information about the experiences of people with rare diseases, as they allow thorough observations of forum conversations. However, the forums may become hidden, as closed or invitation-only groups, if they become unwelcome targets for pharma and the healthcare industry. Thus, what could have been an opportunity for market researchers can turn into a frustrating difficulty.

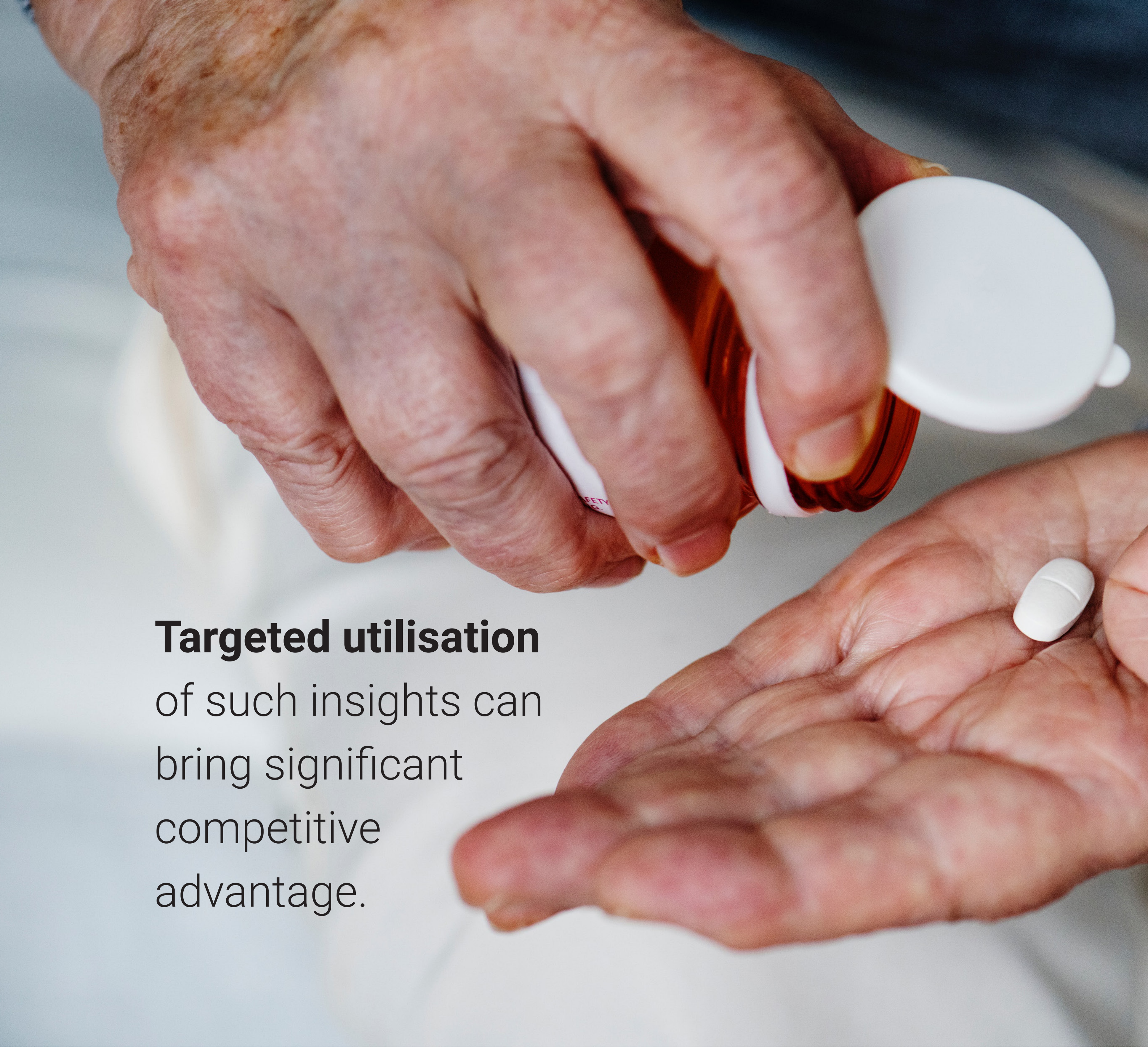
Another problem with forums is that the industry itself has ethical and compliance concerns regarding too much contact with individual patients.

Nevertheless, the emergence and growth of patient forums suggest that the fabric of the rare disease market research world may have to change from ad hoc recruitment for interviews to a multi-prong approach which includes much longer term community observation.

Some solutions

All is not lost, however. When it comes to using online forums and communities for rare disease market research, we can, for example, set up our own ongoing network or community. To be effective, this requires knowledgeable and effective moderation – continuously – which is costly.

However, it does provide the ability to address recruitment issues with a specific set of patients and, more importantly, facilitates an understanding of the longitudinal journeys of different patients



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and their families through listening to their views over time. The community also provides the ability to identify common points on the patient journey and the needs they have and the questions they are asking at each, to build a comprehensive picture of homogenous vs diverse or segmented needs.

Less costly, but effective, is to ask to join the specific closed communities as market researchers. Some forums allow this and some do not.

Patients can also be researched using standard approaches online, which, of course, come with the usual online issues. With rare diseases, questions around patient identification are more significant, as is making absolutely sure the patient really is a patient. Guaranteed security and compliance must also be in place. Finally, if clients are given access to listen to communities, specific patient identifiers need to be removed, and if it is a short term moderated community, any inputs or questions should not reveal the identity of patients.

Market research – a change in approach

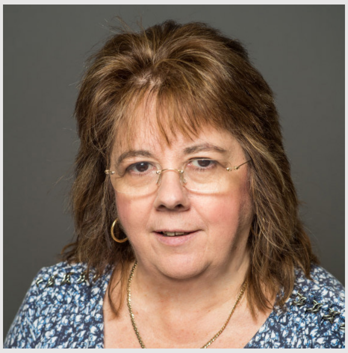
As part of the market research industry we need to explore both a change in approach and, potentially, a change in the business relationship we have with our clients if rare disease market research is to be achieved successfully.

We also need to discuss our approach to dealing with duty-of-care issues up front, if we establish a long-term, moderated community.

Clearly there is also a role for traditional research approaches for these isolated patients using the web, such as teleweb interviews and Skype. Visual connection is also helpful to create rapport and reduce the sense of isolation.

In summary

The market research model for rare diseases is changing and to benefit from the richness of data produced by long-term patient communities, we need to think differently, set up relationships differently and consider not only the market research, security and compliance aspects, but also the ethical duty-of-care issues to sustain communities that may become important components of patients' lives, especially genuine patients' perspectives. Targeted utilisation of such insights can bring significant competitive advantage. Therefore, pharma companies should embrace social media listening to help them implement further actions appropriately for greater commercial success.



About the Author

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Caroline leads the Rare Disease Group at THE PLANNING SHOP. After studying Medical Biochemistry, she spent 14 years at IMS Health, over 10 years as an independent, director level consultant and four years as a director at J & D Associates. Throughout her career, Caroline has had an active interest in orphan diseases and has expertise in a wide range of orphan disease conditions, including various lysosomal storage disorders.

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