Patient-led design: from concept to reality

The time for patient input is now

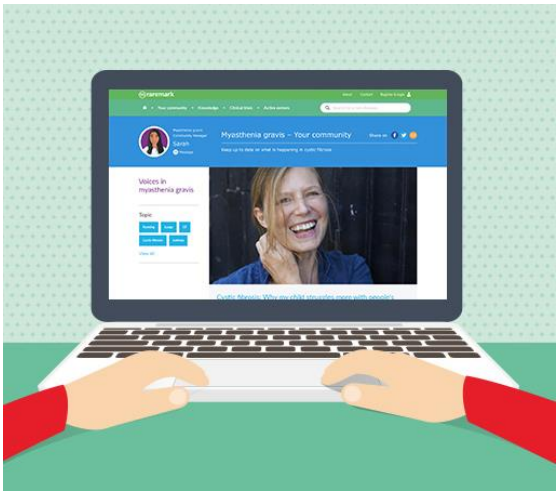
It's hard to think of another industry where the views of the user have been largely ignored. This is partly because the pharmaceutical sector employs a lot of doctors, and traditionally the view has been that the doctor knows best. It's also because drug development is complicated: the work involved in designing, planning and implementing complex clinical trials in multiple countries means it can take 10 years to get a drug to market. So it's understandable that drug developers have been averse to adding another variable into the mix by asking the users – that's the patients – what they want.

Delegates at the recent eyeforpharma Clinical Excellence Europe 2016 conference in London heard that what doctors think is NOT the same as what patients think. The conference was attended by around 200 leaders from the pharmaceutical industry, patient groups and the academic community, as well as patients themselves. The good news is that everyone wants patient input to become a core part of clinical trials, so that they can be more patient-centric in design.

Lessons in rare disease

Raremark led an interactive session, challenging the audience to think about what patient input really means, using insights from one of our own rare disease communities to spark discussion.

Before the event, using a real clinical trial protocol, we had conducted a qualitative study in myasthenia gravis (MG), setting up a secure, online focus group with 13 people living with the condition. MG is a rare neurological disease, affecting about 14-40 per 100,000 people in the US[1].



A qualitative study allowed us to get a good understanding of patients' views from a small group of people. It meant we were able to understand different perspectives and motivations, as opposed to generalizing our findings from a statistical perspective[2]. This type of study also allowed the participants to tell us what was important to them.

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Over a two-week period, we asked our panel questions about aspects of the trial that we believed may affect their daily lives and their willingness to participate. They also told us more about what it is really like to live with the condition every day.

We shared a number of top-level themes with delegates.

Understand the individual needs of each patient: I am more than a number

Each individual has a different experience of living with a condition and different reasons for taking part in a clinical trial – people wanted to be treated as more than just a number. Doctors might tell patients they are stable, but often patients have a different definition of stability. Much of the panel agreed that often the questions being asked of them by doctors during clinic appointments about how well they were doing didn't accurately represent how they felt. One member of the panel described how she would have to answer vague quality-of-life questions with very specific answers, in order to make her doctor truly understand how she felt.

"I have found the best thing to do is use very specific phrases... [like] 'I used to be able to walk around the block and speak at the same time, now, my speech becomes garbled when I walk halfway around the block.'"

MG patient, US

Education and support is needed outside of clinic visits

Participants in a clinical trial live with their condition every day, so support should go beyond what happens at the study visit. Our panel wanted trial sponsors to think about the support, education and advice that might be needed outside of visits to the clinic. These could include advice on exercise or diet whilst trying a new medication, or a way to record any questions or issues they had so that they wouldn't forget them during their next appointment.

"What would be helpful within a trial would be education, education, education! I realize there is little research out there, but any education would be appreciated. My physicians have never taken the time to educate me on how to deal with MG, especially in the area of exercise. Any information which could be shared would be great… Tips on how to exercise, work etc. whilst trying a new medication."

MG patient, US

An individual, round-the-clock condition

Our panel wanted patients to be consulted before a trial, so that those running the study understood what living with the condition is really like, as it affects everyone differently. Although the majority of the panel understood the need for common endpoints to ensure a drug works, they also wanted to be asked about their own measures of improvement, based on what is important to them. For example, some of the panel wanted to measure their improvement by how well or for how long they were able to do tasks in the morning, such as washing or drying their hair, before their arms became weak. This is important to MG patients because their strength wanes throughout the day. A balance is needed between getting what clinical teams need from the research and giving patients what they need.

"I have begged for a different tablet over the years, but my consultant feels I'm coping, as I am, but surely if you can't chew in the morning or hold your child at night or read a full bedtime story then something needs to change."

MG patient, UK

We asked conference delegates: what challenges exist in helping patients measure what's important to them?

Patient-reported outcome measures (PROMs) have to be relevant for everyone

One of the biggest challenges for industry is ensuring that any patient-reported outcome measures (PROMs), that is, data collected directly from the patient, is relevant for everyone. Particularly in rare disease, every patient's experience can be different, which makes standardizing data collection and interpretation difficult.

PROMs need to be widely recognised by regulators

There was a general feeling that regulators can still be behind industry when it comes to recognizing and accepting PROMs as a statistically-significant endpoint in clinical trials. Although things are moving in the right direction, more guidance from regulators is needed on how industry can ensure PROMs are more widely accepted in research applications.

Time is of the essence

Drug developers are often working to tight schedules when designing and recruiting patients to a clinical trial. Building relationships with patient groups, patients and other key stakeholders, organizing an appropriate way to get their input and understanding any changes that need to be made to the trial protocol takes time. That's a luxury that many clinical teams do not have.

What can we do to overcome these challenges?

Get patients involved as early as possible in drug development

Involving patient groups and patients in the drug development process should happen as early as possible. Some examples mentioned by delegates included nurse advisory boards to shed light on logistical support and trial site experiences, large-scale surveys using existing online communities to understand PROMs and disease burden, and patient panels who are formally trained in the research process and able to provide input at each stage of drug development. For those who had experience of patient input, the outcome was clear: not only did involving patients as early as possible make timely input easier, but recruitment was faster and more effective because of the improved trial design.

It's not one-size-fits-all: design PROMs questionnaires that are fit-for-purpose

Although PROMs need to be representative and relevant for everyone, questionnaires designed to collect PROMs should be tailored and specific to the clinical trial and therapeutic area. The *FasterCures* initiative, a think tank working to improve the clinical research system, has already identified 70 resources available to support the science of patient input[3], and some of these frameworks are being used already.

Standardization to some degree is useful, but clinical teams should adapt any existing PROMs questionnaires with the help of patients, caregivers and regulators, for each individual trial, to ensure that they are truly patient-important.

Share experience to educate each other and regulators

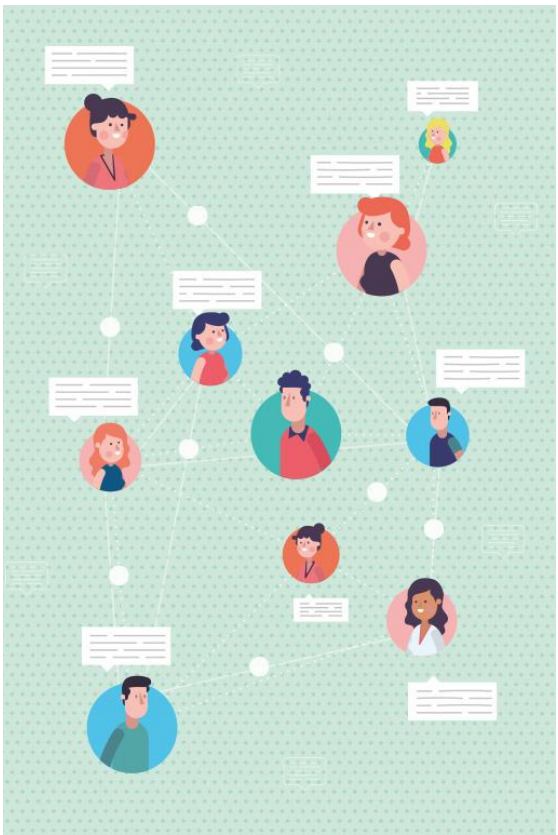
Regulatory bodies are recognizing the importance of getting input from patients in the trial design process. The US Food and Drug Administration (FDA), for example, has launched the Patient-Focused Drug Development Initiative. This is the agency's commitment to gather patients' perspectives on their condition, in order to support education within the industry[4]. There are growing calls for this type of work to happen on a larger scale; industry and regulators need to come together to educate and share real-world experience and success in designing and measuring PROMs in each therapeutic area, so that they can be improved upon by the industry as a whole.

The patient voice is slowly being heard

The pharmaceutical industry still faces many challenges in making sure the patient voice is properly represented. Designing and running a clinical trial is a complicated and highly-regulated process, but the industry is slowly moving in the right direction. The more patients are given a voice in discussions like this one, the more they will be heard. Raremark is leading on making the patient voice heard in rare disease.

"Just as a summary I want to write about me and MG. I am female, aged 48, been with MG since age 14. I have had every symptom, even blue-lighted to hospital a few times, not breathing. I am all for clinical trials to improve the lives of the younger ones [to help them live] a full, fruitful life. I'll travel, I'll take meds be that by tablet or infusions, so long as medical advice etc. is on hand. MG has taken my life plan on a different route than the life I wanted, and I want youngsters to be able to live without symptoms and to live their dreams."

MG patient, UK



Thank you to everyone who took part

Thank you to the members of our community who took part in the research, giving up their time to share their thoughts and experiences with each other and with us.

About Raremark

Raremark's mission is to transform one million lives in rare disease. Our online platform and dedicated community managers connect families affected by rare diseases with up-to-date scientific information, community insights and clinical trials. We work with pharmaceutical companies, clinical research organizations and academic institutions to support them in patient recruitment and retention in clinical trials.

Authors

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