Patient-Enhanced Drug Discovery and Development

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Patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.

In other industries, it would seem odd not to get input from the end user of a product throughout the development stages. But it seems that pharmaceutical companies — some would say belatedly — are now waking up to the idea that getting meaningful patient input throughout the drug development life cycle could potentially save them a lot of money.

According to the FDA, patient-focused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. The primary goal of patient-focused drug development is to better incorporate the patient's voice in the process of drug development and evaluation. including but not limited to:

Patients as Partners

Industry reports find that pharmaceutical companies are beginning to appreciate that involving patients in the drug development life cycle is extremely beneficial. For example, in 2017, AstraZeneca launched a global patient partnership program to engage patients in the drug development process.

"We have to involve patients early and often," says Helena Chung, patient engagement director at AstraZeneca. "It is about improving the experience of patients in the clinical trial process, but it is also about developing the right medicine that reflects the patient need."

In 2017, AstraZeneca launched a global patient partnership program (PPP) to engage patients in the drug development process. The 68 members of the program were involved in everything from scientific discovery and drug development, to disease management solutions and patient education. The PPP advisors participated in global patient advisory boards and helped clinical and medical teams identify new ways to make science work for patients.

According to AstraZeneca, the program was particularly helpful in early-stage development, where researchers would not normally have much input from patients, and patient involvement has fundamentally changed the way the company's researchers think about developing medicines.

To better understand the true extent of how companies are incorporating the patient voice into clinical research, Evidera undertook a market research project. "We've identified that many mid- to large pharma companies have incorporated the patient voice into R&D efforts to some degree — however this is not being done in a consistent manner," says Erem Latif, director, patient engagement. "For the most part, efforts are focused on rare and orphan indications, specific therapeutic areas, or higher profile products. In order to be truly patient-centric some aspect of patient insights would ideally be incorporated into every clinical program — be that is as simple as an informed consent form or recruitment materials or something more complex, such as study design and protocol development."

Bill Byrom, VP product strategy and innovation, Signant Health, is passionate about the development of clinical endpoints when using wearables and sensors in clinical trials. "In my published review of the use of activity monitoring in COPD trials, I found more than 80 different clinical endpoints described in 76 clinical studies," he says. "In none of the studies I examined were patients consulted to determine what endpoints might measure constructs that are the most meaningful to patients. Instead, endpoints were selected solely by the researchers. For example, it may be easy to assume that total steps per day might be a good measure of activity that would be relevant and meaningful to most populations. However, if a truly meaningful aspect of health in a less active population might be being able to walk a child to their school, then being able to measure short bouts of continuous walking may be a more pertinent measure which could be lost in an overall summary measure like total steps per day. Researching what is meaningful to patients, by talking with patients, is a vital component of determining the concepts that should be measured and how to derive endpoints to describe these. With the development of the new FDA patient-focused drug development guidances, we will see more work addressing this gap area."

To ensure it was addressing its patient population adequately, Diasome collaborated with a company called dQ&A, which administers questionnaires to gain insights from the diabetes community, to issue a comprehensive online survey. Respondents were asked to answer in multiple choice format the likelihood of using a therapy. Diasome is developing hepatocyte directed vesicle (HDV) technology to reduce the incidence of high and low blood sugar levels in people with diabetes to improve disease management and quality of life. "One goal of this research study was obvious — to understand the interest of patients in our technology as compared with others — but we also wanted to uncover the unaddressed consumer needs in the diabetes community," says Bob Geho, CEO of Diasome. "Therefore, this research also included a 'jobs-to-be-done' analysis measuring needs in disease management that are important but underserved."

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He says engaging with patients in this way can present additional opportunities for innovation and differentiation. "It is mutually beneficial for pharmaceutical companies and patient communities to include this type of analysis in all market research," Mr. Geho says. "It is helpful in identifying the biggest areas of business opportunity, but more importantly, key areas that would make a difference in patients' lives. In our case, we found that 'less variable blood glucose levels during the day' represents the biggest unmet need for Type 1 diabetes patient respondents. This feedback has shaped our entire company focus from corporate messaging to how we gauge our technology's success."

Lindsey Wahlstrom-Edwards, head of partnerships at Antidote, says the most important aspect of adding the patient voice to R&D efforts is to always remember that patients are the reason for the research — and that it would be impossible without them. "With this notion as a north star, the industry should be striving to form relationships with patients as partners and stakeholders, rather than subjects," she says. "We conducted a survey of more than 4,000 patients and caregivers with eight of our advocacy partners. The survey revealed that across condition areas, patients agree on what would make them feel like a partner, rather than just a participant in research: talking with doctors involved in research, talking with nurses involved, and talking with other patients who have taken part in research. The implications for researchers here are clear: ensure that these conversations are able to happen. Our survey also revealed that information is critical to patients — across conditions and demographics, patients considering a clinical trial want to know the intent of the research, what to expect exactly, risks, benefits, etc. Patients simply cannot become partners in research if they are not properly informed."

Patients want to be part of the process, as nobody knows how their disease affects them more than they do. They want to have input across every stage of the discovery process.

Antidote research supports this as well. "We believe in the initial stages, researchers should be asking patients, 'What matters to you? What problems is your condition causing that we may be able to solve?'" Ms. Wahlstrom-Edwards says. "These questions should frame the entire research endeavor, including key decisions about endpoints, procedures, and patient burden. We are pleased to see more of our patient advocacy group partners being invited to the table earlier in the process and are hopeful that this trend continues."(PV)