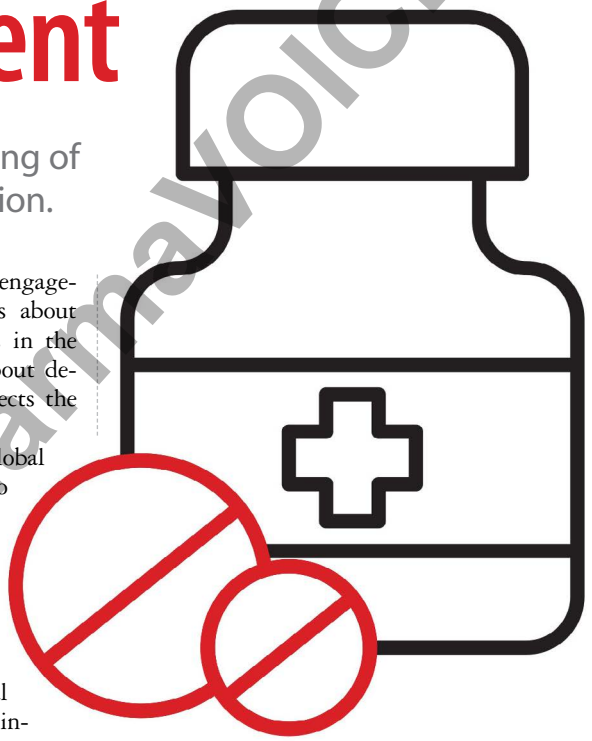
by Taren Grom

Patient-Enhanced Drug Discovery and Development

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

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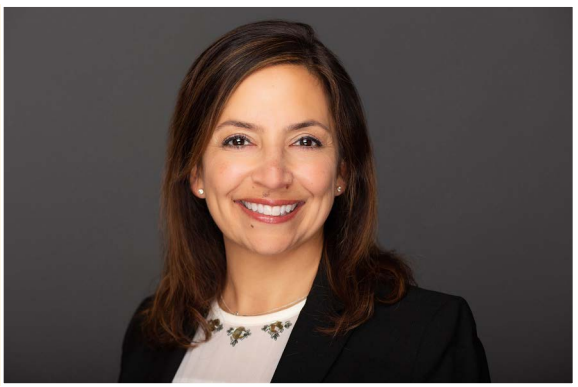
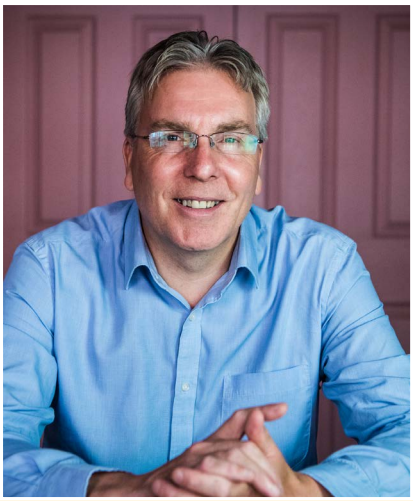
EREM LATIF
Evidera





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.

BILL BYROM
Signant

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

Patients' Voices

MELISSA ADAMS VANHOUTEN
AGMD Public Policy & Outreach Director
Gastroparesis
@MelissaRVH

We long for cures above all else, of course, but shy of that, we seek treatments that improve the quality of our lives — quality of life as we define it for ourselves and not as characterized and imposed upon us by detached observers. We urge the industry to hear us and address the characteristics of our illnesses that matter most to us, as patients. We need pharmaceutical companies to recognize the symptoms most significant in our lives and work toward alleviating those symptoms with treatments convenient to our lifestyles and befitting of our needs.

Pharmaceutical companies should seek to gather and include patient input from predesign to end. Patient advocates have the ability to reach vast audiences of diverse patients and can assist in sharing opportunities for input and in recruiting participants. Further, there is no one better than the patient to inform pharma of the symptoms that are most burdensome, the issues that most impact their daily lives, and what sort of improvements they would need to see to make the study and the potential resulting therapies worth their involvement. Patients are likewise best positioned to describe their study-related limitations — time commitment, travel, costs, physical difficulties, and the like.

During and after completion of the project, patients can still provide helpful input, as they can offer feedback about what worked, what did not, and what ideas they have for improving the process.

ERICA DERMER
Ehlers Danlos Syndrome, Endometriosis, Adenomyosis, Celiac Disease, IBS
@CeliacBeast

Why can't patients be more vocal about their experience in clinical trials? I'd love to hear from actual patients.

MELISSA DUNFORD
Their Faces, Our Voices
Substance Use Disorder
@FacesOpioids

In my career as a software developer, I always thought it was key to get the end users' point of view to make a product that works for them within their daily lives. This industry should do the same and the outcomes of patient care would increase significantly.

CHRISTINE FROST
Menopause
@Shahrazad1001

At my last role in a nonprofit that helped advance research for Type 1 diabetes, we frequently surveyed our online patient community to find out where they saw the gaps. What meds were not as effective? What functions of a medical device were inconvenient? The user experience should be taken into consideration when developing something to improve the lives of patients. Human factor research is key, but this seems to be overlooked/or frequently disregarded. In my experience, patients were delighted to be asked, and most of the time, the companies were grateful for actual insights.

LYNN JULIAN
Patient Advocate
Lyme disease; Ehlers Danlos Syndrome, Brain Injury, Arthritis
@popsuperhero

I believe companies should ask for patient input before a drug is even marketed. Patients can give priceless insight that researchers and marketers would not come to on their own. We are the community actually engaging with the product.

MICHELE NADEEM
Patient Influencer
Chronic Lymphocytic Leukemia (CLL)
@globalcorpcomms

I have heard from researchers that I was the first patient they met with the disease they were working on. It was fascinating for them to be able to speak with a patient and ask questions, and for me to learn about areas they were considering and those they had not considered until speaking with a patient.

SHARON ROSE NISSLEY
Founder
Klippel-Feil Syndrome Freedom
@KFS_Freedom; @Rose_Of_Sharon8

Ten years ago, I was diagnosed with Klippel-Feil syndrome (KFS), a rare skeletal disease, which affects one in 42,000 people worldwide.

I was 38 years old at the time. The diagnosis explained a list of medical mysteries. I have been advocating earnestly for myself, Klippel-Feil syndrome patients and families, and the rare disease community, since then. I founded Klippel-Feil Syndrome Freedom, a first for KFS. I also have

Patients' Voices

associated rare conditions such as Ehlers Danlos syndrome, cervical dystonia, and an undiagnosed bleeding disorder.

Klippel-Feil syndrome (KFS) is so underserved, misunderstood, and needs focused discovery. KFS is a rare disease that namely affects the spine, bone development, spinal cord, nerves, muscles, and organ development. Specifically the cervical spine (neck) is congenitally fused at various levels, and organs such as the heart, kidneys, eyes, ears, etc. can have congenital defects. Bones don't develop properly which can cause issues such as restricted lung function, abnormal limbs and joints. KFS is musculoskeletal in nature. For me personally, KFS has caused chronic debilitating head pain, nerve, muscle, and bone/joint pain and deformity, which ended my full time career as an interior designer. When I couldn't find an organization I started one, to provide a full resource of information, support, and an active international network for our community. KFS Freedom has 6000 followers online, a patient registry specific to KFS, and a dedicated experienced geneticist onboard. There are no drugs or treatments specific to KFS. I think that because our treatment is mostly needed in the chronic pain, neuro, orthopedic, and palliative care areas, that we are overlooked or lack interest from pharma, which is frustrating.

Companies need to hear the first-hand experience and feedback from someone living with a rare disease, which is second to none. We hold the critical data that they need. Embrace us. Always include us in your discussions, allow us to attend workshops, roundtables, panels. Make it easy enough for us to attend or participate remotely. Provide access without obstacles. For me the biggest obstacles are transportation, out-of-pocket costs, followed by fatigue due to pain. Organize events with more frequent breaks than you typically would. Schedule discussions, phone conferences, or webinars at different times of the day and evening too. Often meetings, panels, and workshops are in big cities, but many patients might find them easier to attend if they were held in the suburbs — less time, travel, expense — which would increase attendance and interest. Maybe a series of educational and/or discussion-driven lunch and learns in various locations. Companies need diversity, and they need to include patients of all ages equally, from infants to seniors, to capture the entire landscape.

SIMON STONES
Patient Advocate and Consultant
Musculoskeletal Diseases, Inflammatory Bowel Disease, Pancreatic Cancer
@SimonRStones

Patients should be involved right from the very start. From understanding realistic unmet needs and abilities, to understanding how people want to take medications, designing clinical trials to fit around the needs of participants and designing how best to dispense and market medications — there's a role for patients throughout. If you look to other sectors, nothing is ever done without involving the end user, yet medicine seems to forget this principle, until something happens that they weren't expecting. Not only will patient involvement yield a better experience, it will also deliver a return on investment through the identification of issues early on.

MOLLIE TINNIN
Ulcerative Colitis, Ostomy, Neurogenic Bladder, Depression, Anxiety, PTSD
@chronicallyconnected (Instagram)

I would like to be able to give input at all stages of discovery because as patients we are subject matter experts about our experience with our condition. But I think it's also important for patients to feel as if they are contributing to the future of care for their condition. We want to feel a sense of ownership.

ALICIA TRAUTWEIN
Autism
@themomkind.com

When it comes to R&D efforts, I believe the best way to talk directly to patients is to ask them about their medications. They would be more than happy to let companies know how the medication affects them and what they are looking for long term. Also, a better understanding of how their disability/diagnosis affects them will help pharmaceutical companies know what patients actually want when it comes to patient care. While there will be some differences between each patient, the core issues will likely be very similar.

ysis measuring needs in disease management that are important but underserved."

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

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- 3,280 life sciences establishments
- A national leader in cell and gene therapy research, development and manufacturing
- First-ever FDA CAR-T approvals came from companies in New Jersey
- More than 25% of all cell and gene therapies in development are being done in New Jersey region
- Leads the nation with 139 FDA-registered biopharmaceutical manufacturing facilities
- Had 792 active clinical trials in 2018 with an economic impact of nearly $1 billion
- The world's highest concentration of scientists and engineers per square mile – more than 225,000 statewide
- 63 academic institutions turning out 27,000 life sciences graduates each year

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