Pfizer Logo

Patients’ Voices Shape the Drug Development Process Through a Preference Survey

Saturday, November 5, 2022

Cory Lewis has sickle cell disease. Some days, the pain from the blood disorder hits him hard. Even on days without pain, he worries about his future and the health conditions that might arise. “It’s definitely 365 for me,” he says.

As a program coordinator with Sick Cells, a sickle cell disease advocacy organization in Washington, D.C., Lewis regularly educates people about what it’s like to live with this disease—a rare, inherited condition, predominately (but not exclusively) impacting Black and Hispanic people.[1,2] In sickle cell disease, red blood cells resemble a sickle rather than a disc and can block blood flow, causing pain and damage throughout the body.[3]

Recently, Lewis, his colleagues at Sick Cells, and other patients with sickle cell disease had an opportunity to share their experiences by collaborating on a patient preference survey with Pfizer. “I feel empowered when I can tell my story about how I'm living with an illness that potentially could put me down or could slow me down," says Lewis. "It makes me feel better to really get that message out and educate people about my health and how they can help."

His team's work on that survey will, indeed, help to educate others. But it'll do much more than that.

Mariah Kelly, Patient Advocacy Director at Pfizer, helped to design the survey using an app that was co-created by Pfizer and Qualtrics. Incidentally, that app is subsequently being used by other rare disease teams at Pfizer for better patient engagement.

Kelly says the insights from respondents with sickle cell disease and their caregivers will help Pfizer scientists better understand patients' priorities, the effects of sickle cell disease that matter most, and preferences for treatment options. In time, that could help lead to new therapies. "Obtaining patients' perspectives on the impact of sickle cell disease on daily life informs many of our decisions during drug development," says Kelly.

Collaborations like this are a priority at Pfizer. After all, nobody understands a health condition better than the person experiencing it. Through formal and informal means—including surveys, advisory boards, focus groups, interviews, and discussions—people like Kelly build relationships with patients and patient advocates and listen to them in order to better understand and empathize with what they're going through. "We are always seeking to learn," says Kelly. "We are acutely aware that we don't have all the answers and are unable to advance science without the valuable personal experiences only patients can provide."

The Sick Cells survey collaboration, while just one example, illustrates Pfizer's ongoing efforts to prioritize patients' voices, when possible while researching and developing therapies that have the potential to impact patients' lives.

A shared mission in survey design

The survey partnership was an educational initiative for the Pfizer and Sick Cells. The two organizations had worked together for years on different projects. Pfizer approached the patient advocacy organization in December 2021 and asked if they'd help develop a survey for patients and caregivers to better understand their priorities better. Maggie Jalowsky, Director of Advocacy at Sick Cells, recalls it was an opportunity that fit well with the organization's mission.

"Something that we're really excited about across our advocacy priorities in research is ensuring that the data is reflective of the patient needs. So, this is very much aligned with our advocacy goals, as well," says Jalowsky. "When patient advocates are involved in this conversation up front, their insights can really help inform what the question is and make sure they're involved right from the start."

Pfizer shared a draft of the survey questions with the Sick Cells team, which suggested a number of possible improvements. One suggestion was simplifying the format of the survey. Another was making the questions more inclusive to help ensure that populations that are often overlooked—such as caregivers of adults with sickle cell disease—were involved. "Pfizer was very open to any of the feedback that we gave them," says Jalowsky.

Jalowsky says her team requested that the survey include concerns women with sickle cell disease face. "In addition to prioritizing issues that impact men, we advised them to also consider prioritizing women's health issues, like infertility and pain during menstruation," she says.

In its final form, the survey asked patients about the number of pain crises they have had in a year. It also had them rank what was important to them and what was important to caregivers regarding treatments. Sick Cells distributed it to patients with the help of community health workers and community organizations. Their recruitment strategy, says Jalowsky, included seeking responses from underrepresented communities—including older adults, who are often ineligible to participate in clinical trials because of their age.

Lewis, who helped distribute the survey and collect responses, encouraged people to complete the survey, explaining that more respondents would lead to a greater understanding of sickle cell disease. The experience, he says, inspired more trust in Pfizer and clinical trials in general. “We already are an underserved community, just being people of color, and that's a hard pill to swallow as it is,” he says. But in this instance, he felt valued for sharing his experience. That, in turn, gives him hope for developing future therapies. “By collaborating and doing more initiatives with the actual patient voice, that will always get you to the next level,” says Lewis.

Empathy's role in science

In Brett Hauber’s job as a Patient Preference Expert at Pfizer, everything hinges on the treatment of the individual being treated. “The patient is the one that bears the risk. The patient is the one who actually has to show up for treatment or take the treatment every day,” he says. “No matter what we do, we should always have that in mind. No one can speak for the patient the way a patient can speak for a patient.”

Hauber says understanding what patients want and need, and using those insights to inform therapeutic development and clinical trial design, can be a multi-year process. The Sick Cells patient preference survey, for example, is just one piece of information that could go on to shape future work around therapies aimed at treating sickle cell disease. By bringing the patient voice in early, Hauber says, decision makers at Pfizer have a deeper reservoir of data about patients’ concerns and priorities to draw from in future work.

One learning from the survey, for example, illuminated the values that respondents placed on what a potential treatment could do. Options included, “shorten the length of pain crises,” “reduce the level of pain,” “reduce the risk of having a stroke,” “reduce fatigue,” and “reduce the risk of having organ damage.” Patients were told they had 100 points to allocate among the five outcomes to demonstrate how important it was to improve each of them.

Hauber says his team was surprised by the responses. “We all assumed that pain is the most important thing to address because it is something that people experience regularly,” he says. “End organ damage is more of a longer-term consequence that we always assumed was in the back of people's minds. In this case, it turns out that they said, yes, those other things are important, but ultimately, we need to address the issue of end-organ damage. Because once I damage my organs, I can't repair them.”

Messages from patients are essential to hear, not just for Pfizer. The study results are available to share with Sick Cells participants and other members, as well as the healthcare community at large, including doctors, insurance companies, and caregivers. In fact, the results are available to anyone who might benefit from a deeper understanding of what patients are experiencing, what they value, and what they need. “The real impact is when we can take what matters to patients and turn that into data to inform a decision,” says Hauber. “It becomes evidence rather than an anecdote.”

Giving back to the community

In October, at the 2022 National Convention of the Sickle Cell Disease Association of America, the Sick Cells team shared the results of the patient preference survey, in plain language designed for anyone to understand. “Pfizer has done a great job of preparing the lay summary, and were thrilled to present those findings and really presenting this project, and what kind of role Sick Cells had,” says Jalowsky. “It’s a way that the participants in the research and other community members can receive the results back, and that’s something that's really important to us.”

Kelly says that it’s important for Pfizer, too. “Our goal is to bring treatment options to sickle cell patients.,” she says. “This will only be achieved by working together.”

As someone who has invested years in building relationships with patients and patient advocates, Kelly gets excited when she talks about the partnership with Sick Cells. "In so many ways, the collaboration with Sick Cells symbolizes the very definition of putting the patient at the center of the work we do at Pfizer," she says. The result, she hopes, may one day change lives.

“We’re working side by side and have the same mission,” she says. “Our goal is to bring hope to patients. Collectively, we all want patients to have as many treatment options as possible and one day, if possible, a cure.”

References:

1. Data and Statistics on Sickle Cell Disease. CDC. Available at https://www.cdc.gov/ncbddd/sicklecell/data.html. Page last reviewed October 16, 2022. Accessed August 25, 2022.

2. Sickle Cell Disease. NORD. Available at https://rarediseases.org/rare-diseases/sickle-cell-disease/. Page last reviewed on July 22, 2022. Accessed October 16, 2022.

3. What is Sickle Cell Disease? National Heart, Lung, and Blood Institute. Available at https://www.nhlbi.nih.gov/health/sickle-cell-disease. Updated July 22, 2022. Accessed October 16, 2022.

, The insights gleaned from respondents with sickle cell disease and their caregivers will help Pfizer scientists better understand patients' priorities.

Originally published, Saturday, November 5, 2022