Questioning Medicine to Get Answers for Patients

We've formed an inclusive environment that allows patients to be involved from day one, but we have room to grow.

I began my journey running the medical and surgical intensive care units at Ohio State University Hospital. The moment that impacted me the most, and put me on the trajectory I've taken, was when I was identified to be the supervisor.

I'll never forget noticing on the door that family visiting hours were restricted to five minutes on the hour, twice in the morning and twice in the afternoon. Innovations in medicine 35 years go hadn't really occurred yet; we lost people that we certainly wouldn't lose today. Knowing that most patients weren't going to survive the ICU, I remember sitting in a meeting and saying to my colleagues, "So we give people, and those who they care about, 20 minutes a day, and not even at one time, to say everything they need to say?" The answer was simply, "Yes, because we can't afford for them to be in the way." It raised a blatant red flag for me in the way we were interacting with patients and their families. We desperately needed to change our outlook; patients and their families should never be considered "in the way."

We began considering if there was any real harm if we let a partner or family member come into the room and sit through the hours with the patients. What harm would it do to have a hand to hold, a shoulder to lean on, and an advocate by their side? How intrusive would that really be for the doctors, and, in comparison, how much comfort would it give? Ultimately the question we posed was: Could this movement toward inclusivity of patients and their familial needs change the outcome?

That was the first set of questions, and since then I have never stopped questioning medicine.

Fast forward to today, and we are seeing patients at the forefront. Instead of restrictions and rules, we are seeing patients more in the spotlight for treatment and decision-making. If you think about it, patients are the reason—for cures, for hospitals to exist, and for medicine in general. Although we're evolving, I'm concerned that in the past and even today we have let medicine simply be treating a disease instead of bettering a human being. I remember seeing attending doctors walking around with medical students, dictating treatments and decisions in a paternalistic kind of way. That kind of approach doesn't put each patient in the context in which they live. We have to look at the holistic view of each patient's life and put that into our perspective when treating them.

If we look now at patient-focused drug development, this is exactly where patients are and can be empowered. Patients live with their disease every day. They know the effects, the realities, the symptoms, the needs, the pain, the clues, and so much more. Because of this, patients need to be empowered to understand what's wrong, how it affects their overall health, and how it impacts their families.

Organizations and foundations can use the individual and collective voices to make a major impact. We can go to the FDA and regulatory agencies involved in the making of drugs and say, "We're experts in this particular disease." Agencies don't look at the data of a patient living with the disease day in and day out. This is where patients come in. Patients and their family members are the ones constantly doing a benefit-risk evaluation. The drug-making agencies need to be using this patient benefit-risk constantly in the forming of a solution. What risks are patients willing to secede to with the benefit that comes from a particular drug? Patients can collectively provide data that can impact the FDA's decision-making process.

Groups like Parent Project Muscular Dystrophy take this benefit-risk equation to the regulatory process, advocating for its potential despite the data-driven focus of the past. It's a new kind of data that we offer, illustrating to agencies what patient priorities are, and quantifying the data in real-life context. Stories are impactful, but they need data. Patients and their communities are now faced with the opportunity to provide the expertise to these agencies—granting immense influence from those who know a disease best.

We—patients and families—are setting the stage, developing models and systems that go beyond one-person stories and experience. We're developing a way to include the patient voice in a systematic, collective way that the regulatory process can put into play. How could we imagine that patient voice doesn't have a meaningful, integral, and relevant impact on decision-making? We've formed an inclusive environment that allows patients to be involved from day one to day forever—but we have room to grow.

Many doctors used to say that patients can't understand medicine. But if we provide the facts in a way patients can understand, they are very willing and anxious to take a role in their own lives and the lives of those they care about. It's a compelling trajectory.

Two hurdles that keep me up at night

1. The outcome measures of any disease have to be thought about early, early on. If not, functional outcome measures become restrictive on which patients can participate and when. We have to provide and design trials that are inclusive and grant opportunity to every patient.

2. Drugs are approved one by one, but patients may be better served with combinations. We need to consider the benefits and risks of this process.