VIEWPOINT

The Most Urgent Voice in Eye Research—The Patient's

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"Nothing about us without us."[1] This common refrain among the patient advocacy community emphasizes the importance of including patients in the research process early and often to ensure scientists think beyond the eye, beyond the disease, and consider the individual who will benefit from their labors.

When we think of eye health research, the National Eye Institute comes immediately to mind. With roughly $810 million in annual expenditures, the National Eye Institute is the world's largest research funder. And yet, multiple sectors collaborate to advance the state of eye health research. The pharmaceutical and biotechnology sectors bring new and improved treatments to market. Universities, sometimes through philanthropy, conduct their own research and train future scientists. And the nonprofit sector advances research from multiple vantage points through convenors, such as the Association for Research in Vision and Ophthalmology, federal funding advocates like the National Alliance for Eye and Vision Research, and research funders, such as the Foundation Fighting Blindness and Research to Prevent Blindness.

The goal of research, of course, is to advance outcomes that benefit the patient. And so, the patient voice must be heard throughout the process. While this engagement has evolved slowly, we are seeing its intrinsic value being recognized and sought out more each day. This is in part due to the US Food and Drug Administration's (FDA) Patient-Focused Drug Development (PFDD) initiative, established in 2012, to help ensure patients' experiences, perspectives, needs, and priorities are captured and incorporated into drug development and evaluation.

Yet well before PFDD—for more than a century, in fact—patient advocacy organizations (PAOs) have been supporting patients in finding community, educating them about systems of research, policy, and public health, and guiding them in sharing their stories to advance policy and research. There are PAOs across many health care conditions, with Prevent Blindness among the nation's oldest. Not known primarily as a research organization, the very origin story of Prevent Blindness is nonetheless one of research and patient advocacy coming together to advance the nation's public health. In 1880, Carl Siegmund Franz Credé, a German gynecologist and obstetrician, discovered the value of using silver nitrate eye drops as an antiseptic for the prevention of ophthalmia neonatorum, also then known as *babies' sore eyes*, a blinding condition among infants. During a 3-year period, Credé treated 1160 newborns with silver nitrate with only 0.15% of them developing the disease.[2] A sight-saving therapy was unearthed.

Unfortunately, what we know today, that a research-based treatment for a disease does not automatically solve a public health problem, was just as true back then. Thus, in 1907, a report issued by F. Park Lewis, a New York ophthalmologist, highlighted that 28% of children in the state's schools for the blind had lost sight needlessly due to ophthalmia neonatorum, despite the discovery decades earlier of the preventive nature of silver nitrate. Shortly after its publication, Louisa Lee Schuyler, a leader in New York's charitable society (also, the great granddaughter of Alexander Hamilton), received Park's report in the mail. Upon seeing photographs of children blind from babies' sore eyes, one photograph's caption—"Unnecessarily blind"—spurred her to action. In 1908, Schuyler and Lewis formed the Committee on the Prevention of Blindness of the New York Association for the Blind, which spent the next decade successfully advocating for policies requiring the use of this treatment for all newborns. The committee, now known as Prevent Blindness, shines as the earliest example in the US of patient advocacy and research coming together on behalf of the nation's eye health.

Today, 115 years after patient advocates first brought the fruits of research to the mainstream of pediatric eye care, we find ourselves in a time of incredible innovation in ophthalmic research, promising numerous sight-saving advances on the horizon. Our experience suggests that facilitating successful outcomes and uptake of new therapies requires that patients be engaged from the start. Patients and PAOs can guide scientists in determining what conditions need research, provide insights to clinical trial design, support the diversification of trial participation, serve on patient advisory councils, and provide real-world evidence and patient perspectives to the FDA and PFDD. Furthermore, they can share results with key decision makers to promote policy changes necessary to ensure treatments get to the intended patient populations, establish patient and data registries to facilitate faster development of new discoveries, and educate companies about the communities they hope to reach as therapies come to market.

Of course, many of these actions can be daunting, particularly for patients already overwhelmed with the challenges of managing their condition and navigating an often burdensome health care system. So one important role that PAOs often play is to work with patients and care partners to educate and prepare them to step into these advocacy roles they are often pulled into due to their circumstances and their drive for new therapies. At Prevent Blindness, the ASPECT (Advocacy, Support, Perspective, Education, Communication, and Training) patient engagement program helps each participant shape their vision story to influence various audiences, provides them guidance on how to foster desired change, and guides them in gauging when, where, and how best to interject.

In recent months, the ophthalmic research community has seen 2 groundbreaking advancements with the

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first-ever FDA-approved pharmacological treatments for both retinopathy of prematurity (ROP) and geographic atrophy (GA), the advanced form of dry macular degeneration. The patient community was actively involved in bringing both therapies to market.

At the FDA hearing for the approval of the ROP treatment, one mother shared her challenges as a single working mom raising a child born with ROP. Her words stressed the urgency of bringing new options to bear for patients, parents, and health care professionals, as well as the importance of ensuring that parents and health care professionals are well educated as new treatments emerge. "Based on what you all do today, parents in the near future may have an additional option for ROP, as well as the information to understand the pros and cons of different treatments, the possible outcomes, the safety, and the impact on their kids and families," she expertly and passionately testified.[3]

Similarly, the patient community played a critical role along the long road to approval of a treatment for GA. ASPECT-trained advocates shared their lived experiences with multiple research groups and provided feedback on study design, including what clinical end points were relevant for patients, what enrollment protocol might improve engagement, and how realistic the trial procedures might be for participants. As part of a patient advisory panel, they also developed and reviewed communications materials to assist health care professionals in educating patients about GA. Simultaneously, Prevent Blindness helped design, promote, and recruit for a patient survey study to better understand the clinical, financial, and humanistic burden of GA on patients.

These are but 2 examples of how patient engagement contributes to research outcomes by focusing on the person living with the condition for which a treatment is being sought. The interdependency is clear—patients are ultimately made better through evidence-based and patient-centric research, just as the research process is made better by the engagement of diverse patient voices across the development spectrum.

ARTICLE INFORMATION

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