Epilepsy & Behavior 94 (2019) 307

Contents lists available at ScienceDirect

Epilepsy & Behavior

journal homepage: www.elsevier.com/locate/yebeh

Editorial

The voices of patients and families must be heard

The long road of drug development and approval is traveled by numerous stakeholders, some for only limited portions and others from start to finish. Among the latter are patients and their families. The roles that patients and their families have in drug development and approval have evolved through their activism, driven in part by desperation for improved therapies, increased grass roots organization among disease-specific patient communities, and a slowdown in drug discovery and early-stage preclinical and clinical development at pharmaceutical companies.

Patients and their families are there at the beginning of the journey, often when no one else is showing an interest, to strongly and repeatedly articulate why they need new therapies. Once their voices attract enough attention and funding, sometimes from patient groups themselves, early-stage translational research may commence, and if successful, lead eventually to clinical trials. While some of the unmet needs voiced by patients and families may be built into clinical trials as outcomes, such as the length of survival or reduced frequency and severity of symptoms, others may not be readily or quantitatively measurable, or may not fit into a sponsor's plans for labeling. Further, the statistical analysis of outcome measures may not readily translate to day-to-day changes in the lives of patients that meaningfully resolve the unmet needs they previously identified. Therefore, near the end of the road, when a therapy is under review by the Food and Drug Administration (FDA) for possible approval, patients and their families are there again to publicly state the reasons they seek new therapies and to discuss the therapy under review in the context of their lives. Their testimony about the realities of living with their disease, delivered in an open forum before FDA advisory committees, may result in committee members reevaluating the benefit-to-risk ratio of the new therapy as determined by protocol-specified trial outcome measures.

In their interesting recent analysis, Arthur et al. found a significant relationship between financial support from drug companies for public speakers providing testimony in front of the Peripheral and Central Nervous System Drug Advisory Committee (PCNSDAC) of the FDA and the likelihood of that testimony being favorable towards the therapy [1]. Many public speakers in this study were personally impacted by the disease for which the drug was indicated, and some even had taken the drug. One logical inference of Arthur et al.'s findings is that the publicly stated viewpoints of a patient or family member in front of the PCNSDAC would be potentially tilted more in favor of a new therapy under review when speakers were selected and encouraged to provide testimony by a pharmaceutical company and provided financial support to do so. Another implication is that committee members should accordingly interpret the statements of drug company-supported patients and family members as possibly biased by compensation. Each of these interpretations seem unlikely but are nonetheless important to address and eliminate so that there is no doubt about the veracity of patients and their family members who speak before FDA advisory committees.

The crux of this potential conflict appears to be the financial support for attending advisory committee meetings. Therefore, recognizing the importance of this testimony in the process of drug approval, the FDA should explore other mechanisms for compensating the expenses of a reasonable number of patients and family members to attend these meetings or perhaps to facilitate the remote participation of speakers in the open forum so that financial support is not needed. The truths spoken by patients and their families at FDA committee advisory meetings are too important to be questioned because of possible financial conflict of interest. They alone among all the stakeholders in drug development and approval traveled the long road from the very beginning to the very end. They should be honored for their passion and steadfast dedication.

The voices of patients and families must be heard and listened to, not just at advisory committee meetings, but all along the journey of new therapy development. They are the inspiration and beacon showing the rest of us what we all need to do — together.

Conflict of interest

The author has no conflict of interest.

Reference

[1] Arthur W, Austin J, Wayant C, Vassar M. Association of conflicts of interest for public speakers for the Peripheral and Central Nervous System Drugs Advisory Committee of the US Food and Drug Administration with their statements. JAMA Neurol 2018. https://doi.org/10.1001/jamaneurol.2018.3997 Dec 17. [Epub ahead of print].

Steven C. Schachter
Departments of Neurology, Beth Israel Deaconess Medical Center, Massachusetts General Hospital, and Harvard Medical School, United States of America
E-mail address: sschacht@bidmc.harvard.edu.

7 January 2019

https://doi.org/10.1016/j.yebeh.2019.01.013