PATIENT EXPERIENCE DATA COLLECTION


This information can provide regulatory authorities, payers, providers, and patient communities with a more holistic view of a therapy's impact.

BY RICHIE KAHN, MPH, AND JENN MCNARY

A traditional clinical endpoint is not the only indicator that an investigational therapy holds promise. Patient experience data can be utilized to inform clinical development, contextualize the impact of a treatment, support regulatory submissions, and more.

"PATIENT EXPERIENCE DATA COLLECTION IS A METHODOLOGY DESIGNED TO COMPLEMENT, NOT REPLACE, CLINICAL OUTCOMES ASSESSMENTS AND ULTIMATELY BETTER INFORM FUTURE RESEARCH."

THE VALUE OF PATIENT EXPERIENCE DATA

Section 506(c) of the Food, Drug, and Cosmetic Act enables the FDA to grant accelerated approval to "… a product for a serious or life-threatening disease or condition … upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments."[1]

Surrogate endpoints are essentially indicators that an investigational therapy has value. Although a clinical trial for cancer will focus most on increased survival or improved quality of life, a decrease in tumor burden might be an adequate early indicator of these results. Thus, a decrease in tumor burden may be used as a surrogate endpoint to push for earlier approval of a promising new therapy.[2] Recently, patient experience data was used in an open public hearing on an investigational gene therapy for Duchenne muscular dystrophy. The treatment was ultimately granted accelerated approval by the FDA based on a surrogate biomarker for efficacy.

Clinical benefit is more than just a quantitative score or numbers in a spreadsheet. If a sponsor wants to convince regulatory authorities of the rationale for using a proposed surrogate endpoint that demonstrates how the benefits of an investigational therapy outweigh potential risks, it might consider patient experience data collection. Section 3002 of the 21st Century Cures Act (legislation intended to accelerate the pace of therapeutic development) demonstrates the FDA's commitment to considering patient experience data as part of its regulatory review.[3]

By integrating patient experience data into clinical development roadmaps, sponsors can increase their likelihood of designing a commercialization program that adequately addresses patient preferences and unmet needs. Failure to do so can result in unnecessary delays and extended timelines, and time is crucial when it comes to progressive vision loss. A formalized patient experience initiative can help significantly to ensure alignment.

Patient experience research is intended to explore how patients feel and function as a result of clinical trial participation. Patient experience data can be collected outside of the formal clinical trial protocol and used to support regulatory submissions.

A STEPWISE APPROACH TO COLLECTION AND SUBMISSION

Patient experience data collection begins before a sponsor even initiates a clinical trial design process. It starts by learning more about how patients with a particular disease feel and

function. Then, patient experience data is collected throughout the clinical development lifespan to contextualize the impact of a treatment. This can support regulatory review and approval in a variety of ways.

Clinical outcomes assessments, or COAs, have traditionally been used to evaluate patient experience. Patient experience data collection is a methodology designed to complement, not replace, clinical outcomes assessments and to ultimately better inform future research.

Several benefits are associated with patient experience data collection. This practice helps to:

- Contextualize traditional clinical endpoints;
- Identify how a treatment affects patients' activities of daily living and how patients feel and function at home; and
- Measure change over time.

When using patient experience data, planning is key. Sponsors must determine the information that is most meaningful and the appropriate timepoints for its collection. For example, is the goal to assess patients' experiences in a clinical trial, or is it to identify how visual function impacts patients' activities of daily living or their emotional well-being?

If a sponsor learns at baseline that a patient with glaucoma no longer drives because they are concerned about the deterioration of their visual field, the company can recognize that this lifestyle change may negatively affect the patient's independence and mental health. If visual acuity is a primary clinical outcome measure and a patient experiences an improvement, this will likely impact how they feel and function, and perhaps they will be more likely to drive and feel less anxious. At a granular level, this could mean that a patient feels confident enough to drive their daughter to the park for ice cream and a ride on the merry-go-round. This type of patient experience data can help clinical teams better understand the meaning behind traditional clinical endpoints. Imagine the impact of capturing these experiences on video!

THE BIGGER PICTURE

The utility of patient experience data does not stop with regulatory approval. This information can impact access, reimbursement, and policy decisions, and it can help with care considerations and treatment guidelines. It can also help prescribers solidify treatment decisions, which is especially important with indications such as glaucoma, for which a number of approved therapies are available.

Although not a replacement for clinical outcomes assessments, patient experience data collection can help provide regulatory authorities, payers, providers, and patient communities with a more holistic view of a proposed therapy and its potential impact. ■

1. Guidance for industry: expedited programs for serious conditions – drugs and biologics. FDA. May 2014. Accessed July 10, 2023. www.fda.gov/media/86377/download
2. Surrogate endpoint. National Cancer Institute. Accessed July 10, 2023. www.cancer.gov/publications/dictionaries/cancer-terms/def/surrogate-endpoint
3. Voice of the patient report. FSHD Society. Accessed July 10, 2023. https://www.fshdsociety.org/fsh-events/vopf/

ABOUT THE AUTHORS

Richie Kahn, MPH, is a public health professional by training, clinical researcher by trade, and patient advocate by necessity. He is a cofounder of and principal at Canary Advisors, a patient engagement firm that partners with organizations looking to better align their clinical development programs with patient wants and needs.

Jenn McNary is an educator and advocate with 18 years of experience on the front lines of rare disease clinical development, drug approval and reimbursement, and advocacy. Jenn is a cofounder of One Rare, a nonprofit that serves young adults aged 18 to 35 years who are impacted by rare disease, as well as a cofounder of and principal at Canary Advisors.

RICHIE KAHN, MPH
- Cofounder and Principal, Canary Advisors
- richardgkahn@gmail.com; Instagram and Twitter @rbaltikahn; www.linkedin.com/in/richiekahn
- Financial disclosure: None

JENN MCNARY
- Cofounder and Principal, Canary Advisors
- Cofounder, One Rare
- jenn@canaryadvisorsllc.com; Instagram @raremomof4 and Twitter @jennmcnary; www.linkedin.com/in/jennifer-mcnary-a2723669
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