CTTI Monthly Webinar
Patient Engagement in Action: Insights from Patients & the FDA
Question & Answers
November 21, 2019

Q1. Please share the resource guide for the Patient Listening Sessions. Also, how do the Patient Listening Sessions differ from Focus Groups?

A1. Information about Patient Listening Sessions is available at https://www.fda.gov/patients/learn-about-fda-patient-engagement/patient-listening-sessions.

The Staff Manual Guide provides information about the U.S. Food and Drug Administration's (FDA) procedures for the management of cross-center Rare Disease Patient Listening Sessions (RDPLS).

Patient Listening Sessions are similar to focus groups in that they provide an opportunity to learn about participant perspectives. Unlike focus groups, they are not focused on experience specific to any particular product, service, or concept. The listening session discussion is typically moderated by a facilitator, compared to a focus group that typically allows more free conversation and discussion amongst the participants.

Q2. Will the CTTI/FDA PEC work with other patient engagement groups working on trial design issues before clinical trial design is set? How do we connect with CTTI and/or the FDA PEC for future collaboration?

A2. The Patient Engagement Collaborative (PEC) is an ongoing, collaborative forum in which the patient community and regulators discuss an array of topics regarding increasing patient engagement in medical product development and regulatory discussions at FDA. This group does not provide feedback on specific trial designs, and CTTI does not provide patient advisory board services.

However, there are many patient groups who do provide feedback on trial designs, and CTTI has a number of resources to support effective engagement between sponsors and patient groups. Please see https://www.ctti-clinicaltrials.org/our-work/patient-engagement/patients-groups-clinical-trials/

For information about the PEC, please contact CTTI Senior Project Manager, Zachary Hallinan, at Zachary.hallinan@duke.edu.

For general CTTI inquiries, contact CTTI@duke.edu.

Q3. Do you have data on the patient portal usage that is available to the public and other researchers?

A3. As we near the one-year mark of the patient portal, we are hoping to share a report that will summarize the improvements made to the portal, as well as the number and type of questions made to date.

Q4. Do you have data or a report on what components and recommendations you used in the design of your patient portal?

A4. The patient portal was designed based on needs identified via public comments to a Federal Register Notice on methods to enhance FDA patient engagement efforts.

Q5. How [can] we connect our patient advocacy organizations with the FDA to collaborate on patient and caregiver conversations data to inform trials in the ICU, TBI, and epilepsy? I noticed these are all areas that are not represented in your current collaborations.

A5. Critical Path Innovation Meetings (CPIM) is a way to discuss methods, technologies, or approaches that may improve and advance the development of drugs through a scientific discussion between FDA staff and stakeholders (e.g., industry, academia, patient advocacy groups, government agencies). Contact: CPIMInquiries@fda.hhs.gov.

Q6. You mentioned that IRB approval is not needed for Patient Engagement (PE) activities. Does this also apply to activities that are conducted during the trial, e.g. study participant experience survey?

A6. Research involving human subjects requires IRB approval; therefore, conducting an experience survey with research participants would likely require IRB approval. We suggest confirming local requirements with your organization's research/ethics office, and accessing resources and regulations available at https://www.hhs.gov/ohrp/.

Q7. Is there an FDA registry related to rare diseases in the pediatric population? If not, where is it possible to find this type of registry?

A7. The FDA does not maintain registries. CTTI suggests contacting the patient advocacy organization dedicated to the rare disease in which you are interested. More information about rare disease registries is available at https://www.ncbi.nlm.nih.gov/books/NBK208609/.

Q8. Does FDA measure the impact of Patient Engagement efforts? If so, are results of metrics publicly available?

A8. FDA strives to ensure that patient engagement programs and initiatives inform regulatory discussions to ultimately help patients manage their diseases and conditions and improve quality of life. For example, the Patient Affairs Staff (PAS) conducted pilot listening sessions to assess the value and establish an efficient process. PAS evaluates the impact of these sessions through feedback received from FDA staff and patient/caregiver participants. Information gained from listening sessions has informed FDA staff what is important to patients, guidance development, public meeting agendas, and discussions with product sponsors among others. Currently the post-listening session feedback is not publicly available.

In addition, the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) will be releasing a report assessing the use of patient experience data in regulatory decision making by June 2021. It would be helpful to hear from stakeholders what kind of impact measures they would be interested in seeing, both as part of the report, and outside of the report.

Q9. For externally led PFDDs, does the host organizations typically pay for the time of the external leader, for the cost of venue, for travel costs? Does Industry sponsor these meetings?

A9. It is at the patient organization's discretion to determine its capabilities and resources for planning an externally led patient-focused drug development (PFDD) meeting. The planning of an externally led PFDD meeting can be done without being resource intensive (e.g, FDA does not encourage enlisting event planners, consultants, scientific writers, or other external resources on your team, especially when resources may be limited). The key is to begin planning early. FDA does encourage patient organizations to consider including externally led PFDD sessions as part of annual meetings or symposiums to help maximize resources.

A patient organization may seek financial sponsors (e.g., medical product developers, larger patient organizations) for their externally led PFDD meeting. To facilitate transparency, the patient organization should identify any financial sponsors in their letter of intent (LOI) and any subsequent meeting materials and deliverables. The patient organization and any other meeting planning team members and collaborators are also encouraged to disclose any interactions (financial and non-financial) that could be considered relevant to the planning and conduct of the meeting. All decision-making related to the externally led PFDD meeting (e.g., development of agenda, discussion/polling questions, selection of patient panelists) should be done independent of medical product sponsor input.

Q10. Can similar diseases be combined for a single PFDD? Is it possible to combine diseases for a meeting but still separate them to highlight key differences?

A10. Yes. Similar diseases can be combined for a single PFDD if the questions being asked are relevant to both diseases. For example, in September 2018, the Hereditary Neuropathy Foundation held a meeting to hear directly from individuals living with Charcot-Marie-Tooth and inherited neuropathies and their loved ones on the impact of these disorders on their daily lives, and their perspectives on approaches to treating Charcot-Marie-Tooth and inherited neuropathies. Other organizations focusing on similar rare diseases have come together to organize a day of meetings in the same location, where one meeting was held in the morning and another in the afternoon.

Q11. From the perspective of a patient organization, what are the key considerations for getting involved with patient engagement activities with FDA and sponsors? How might sponsors help patient organizations get involved?

A11. We encourage you to review CTTI's Patient Groups and Clinical Trials Recommendations. These recommendations and resources provide best practices that sponsors, patient groups, and other stakeholders can use to ensure the relationship is mutually beneficial.

FDA's Patient Affairs Staff is committed to working with individual patients and caregivers, and as well as patient advocacy organizations. If you would like to learn more about ways to work with FDA please see information about *Initiatives for Patients to Engage with FDA* on FDA's *For Patients* web page, or contact PatientAffairs@fda.gov. We are happy to work with you to help better understand your needs and suggest an appropriate avenue for engagement.

Q12. As we know, lower socioeconomic status correlates with health disparities. Yet the burden of being "at the table" can be very hard for this population -- losing a job if not present, childcare costs, even knowing there are opportunities to give comments. I am wondering if there are any intentional outreach or community-based initiatives to assure this population has equal opportunity for involvement?

A12. The FDA's Office of Minority Health and Health Equity (OMHHE) aims to protect and promote the health of racial and ethnic minority, under-represented, and underserved populations through research and communication that addresses health disparities. FDA's OMHHE also works across the Agency and with public and private sector stakeholders to strengthen FDA's ability to respond to minority health concerns. More information on OMHHE and their work can be found at https://www.fda.gov/consumers/consumer-information-audience/minority-health-and-health-equity

When planning FDA-led PFDD meetings, FDA has taken steps for intentional outreach. We have worked with advocacy groups to make people aware of the PFDD meetings and the opportunity for comment. All PFDD meeting are webcast and recordings are available. In the past, we have also worked with advocacy organizations to provide "listening stations" across the

country where stakeholders could come together to view the meetings and participate remotely. We are open to other recommendations.

Q13. Recently [a pharmaceutical company] was able to incorporate a Patient Experience section in a drug label. Have there been other examples of this? Also have PROs been used as primary endpoints for registration/approval? In what therapeutic area(s)?

A13. Patient-reported outcomes (PROs) have been used as primary endpoints. We refer you to CDER's Clinical Outcome Assessment Compendium https://www.fda.gov/media/130138/download for a non-exhaustive list by therapeutic area.