



January 8, 2019

Division of Dockets Management (HFA-305)
U.S. Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Docket No. FDA-2018-N-4171: PEAC Discussion 2018 - Patient Engagement in Clinical Trials

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Food and Drug Administration (FDA) for the opportunity to provide comments on the Agency's "PEAC Discussion 2018 - Patient Engagement in Clinical Trials."

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

We are pleased to support all FDA efforts to better integrate the patient voice within medical product development and review. The Center for Device and Radiological Health's (CDRH) Patient Engagement Advisory Committee (PEAC) is one of the most exciting programs FDA is undertaking to better accomplish this goal. We have been supportive of its work since its inception and are pleased with its progress thus far.

We were grateful for the PEAC's invitation to testify at its inaugural meeting in 2017. At this meeting, we presented several considerations for the PEAC on how to better recruit and retain rare disease patients in medical device clinical trials. Several of those comments will be reflected again in this document.

Overall, we are pleased with the PEAC's discussion document on patient engagement in clinical trials. Through this document, the PEAC shows a clear understanding of the importance of patient viewpoints in medical device development. Our specific recommendations for improving the document and developing further guidance as well as our strong support for specific aspects of the document are explained below.

Is the proposed definition of “patient engagement” clear? If not, what changes provide further clarity to define and distinguish patient engagement activities from other patient-related activities? Is the proposed term “patient advisor” clear? If not, is there another term that better reflects a patient’s role in the context described in this document?

We believe that the definition of “patient engagement” is clear but somewhat limited. First, the proposed definition encompasses many situations in which patients offer their viewpoints to decision makers for consideration. However, this definition does not include an even stronger form of patient engagement: allowing patients to take part in the decision-making itself.

While we encourage medical device sponsors to collect and consider patient experiences as they develop their products, we also encourage them to actually include patients with the relevant condition, or their representatives, within the decision making process. This proposed definition of patient engagement does not encompass this second, and more patient-focused, scenario.

Second, FDA’s proposed definition could better include the equally important patient perspectives collected during the testing of these devices. The patient is vital to the entire research process. Patient involvement ultimately ensures that researchers ask the right questions to address the needs of the patient community while keeping safety at the forefront.

The definition of “patient advisor” is clear but perhaps too broad. Under this definition, patient advisors could be anyone from a caretaker with direct and intense understanding of a disease to other stakeholders who are perhaps only casually interested. We encourage FDA to consider whether caretakers should have their own category in order to reflect their own unique experiences and perspectives.

Finally, we thank FDA for avoiding the term “human subject” as it is indeed demeaning and devaluing. While the term “patient research participants” is a step in the right direction, we also ask that FDA ensure that this term become not simply a euphemism for the same demeaning practices but, instead, a very different and more empowering title than “human subject.”

Are the value and beneficial impact of engaging with patients during the clinical trial process clearly identified and compelling? If not, how could this be strengthened?

Similar to our commentary on the first question, we believe this section can be strengthened by re-emphasizing the value of patients and their representatives participating in the decision-making process itself. For example, rather than stating that “patients may provide recommendations that positively impact how a study is designed and conducted,” FDA could emphasize that patient participation in deciding the structure of medical device trials could lead to more patient-focused trials.

Other than this omission, FDA makes a compelling case for patient participation. An additional beneficial impact of patient participation that FDA could consider is that patients become more invested in the success of the product as it better targets their health and quality-of-life needs.

Does this discussion document identify major barriers and challenges currently impeding sponsors from engaging with patients throughout the clinical trial process? If not, what additional barriers and challenges limit patient engagement opportunities? What additional steps/processes can be considered to address such barriers and challenges to further promote patient engagement?

FDA successfully cites various challenges to engaging with patients throughout the clinical trial process. There are various other challenges that we wish for FDA to consider, though, including:

- Institutional Review Boards (IRB) can be reluctant, even intransigent, to innovatively approaching trials. IRBs can also be reticent to accept other IRB decisions, which can greatly delay trials.
- Site investigators may be ready and willing to engage with patients, but they might not know how. Further educational outreach to site investigators could obviate this barrier.
- The very nature of rare diseases, their low prevalence, creates challenges for sponsors to engage with patients in a broad and unbiased manner.
- Patient communities may be fragmented and competitive, and it may be difficult to engage with a patient community as two, or more, factions may say very different things.
- Even if patients want to engage, their caregivers may not be able to for various different reasons, potentially limiting the opportunities for the patient to participate.
- If patient engagement must happen in-person, travel and lodging barriers may prevent them from doing so.
- Patients may decline opportunities to participate due to the lack of communication around how valuable their perspectives are.

These are just several of many barriers that patients, sponsors, site investigators, and more face in engaging with patients as part of the medical device clinical trial process.

Do the opportunities and timeframes outlined in this document for engaging patients include the major categories of interest to patient, sponsor, provider, and clinical researcher stakeholders? If not, what additional measures are relevant for consideration?

We are particularly grateful for FDA's emphasis on the importance of starting patient engagement early on within the device development process. We could not agree more.

We have several small suggestions to improve this section. First, if sponsors are encouraged to educate patients "about clinical trials, the approaches to managing the condition of interest, and how the device may work," we need to be sure this education does not inappropriately skew patient opinion or viewpoints. Unfiltered and unadulterated patient input can often be the most valuable.

Second, we encourage FDA to include more quality-of-life improvement efforts within its list of "examples of patient engagement activities to potentially improve clinical studies." It is often the case that the patient's experience outside of any clinical situation can be the most impactful in participation and retention.

Third, we suggest that FDA emphasize that patient organization participation can be helpful in pursuing these aims and opportunities, rather than being a separate, siloed opportunity.

Fourth, FDA should include activities that ensure that the benefits of engagement and participation can continue for the patient following the conclusion of the trial. This could include access to the resulting data or even access to the investigational device through an expanded access program.

Are there other opportunities not addressed in this document that FDA could consider to better facilitate patient engagement in clinical trials?

We would ask that FDA be more mindful of the caregiver's role throughout the document as their experience and engagement can often be almost as important as the patient's. FDA could also better integrate patient organizations and their potential role within the document.

We thank FDA for the opportunity to comment, and we look forward to working with FDA to further integrate the patient voice into medical device development. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org, or 202-545-3828.

Thank you in advance for your consideration.

Sincerely,

A handwritten signature in black ink, appearing to read 'Paul Melmeyer', with a long horizontal flourish extending to the right.

Paul Melmeyer
Director of Federal Policy