

## FDA and CTTI Patient Engagement Collaborative (PEC) Meeting

January 27, 2022 | 11:00am – 2:00pm EST

**DISCLAIMER:** *The purpose of this meeting was to facilitate a discussion of ideas, and as such, not all of the below content will be within the scope of the FDA or PEC. The views and opinions expressed in this meeting are those of the individual speakers and participants and do not necessarily reflect the official views of their organizations, the FDA, or CTTI.*

### MEETING OVERVIEW

The purpose of this virtual meeting was to facilitate open brainstorming and discussion identifying critical issues for patients related to engaging in clinical trials and/or the medical product regulatory process. The discussion included strategies for increasing patient engagement with the U.S. Food & Drug Administration (FDA) and incorporating patient insight into the regulatory decision-making process. Some of the ideas generated during this meeting will inform future PEC discussion topics.

### DISCUSSION THEMES:

- *The patient voice is a critical part of how the FDA gains an understanding of various diseases and their treatments, and patient insight can complement, support, and strengthen empirical data within the FDA's regulatory decision-making process.*
- *The FDA should consider investing in communications strategies that can reach patients who are not involved in patient advocacy groups, including minority patients and those without access to specialist doctors.*
- *There is a need to help to educate patients/patient groups about their opportunities for engaging with clinical trials and the regulatory process.*
- *Better tools for quantifying patient experience, quality of life, patient-reported outcomes, and activities of daily living are needed.*
- *Sponsors should make clinical trials more accessible to a wide variety of patients by taking steps to mitigate the burdens of participation (e.g., reasonable reimbursement that takes travel costs into account, indemnification support, more sites spread across U.S., more remote options).*

## **KEYNOTE FDA PRESENTATION**

### ***Janet Woodcock, Acting FDA Commissioner***

Acting FDA commissioner Janet Woodcock introduced the FDA's patient engagement efforts, which aim to foster patient participation and incorporate patient experiences into the regulatory process. An important part of the FDA's efforts to improve clinical trials and increase patient engagement is partnership between the FDA and Duke that established the Clinical Trials Transformation Initiative (CTTI) and the Patient Engagement Collaborative (PEC). The patient voice is a critical part of how the FDA gains an understanding of various diseases and their treatments. Patient insight can complement, support, and strengthen empirical data within the FDA's regulatory decision-making process.

Following her opening remarks, Dr. Woodcock answered questions from the members of the PEC.

## **BRAINSTORMING SESSION**

### ***Discussion Themes:***

- *The FDA should consider investing in communications strategies that can reach patients who are not involved in patient advocacy groups, including minority patients and those without access to specialist doctors.*
- *Patient groups need more information about how the FDA operates and how they can engage in the regulatory process.*
- *Patients with rare diseases that may have small/nonexistent patient advocacy groups may need extra guidance on how they can engage with the FDA.*

### ***Review of PEC Scope and Role***

The PEC is an ongoing, collaborative forum in which the patient community and regulators discuss an array of topics regarding enhancing patient engagement in medical product development and regulatory discussions at the FDA. The PEC is a joint endeavor and public-private partnership between CTTI and the FDA. CTTI's mission is to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials. The activities of the PEC may inform relevant FDA and CTTI activities, but the PEC is not intended to advise or otherwise direct the activities of either organization.

### ***Brainstorming Discussion: How can the FDA reach out to patients more?***

The FDA held a brainstorming discussion with members of the PEC to discuss strategies for the FDA to more effectively reach out to patients. The discussion included the following ideas:

- Developing different strategies for reaching patients who are not engaged with patient advocacy groups (e.g., social media campaigns, town halls, media outreach, webinars), including strategies to reach more underrepresented and underserved patients

- Expanding communications strategies to better inform patient groups about the available avenues for patient engagement with the FDA
- Improving the FDA Adverse Event Reporting System ([FAERS](#)) with more defined parameters and better validation so patient groups can reliably track the safety concerns around particular drugs
- Facilitating the development of national disease registries—especially for rare diseases
- Spreading out FDA resources across the United States

## **SMALL GROUP DISCUSSIONS**

The PEC broke up into two smaller discussion groups to discuss critical issues for patients related to engaging in clinical trials and the regulatory process. The groups each identified possible high-priority topics for the PEC to consider in following meetings this year, including the following:

- Improving FDA communications and public educational materials
- Leveraging the strategies and tools developed during the COVID-19 pandemic to benefit patients in clinical trials going forward
- Educating patient groups about the opportunities for patient engagement with clinical trials and the FDA and the potential impacts of that engagement
- Increasing the accessibility of clinical trials by promoting less restrictive exclusion/inclusion criteria
- Promoting more patient-oriented endpoint development
- Increasing the quality of clinical trial institutions and promoting public trust

## **NEXT STEPS**

The FDA and CTTI will be reviewing the discussion points and ideas generated during this meeting. The next PEC meeting, on April 28<sup>th</sup>, will delve a little deeper into some of the topics that were highlighted during this meeting. The PEC is also planning to hold its second joint meeting with the European Medicines Agency (EMA) this summer.