Patient Engagement Collaborative

Overview of the PEC

Stephanie Monroe, PEC Member

FDA U.S. FOOD & DRUG ADMINISTRATION

CLINICAL TRIALS TRANSFORMATION INITIATIVE

What is the Patient Engagement Collaborative (PEC)?

- FDA & Clinical Trials Transformation Initiative (CTTI)
- Modeled on the EMA's Patients and Consumers Working Party (PCWP)
- Purpose: Discussions about engaging patients in medical product development and regulatory discussions

Current PEC Members

- Dawn Aldrich
- Ronald Bartek
- Christine Brown
- Jeffrey Goldstein
- Anne Hall
- Melissa Hogan
- Elizabeth Joniak-Grant
- Nancy Lenfestey
- Isabelle Lousada
- Stephanie Monroe
- Lawrence "Rick" Phillips
- Philip Posner
- Lynne Quittell
- Adrienne Shapiro
- Theresa Strong
- Dave White

Representatives and Membership Selection

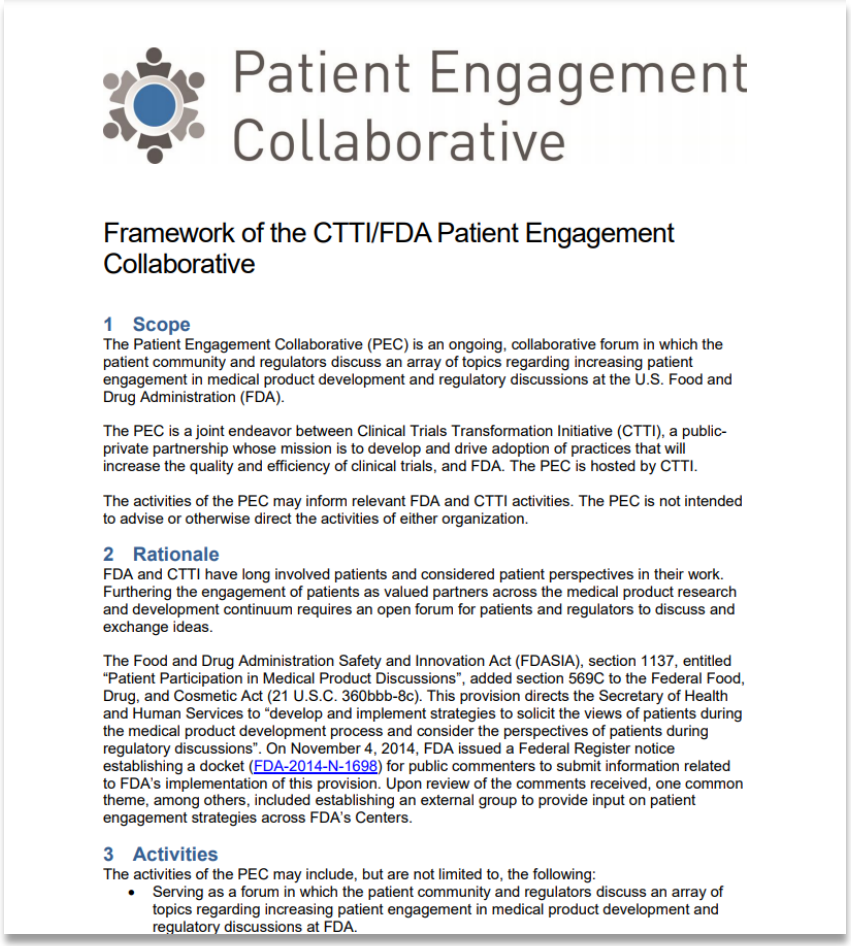
Representatives from the patient community include:

- Patients who have personal disease experience.
- Caregivers who have personal experience supporting someone with a health condition (e.g., a parent, child, partner, family member or friend).
- Representatives from patient groups who have direct or indirect disease experience.

Members selected through an open application process:

- Some members affiliated with patient groups/organizations
- Some are individual members (not required to be affiliated with a patient organization)
- Caregivers (past or present) are included
- Diversity of members is important

How does the PEC work?



Patient Engagement Collaborative

Framework of the CTTI/FDA Patient Engagement Collaborative

1 Scope

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 the U.S. Food and Drug Administration (FDA).

The PEC is a joint endeavor between Clinical Trials Transformation Initiative (CTTI), a public-private partnership whose mission is to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials, and FDA. The PEC is hosted by CTTI.

The activities of the PEC may inform relevant FDA and CTTI activities. The PEC is not intended to advise or otherwise direct the activities of either organization.

2 Rationale

FDA and CTTI have long involved patients and considered patient perspectives in their work. Furthering the engagement of patients as valued partners across the medical product research and development continuum requires an open forum for patients and regulators to discuss and exchange ideas.

The Food and Drug Administration Safety and Innovation Act (FDASIA), section 1137, entitled "Patient Participation in Medical Product Discussions", added section 569C to the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb-8c). This provision directs the Secretary of Health and Human Services to "develop and implement strategies to solicit the views of patients during the medical product development process and consider the perspectives of patients during regulatory discussions". On November 4, 2014, FDA issued a Federal Register notice establishing a docket (FDA-2014-N-1698) for public commenters to submit information related to FDA's implementation of this provision. Upon review of the comments received, one common theme, among others, included establishing an external group to provide input on patient engagement strategies across FDA's Centers.

3 Activities

The activities of the PEC may include, but are not limited to, the following:

- Serving as a 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.

- Includes up to 16 diverse representatives of the patient community
- To help ensure continuity in its activities and organizational knowledge, the PEC maintains staggered membership terms
- Currently no member chairs or co-chairs
- Consensus is not necessary
- Discussion forum
  - Meet virtually approximately monthly
  - Longer working meetings typically held 2-4 times per year, either in-person (in the Washington, D.C. area) or virtually

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

Example discussion topics:

- Making patient engagement more systematic
- How to improve transparency, education and communication
- New strategies for enhancing patient engagement
- New models for patients to collaborate as partners in the medical product development and FDA review process

Inaugural PEC Meeting (2018): Enhancing Communications Between the FDA and the Patient Community

- PEC identified lack of knowledge among the public and patients about:
  - the regulatory decision-making process;
  - FDA's role in the regulatory process; and
  - the agency's structure and organization.

- Patients and advocates may struggle with knowing:
  - how to engage with FDA;
  - what kinds of information to provide; and
  - how to ensure that patients' perspectives are adequately represented.

- Potential actions:
  - provide patients and advocacy groups with easy-to-understand resources about the FDA;
  - increase engagement;
  - build trust in the work of the FDA; and
  - enhance the quality of communication between the FDA and the patient community.

PEC Discussion (March 2020): Conduct of Clinical Trials During the COVID-19 Pandemic

Emerging Questions / Confusion about Clinical Trials*

- Scientific side:
  - Delayed startup
  - Sending study drugs
  - Short-term outcome measures: losing entire cohort
  - Ability to re-screen patients
  - What can be done at a distance with people currently enrolled?
  - Will funding be extended? Flexibility has been helpful
- Will labs be able to administer medications, and what does that mean for patients
- Whether therapeutic as well as non-therapeutic trials will be ended
  - Can natural history studies be done via telemedicine?
- Can non-therapeutic trials be done in a safe way?
- Concern about loss of clinical trials; how long can you extend primary endpoint assessments without losing entire trial
- What level of flexibility will FDA have as visit schedules change?
- Are open trials still enrolling patients?
- Consistency of care once pandemic has passed
- Language used… calling these "non-essential trials"; concern about trials being sidelined
- Information on testing kits and approval; untested/unapproved treatments
- Application for orphan drug status and pushback from consumer community

**From Patient Engagement Collaborative monthly teleconference, March 26, 2020*

FDA U.S. FOOD & DRUG ADMINISTRATION PATIENT ENGAGEMENT COLLABORATIVE CTTI

- ***CTTI hosted a webinar informed by PEC discussion***
- ***FDA shared insights with all medical product centers***

Recent Discussion: Potential Topics for the Next 3-5 Years

- Pathways for the individual consumer/patient voice to interact with FDA
- Including teenagers on the PEC and in communications/outreach efforts
- How to help FDA reach out more vs. having patients feel they have to knock on the door
- Carrying forward lessons learned from the COVID-19 pandemic

2021 Call for Applications Coming Soon

- 8 members completing terms in 2021
- Departing PEC members invited to serve on Selection Committee for new members

Scoring application materials

- Each application scored by two reviewers
- Must meet minimum score to proceed to phone interviews

Phone interviews

- Conducted with all remaining applicants
- Provides information to make final selection of PEC members

Final membership slate

- Includes consideration of balancing/diversity criteria
- Select 8 new members