

Joint Meeting:
The U.S. Food & Drug Administration/ Clinical Trials Transformation Initiative's Patient Engagement Collaborative/ (PEC)
and
European Medicines Agency's Patients' and Consumers' Working Party

July 20, 2023 | 9:00 – 11:00 am ET

Zoom Virtual Meeting

Disclaimer: *This purpose of this meeting was to facilitate a discussion of ideas, and as such, not all of the content below will be within the scope of the FDA, CTTI, or EMA. 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, CTTI, or EMA.*

Meeting Overview

Prior to the meeting, in order to tailor the meeting, participants were surveyed regarding their understanding and experience with decentralized clinical trials (DCTs). There were varying levels of understanding and experiences with the topic. The members of the Patient Engagement Collaborative (PEC) and Patients' and Consumers' Working Party (PCWP) met on July 20, 2023 to discuss DCTs. A representative from Clinical Trials Transformation Initiative (CTTI) introduced the work CTTI has done to support the adoption of DCT approaches. Representatives from the European Medicines Agency (EMA) and U.S. Food and Drug Administration (FDA) reviewed regulatory considerations for designing and implementing quality DCTs. Finally, a panel from the FDA, EMA, PEC, and PCWP discussed the benefits and challenges DCTs can pose for patients with all participants

Key Themes

- DCTs are clinical trials that include visits or assessments that are conducted away from the trial site. These trials use DCT elements – including tele-visits, mobile or local healthcare providers, and/or home delivery of investigational products – to varying degrees from nearly traditional to hybrid to fully remote.
- DCTs may offer a number of potential benefits or advantages including:
 - improving participant retention;
 - greater control, convenience, and comfort for participants;
 - reducing participant burden;
 - faster participant recruitment; and
 - increasing participant diversity (underrepresented populations).
- DCTs may offer some disadvantages or perceived risks including:
 - not as much personal interaction or face-to-face contact with the study staff;
 - concerns about data integrity and security;
 - the amount of infrastructure and supporting resources needed;

- the quality of the selected endpoints;
- the reliability of the technology used; and
- some uncertainty around how participants feel about DCT elements.
- Patient input into DCTs is critical to:
 - ensure trial materials such as consent forms are written in clear and patient-friendly language;
 - develop plans for participant diversity and inclusion;
 - provide input into remote data collection and use; and
 - understand connectivity demands, and technical literacy requirements.

Presentations

CTTI Decentralized Clinical Trials Overview

- DCTs are clinical trials that include study visits or assessments that are completed away from the trial site, including telehealth (online) visits, mobile or local healthcare providers, and/or home delivery of investigational products.
- Individual trials should evaluate what DCT elements are appropriate for the endpoints they are measuring, while being mindful of whether these elements would improve patient access or increase participant burden. Quality DCTs should fit into the patient's life, giving them flexibility to control how they engage in the trial.
- The resources in CTTI's [Digital Health Trials Hub](#) aim to support more patient-centered and easily accessible trials through a set of recommendations and tools.

DCTs in the European Union and the United States

EMA and DCTs:

- The [recommendation paper](#) on decentralised elements in clinical trials (December 2022) drafted as part of the [ACT EU](#) initiative takes a risk-based approach to DCTs that is patient-centered, emphasizes the importance of researcher and sponsor oversight, and requires that trial data is reliable, high quality, and fit-for-purpose.
- To ensure proper oversight, it's important to set clear roles and responsibilities by documenting where and when each task is to be performed, who is responsible for each task, and how oversight will be maintained; establishing a clear communication plan between the different parties involved (i.e., sponsor, investigator, participants, and service providers); and informing trial participants on the information flow and who to contact in the event of an acute safety concern, device malfunction, or other question.
- There needs to be a balance between increasing flexibility for trial participants and protecting key endpoints.

FDA and DCTs:

- DCT activities are not new. Over the years, and particularly during the COVID-19 pandemic, many trial related activities were done by patients at home.
- DCTs are an important opportunity to improve the efficiency of trials, convenience for patients, and access for diverse participants and participants with rare diseases.

- DCTs can use digital health technologies to gain access to continuous or frequent data, including data on sporadic events (e.g. seizures, arrhythmias, falls), patient-reported outcomes (e.g. ecological momentary assessments), information about a patient's real-world environment, and objective records of a patient's functionality.
- The FDA published draft guidance documents on [Digital Health Technologies for Remote Data Acquisition in Clinical Investigations](#) (December 2021) and [Decentralized Clinical Trials for Drugs, Biological Products, and Devices](#) (May 2023).

Panel Discussion: Decentralized Clinical Trials

Representatives from the FDA, EMA, PEC, and PCWP discussed the benefits and challenges DCTs can pose for patients. Key points discussed include:

- **Engaging patients in the design of DCTs** from the very earliest planning stages is critical for ensuring trials are patient-centered and accessible.
- **Connecting with each participant through at least one in-person visit** is important for building trust between participants and study staff.
- **Balancing the benefits of DCTs with the potential risks/challenges** is crucial for ensuring patient safety and data integrity.

Conclusion and Next Steps

The FDA, CTTI, and EMA will collect feedback on this meeting and use it to plan future PEC-PCWP joint meetings. They will also look for additional opportunities for PEC and PCWP members to work together on topics on common interest and to improve patient engagement.

The PEC is a public-private partnership between the FDA and the Clinical Trials Transformation Initiative (CTTI) that is not intended to advise or direct the activities of either organization. The PEC is primarily a forum to facilitate the exchange of information between patient community representatives and the FDA on areas of common interest, including regulatory discussions and strategies to increase patient engagement. Public summaries of all PEC meetings, including the last PEC-PCWP Joint Meeting in 2022, are available on [the PEC website](#).

The Patients' and Consumers' Working Party ([PCWP](#)) provides a platform for exchange of information and discussion of issues of common interest between EMA and patients and consumers. The PCWP, established in 2006, has enabled the Agency to build upon its existing interactions with patients and consumers. It provides recommendations to EMA and its human scientific committees on all matters of interest in relation to medicines.