Joint Meeting of the U.S. Food and Drug Administration (FDA) Clinical Trials Transformation Initiative's (CTTI)/ Patient Engagement Collaborative (PEC) and the European Medicines Agency's (EMA) Patients' and Consumers' Working Party (PCWP)

June 18, 2024 | 9:00 – 11:30 am ET

Zoom Virtual Meeting

Disclaimer: *The 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

The purpose of this virtual meeting was to facilitate discussion between members of the PEC and PCWP patient communities related to patient-reported outcomes (PROs) and Health-Related Quality of Life (HRQoL) measures. Representatives from the EMA and FDA presented discussions held at public workshops related to the implementation of PRO and HRQoL data in regulatory decision-making. This was followed by a panel that included the speakers and patient representatives from the PEC, and PCWP who discussed key challenges and considerations around the implementation of PROs in clinical trials.

EMA/FDA Presentations:

EMA and European Organisation for Research and Treatment of Cancer (EORTC) Workshop: How can patient-reported outcomes (PRO) and health-related quality of life (HRQoL) data inform regulatory decisions?:

- A representative from the EMA presented topics discussed during a February 2024 joint EMA/ EORTC public workshop on using PRO and HRQoL data to inform regulatory decision-making in Oncology.
- The workshop was aimed towards creating a convergence between regulators and health technology assessment bodies (HTAs) on the use of PROs and quality of life data in decision-making for the patient benefit.

FDA- National Eye Institute (NEI) Workshop on Patient-reported Outcomes and Vision-Related Quality of Life Questionnaires:

- A representative from FDA's Center for Devices and Radiological Health (CDRH) presented topics discussed during a September 2023 FDA-NEI (a part of National Institutes of Health) public workshop on the potential for patients' perspectives to inform the design and conduct of medical device clinical studies as well as the tools used to collect outcomes.

- The workshop highlighted the importance of patients' perspectives, patient-reported outcomes ("PROs"), and questionnaires to measure visual function and vision-related quality of life ("VFQs") in vision research and clinical care.

Discussion Question*:*

- *What is the difference between patient experience data, HRQoL measures, and PROs?*
    - In the **United States**, the Federal Food, Drug, and Cosmetic Act, as amended by the 21st Century Cures Act and the FDA Reauthorization Act of 2017, defines patient experience data as:
        - (1) "data that are collected by any persons (including patients, family members, and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers); and
        - (2) are intended to provide information about patients' experiences with a disease or condition, including
            - (A) the impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy or clinical investigation, on patients' lives; and
            - (B) patient preferences with respect to treatment of such disease or condition."
    - Patient experience data involves asking patients about their general experiences living with a disease, the treatment they receive, and what treatments they would want. These data may include HRQoL measures that reflect how a person's health impacts their activities and abilities.
    - PROs are a part of the clinical trial, where patients report on specific outcomes, like side effects, and rate them on a scale (e.g., a patient may rate their pain as 8 out of 10*).* It is a measurement based on a report that comes directly from the patient about the status of a patient's health condition without changes or interpretation of the patient's response by a clinician or anyone else.
    - A common understanding of *Patient Experience Data* in the **European Union** was determined during a dedicated *workshop*.
    - The voice of patients and carers is critical during the whole life cycle of a medicine, from early development to reporting of adverse drug reactions (ADRs) and risk minimization. Patients' input should become standard at all stages of medicines development and regulatory decision-making. There was broad agreement on the understanding of key concepts that can be found in the link above.
    - In addition, the EMA guideline 'Appendix 2 to the guideline on the evaluation of anticancer medicinal products in man' provides definition of PROs and HRQoL data as follows:

- A PRO includes any outcome evaluated directly by the patient himself or herself and is based on patient's perception of a disease and its treatment(s). PRO is an umbrella term covering both single dimension and multi-dimension measures of symptoms, HRQL, health status, adherence to treatment and satisfaction with treatment. PRO measures (PROMs) are the tools and/or instruments that have been developed to ensure both a valid and reliable measurement of these PROs. Like any other clinical outcome assessments such as a rating of a symptom, sign or performance by an observer or trained medical care provider, it is recognised that such data have inherent variability related to the assessor.
- Health-related quality of life is a specific type of PRO and is a broad concept which can be defined as the patient's subjective perception of the impact of his/her disease and its treatment(s) on his/her daily life, physical, psychological and social functioning and well-being.

Panel Discussion: PEC and PCWP Member Reactions

Representatives from the FDA, EMA, PEC, and PCWP discussed key challenges and considerations around the implementation of PROs in clinical trials. Key points discussed include:

- **Implementing PROs** to assess and demonstrate certain subjective treatment benefits or risks
- **Harmonizing and validating PRO** tools and regulatory approaches, considering impact of PROs on patient burden
- **Transferring and applying PRO** tools and regulatory approaches to other disease areas, considering unique patient needs and limitations

Conclusion and Next Steps

The FDA, CTTI, and EMA expressed the need to continue to identify opportunities to further expand patient engagement. Feedback from this meeting may be incorporated in future PEC-PCWP joint meetings.

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 2023, 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.