

FDA and CTTI Patient Engagement Collaborative (PEC) Meeting

September 21, 2022 | 11 am – 3 pm ET

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 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 understanding and discussion of orphan drug development for rare diseases. This meeting also included a discussion about how the FDA can improve staff interactions with patients and caregivers.

Discussion Themes

- *Developing drugs and medical products to prevent, diagnose, and treat rare diseases*
- *Improving interactions between FDA staff and patients and caregivers*

Presentation and Group Discussion: FDA Orphan Drug Designation Program

- There are over [7,000 known rare diseases](#) that collectively impact around 25-30 million Americans—about half which are children—but most rare diseases don't have an approved treatment.
- Enacted in 1983, the Orphan Drug Act (ODA) encourages the creation of drugs and other medical products for rare diseases through incentives provided by the Office of Orphan Products Development (OOPD).
 - Through their Orphan Drug Designation Program, the OOPD provides financial incentives for sponsors who want to develop [orphan drugs](#) (drugs or products for the prevention, treatment, or diagnosis of rare diseases).
- Interest in orphan drugs has increased significantly since 1983. Only 10 treatments for rare diseases were FDA approved in the decade before the ODA. Each year since 2016, the OOPD has received over 500 requests for orphan-drug designation. In 2021, FDA's Center for Drug Evaluation and Research (CDER) approved 26 new molecular entities for rare diseases indications..

Discussion

- Meeting attendees suggested simplifying and sharing the presentation with the general patient community and patient advocacy groups.
- Meeting attendees suggested it would be helpful to provide 3 to 4 action items for patients and advocacy groups to get involved in orphan drugs development.
- Meeting attendees suggested it would be helpful to have a plain language webpage—with information similar to the [Designating an Orphan Product webpage](#)—for patients to learn about orphan drug studies and outcomes, studies of rare diseases, and ways to

participate.

Group Discussion: FDA Interactions with Patients and Caregivers

- The FDA recognizes that patients and caregivers often share deeply personal information and stories with the agency, and these can be difficult conversations.
- The FDA is looking for ways to improve staff interactions with patients and caregivers.

Discussion

- Meeting attendees suggested several ways to improve interactions:
 - Actively listen and show empathy for patients' experiences
 - Avoid stigmatizing and recognize the valuable personal knowledge patients offer
 - Welcome patient representatives to meetings and check in with them
 - Make sure study sponsors answer patients' questions in meetings
 - Plan meetings later in the day and plan for patient representatives to speak earlier in the agenda to reduce the burden of participating.
- Meeting attendees suggested training might also be helpful in the following areas:
 - Security – Some people have invisible disabilities or medical devices and may need extra time to get through security lines.
 - Meeting Management – Patients and caregivers may be intimidated by technical and scientific terms and may be hesitant to speak up. It may be helpful if meeting facilitators created opportunities for patients to speak and made sure patients felt that their input is valued.
 - Emails and Phone Calls – Make sure patients and caregivers are connected to the resource(s) that actually address their question(s). Refer them to someone who may be able to share the information they need, or let them know why information isn't available.

Conclusion and Next Steps

The FDA and CTTI will review the discussion points and ideas generated during this meeting. The FDA will share comments from this meeting with agency departments to facilitate improvement of interactions with patients and caregivers.

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 are available on [the PEC website](#).