CONSIDERATIONS FOR HOSTING AN EXTERNALLY-LED, PATIENT-FOCUSED DRUG DEVELOPMENT (EL-PFDD) MEETING

The Food and Drug Administration (FDA) states that an Externally Led Patient-Focused Drug Development (EL-PFDD) can “help ensure that patients’ experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.” (FDA, 2020)

The FDA conducts EL-PFDD meetings to obtain the patient perspective on specific diseases and treatment options.

Patient organizations are encouraged to identify and organize EL-PFDD meetings. This will highlight disease areas where there is a need for patient input on topics related to medical product development.

Before you begin, do your homework:

1. Collect comprehensive and representative input.
   Outlined in Guidance 1: bit.ly/FDA-Guidance-1

2. Determine the best methods to identify what is important to patients.

3. Learn how to select, develop or modify fit-for-purpose clinical outcomes assessments.

4. Incorporate clinical outcome assessments into endpoints for regulatory decision making.
   Outlined in Guidance 4: bit.ly/FDA-Guidance-4

CASE STUDY: EL-PFDD FOR KRABBE DISEASE

The release of this summary report follows the virtual EL-PFDD held in October 2020. The Voice of the Patient Report provided an opportunity for patients and caregivers to share details about the impact of this disease on their daily lives and share their experiences with currently available treatments.

Recording of PFDD meeting held on October 29, 2020: bit.ly/PFDD-recording


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1 fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development