

## 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.”<sup>1</sup> (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](https://bit.ly/FDA-Guidance-1)
- 2. Determine the best methods to identify what is important to patients.**  
Outlined in Guidance 2: [bit.ly/FDA-Guidance-2](https://bit.ly/FDA-Guidance-2)
- 3. Learn how to select, develop or modify fit-for-purpose clinical outcomes assessments.**  
Outlined in Guidance 3: [bit.ly/FDA-Guidance-3](https://bit.ly/FDA-Guidance-3)
- 4. Incorporate clinical outcome assessments into endpoints for regulatory decision making.**  
Outlined in Guidance 4: [bit.ly/FDA-Guidance-4](https://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](https://bit.ly/PFDD-recording)

***Voice of the Patient Report* for Krabbe Disease:** [bit.ly/Krabbe-Disease-Report](https://bit.ly/Krabbe-Disease-Report)

<sup>1</sup> [fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development](https://fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development)