U.S. FOOD & DRUG ADMINISTRATION

Incorporating the Patient Voice into Clinical Trial Design and Conduct

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Better integrating the patient's perspective at critical points in drug development and decision making

Identify and **measure** outcomes and burdens that matter most to patients

- **Design** better clinical studies
- **Recruit** potential patients
- **Retain** study participants

Integrate patient-reported outcomes and patient preference information **into BR assessments**

Better Communicate information about drugs to patients and providers to **facilitate** informed decision-making

Patient-focused drug development (PFDD) is a systematic approach to help ensure that **patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated** into drug development and evaluation.

#PFDD

PFDD Meetings Provide Key Stakeholders an Opportunity to Hear the Patient's Voice

Meetings **Strengthen Understanding** of Disease and Treatment Burden

Patient input from meetings can support FDA staff:

- In conducting benefit-risk assessments for products under review, by informing the therapeutic context
- Advising drug sponsors on their development programs

It might also support drug development more broadly:

- Identify areas of unmet need in the patient population
- Identify or develop tools that assess benefit of potential therapies
- Raise awareness and channel engagement within the patient community

Meeting summary reports from both FDA and Externally-Led Meetings that capture patient experience data are shared on FDA's website

CY2013

- Chronic Fatigue Syndrome/ Myalgic Encephalomyelitis
- HIV
- Lung Cancer
- Narcolepsy

CY2014

- Sickle Cell Disease
- Fibromyalgia
- Pulmonary Arterial Hypertension
- Inborn Errors of Metabolism
- Hemophilia A, B, and Other Heritable Bleeding Disorders*
- Idiopathic Pulmonary Fibrosis
- Female Sexual Dysfunction

CY2015

- Breast Cancer
- Chagas Disease
- Functional Gastrointestinal Disorders
- Parkinson's Disease and Huntington's Disease
- Alpha-1 Antitrypsin Deficiency*
- Non-tuberculous Mycobacterial Lung Infections

CY2016

- Psoriasis
- Neuropathic Pain Associated with Peripheral Neuropathy
- Patients Who Have Received an Organ Transplant

CY2017

- Sarcopenia
- Autism
- Alopecia Areata
- Hereditary Angioedema*

CY2018

- Opioid Use Disorder
- Chronic Pain

CY2019

- None

CY2020

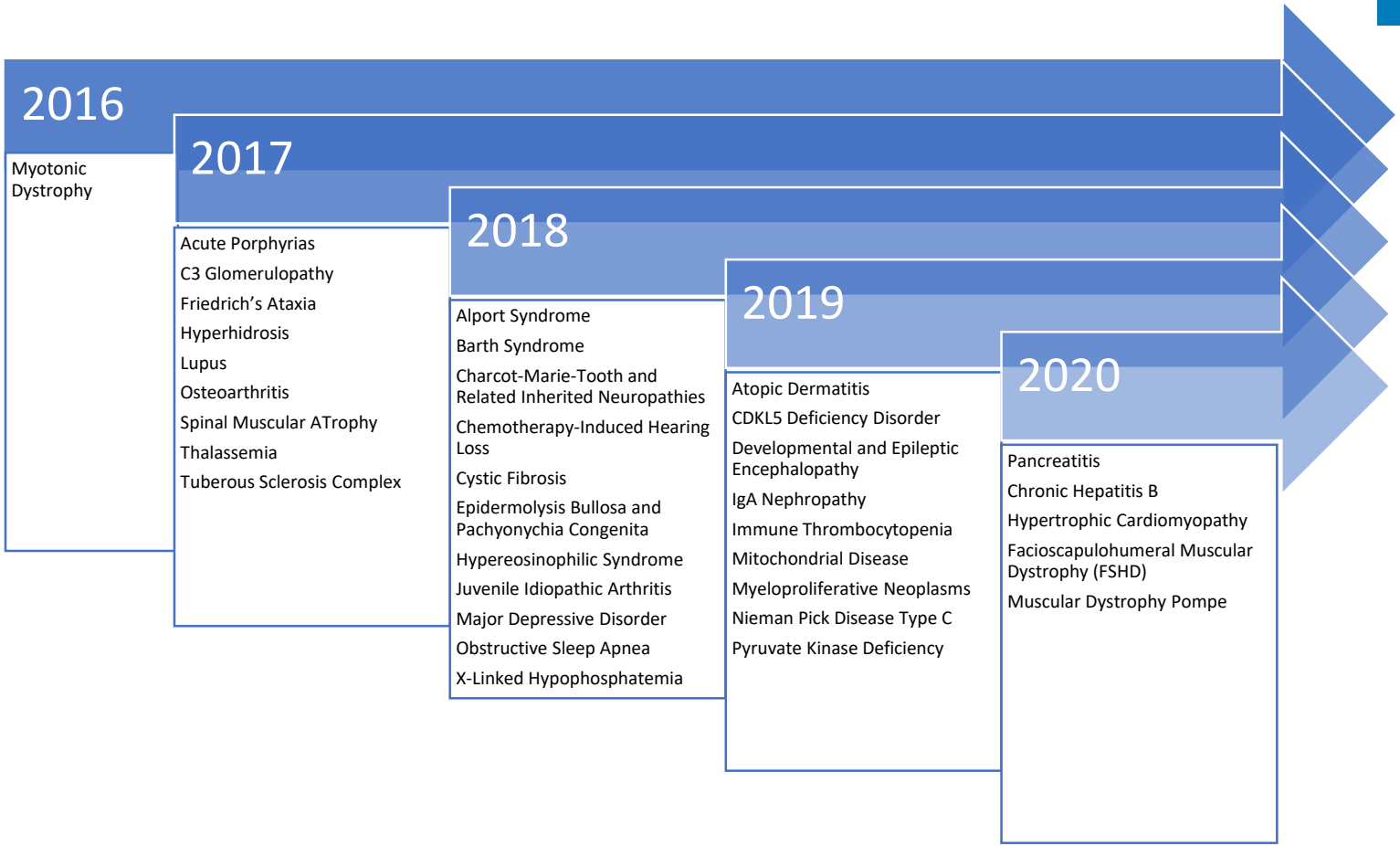
- Systemic Sclerosis
- Stimulant Use Disorder

CY2021

- Vitiligo

** Meetings conducted by FDA's Center for Biologics Evaluation and Research*

Externally-Led Patient Focused Drug Development Meetings



Patients are **experts** in their condition.

It is important to get patient input early in the drug development process.

- Patients' **"chief complaints" may not be factored explicitly into medical product development** plans, including measures of medical product benefit planned in clinical studies.
- Patients want to be as active as possible in the work to develop and evaluate new treatments
- The most obvious symptoms of a disease or illness are not always the most bothersome

FDA's Public Meeting on Patient-Focused Drug Development for Chronic Pain

- July 9, 2018, 9 am – 5pm
- The Voice of the Patient summary report capturing participants' input in their own words was published in March 2019.
- The following is a highlight of select input gathered at the meeting (in-person and webcast) and through the public docket.

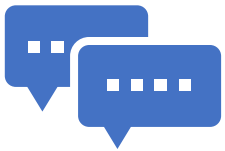
> 120 individuals who experience chronic pain, caregivers or advocates attended the meeting in person

> 300 individuals who experience chronic pain, caregivers or advocates provided input through the interactive webcast

> 2,400 submitted comments through the public docket, majority from individuals who experience chronic pain, caregivers or advocates

Participants varied in gender, race, age, underlying condition, and their current approaches to management.

Participants did represent a certain population with chronic pain who have had successful pain management in the past through opioids, but have experienced a loss of access or reduced access to opioids because recent opioid policies.



Discussion topics focused on experiences and perspectives:

- Impact of chronic pain on daily life
- Current treatment approaches
- Challenges or barriers to accessing treatments

Public Meeting on Patient-Focused Drug Development for Chronic Pain: Key Themes

The health effects of chronic pain are pervasive and wide ranging.

- Participants frequently described living with daily unrelenting chronic widespread pain, in addition to persistent fatigue, sleep issues, and other debilitating health effects.
- Many shared fears that their chronic pain would worsen over time.

Public Meeting on Patient-Focused Drug Development for Chronic Pain: Key Themes

Chronic pain affects all aspects of individuals' lives.

- Many participants described severe limitations and adaptations needed to perform at work or at school, and to care for themselves and their family.
- Many also described the devastating loss of meaningful relationships with family and friends due to their chronic pain.
- Participants shared the emotional toll of experiencing stigma, social isolation, and financial challenges of treatment, and fears of living with an often-misunderstood condition.

Public Meeting on Patient-Focused Drug Development for Chronic Pain: Key Themes

Management of chronic pain requires a multidisciplinary approach tailored to the needs of the individual.

- Some stressed the need for a holistic approach to treating chronic pain, including lifestyle management, diet and exercise, and psychosocial techniques, in addition to medical treatments.
- They expressed varying perspectives on the desired outcomes of treatment. For most, the shared focus, however, was to improve their daily quality of life.

Public Meeting on Patient-Focused Drug Development for Chronic Pain: Key Themes

Several challenges and barriers to accessing treatments to manage their chronic pain.

- They highlighted growing stigma on the use of opioid analgesics and the impact of stigma on their interactions with healthcare providers and others when seeking treatment, particularly being perceived as “an addict.”
- A common experience shared during the meeting is that of an individual living with chronic pain who has had successful pain management in the past using a stable dose of opioid analgesics but who now has severely worsened pain resulting from reduced access to opioids because of a change in their doctor’s practice or willingness to prescribe opioids.

Public Meeting on Patient-Focused Drug Development for Chronic Pain: Key Themes

Need increased awareness and understanding of chronic pain across the medical and policymaking community.

- Participants offered perspectives on how to better help individuals with chronic pain manage their condition, expressing hope for progress in the development of effective non-opioid treatment options.

FDA's Public Meeting on Patient-Focused Drug Development for Opioid Use Disorder

- April 17, 2018, 10 am – 4pm
- The Voice of the Patient summary report capturing participants' input in their own words was published in November 2018.
- The following is a highlight of select input gathered at the meeting (in-person and webcast) and through the public docket.

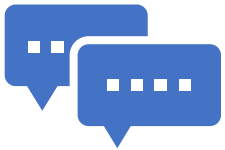
> 100 individuals with opioid use disorder or caregivers attended the meeting in person

> 85 individuals with opioid use disorder or caregivers provided input through the interactive webcast

> 70 submitted comments through the public docket, majority from individuals with opioid use disorder, caregivers, or advocates.

Participants varied in gender, race, age, history of opioid use, experiences with opioid use disorder, and time to recovery.

Participants varied in their overarching perspectives on substance use and treatment.

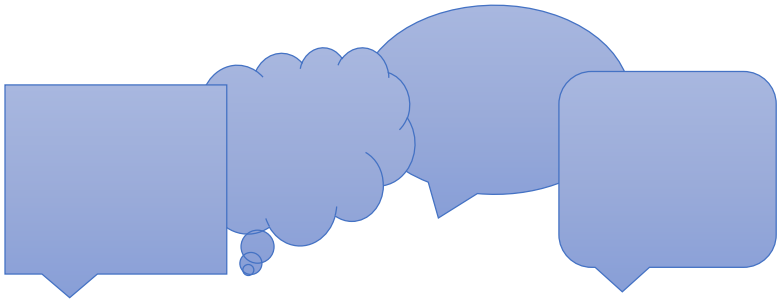


Discussion topics focused on experiences and perspectives:

- Health effects and daily impacts of opioid use disorder
- Individuals' and families' perspectives on current approaches to treating opioid use disorder

Feedback from PFDD Meetings

- Participants shared that more attention should be paid to the social, psychological and financial impact of these diseases
- Many participants cited the need to have a clear understanding of the risk and benefits of participating in a clinical trial.
- More generally, participants stressed the importance of: reducing barriers to clinical trial participation; streamlining the informed consent process



- Participants stressed that quality of life endpoints should be included in the design of clinical trials.
- Other considerations raised included the burden of travel to the clinical trial site, the invasiveness of the procedures, side effects, toxicity of the drug, and how participation would impact a patient's lifestyle.
- They stressed the need to increase patients' and healthcare providers' awareness of clinical trial opportunities, and highlighted the potential of community networks and social media. They also stressed the need to establish more trust and respect between researchers and patients.

Patients can provide:

- Natural History Data
- Input on relevance of research to patients
- Help defining eligibility criteria
- Input on meaningful endpoints
- Education to the patient community
- Input on protocol input and feasibility

- Increased awareness about trials
- Participant feedback on trial experience
- Input on informed consent content and processes
- Members of Data and Safety Monitoring boards
- Collaboration on post-marketing studies and surveillance initiatives

Resources are available on PFDD webpages

CDER Patient-Focused Drug Development Homepage

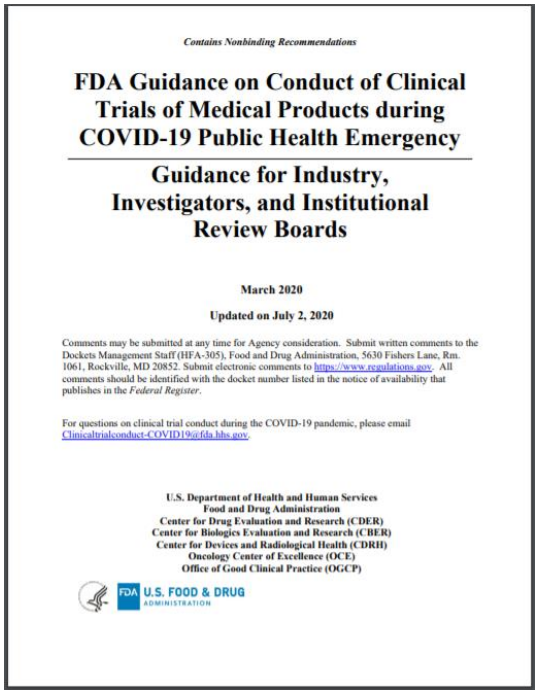
- Guidances
- COA Grant Program
- FDA-led PFDD Meetings
- Externally-led PFDD Meetings
- External Resources

- Browse upcoming meetings
- View past meeting materials
  - Slides, meeting recordings, transcript, agenda, summary report

Questions?
Email PatientFocused@fda.hhs.gov.

To get updates about CDER's Patient-Focused Drug Development programs, subscribe to our **free email subscription service** by clicking on the button near the top of the **homepage**.

FDA Guidance



Contains Nonbinding Recommendations

FDA Guidance on Conduct of Clinical Trials of Medical Products during COVID-19 Public Health Emergency

Guidance for Industry, Investigators, and Institutional Review Boards

March 2020

Updated on July 2, 2020

Comments may be submitted at any time for Agency consideration. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. Submit electronic comments to https://www.regulations.gov. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions on clinical trial conduct during the COVID-19 pandemic, please email ClinicalTrialConduct-COVID19@fda.hhs.gov.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
Oncology Center of Excellence (OCE)
Office of Good Clinical Practice (OGCP)

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Appendix: Questions and Answers

https://www.fda.gov/media/136238/download

Select Resources

- Clinical Trials Transformation Initiative:
  - https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials
- National Health Council
  - https://nationalhealthcouncil.org/issue/patient-engagement/
- SWOG/PCORI
  - https://www.pcori.org/sites/default/files/TeamScience-SWOG-Field-Guide.pdf
- TransCelerate
  - https://www.transceleratebiopharmainc.com/ppet/planning-for-patient-engagement/