

# The VOICE of FTD

WINTER 2020

## EL-PFDD Meeting: March 5, 2021

*Take the survey, watch the live stream, have your FTD voice heard!*

People living with a rare disease are uniquely positioned to provide information to increase understanding for the development and evaluations of treatments. On March 5, 2021, persons affected by FTD will have such an opportunity during an Externally-Led Patient-Focused Drug Discovery (EL-PFDD) meeting with representatives of the [U.S. Food and Drug Administration](#) (FDA).

Led by [The Association for Frontotemporal Degeneration](#) (AFTD), the meeting offers an opportunity for persons diagnosed with FTD, family members, and caregivers to educate the FDA about the challenges of living with frontotemporal degeneration (FTD).

“The forthcoming EL-PFDD meeting focused on FTD is a vital opportunity for people and families affected by FTD to share the lived perspective of this disease with the FDA,” said AFTD’s Chief Executive Officer Susan L-J Dickinson, MSGC. “There has never before been a meeting of this kind focused on FTD.”

In preparation for the March meeting, AFTD will hold an informational session in February. Watch for more details soon.

### Purpose of March Meeting

PFDD meetings, both FDA-led (FDA-PFDD) and externally-led (EL-PFDD), offer a chance to educate and impact the federal agency that regulates all prescription drugs that enter the market. This meeting will specifically provide the FDA with a range of viewpoints and input about living with FTD based on firsthand experience.

The free, open meeting will include pre-recorded testimonials as well as opportunities for the entire FTD community to participate via live polls, chat, and phone-in. Additionally, a live stream of the meeting will be publicly accessible.

Those who participate in the meeting will be able to share details of dealing with disease symptoms and the impact they have on daily life that are most important to them. They also can provide their perspectives on treatments.


“We hope that all who have had their lives impacted by this devastating condition will prioritize taking part in this meeting,” Dickinson said. “The perspectives


*PFDD Meeting continues on Page 2*

### FTD Disorders Registry LLC

637 Carolina Street  
San Francisco, CA 94107  
888-840-9980

 [www.facebook.com/EndFTDregistry](http://www.facebook.com/EndFTDregistry)

 [www.linkedin.com/company/ftd-disorders-registry](http://www.linkedin.com/company/ftd-disorders-registry)

 @EndFTDregistry

 [bit.ly/YouTubeFTDregistry](http://bit.ly/YouTubeFTDregistry)

*PFDD Meeting continued from Page 1*

brought together on March 5 will help the FDA to better understand the priorities of our families for the development of treatments targeting this disease.”

While the meeting focuses on FTD-diagnosed persons, their family members and care partners, research and healthcare professionals are invited to listen to hear directly from the FTD community.

The day-long PFDD meeting, which will be entirely virtual, will start with AFTD Medical Advisory Council Chair Elect Bradford C. Dickerson, M.D., presenting a clinical overview of FTD.

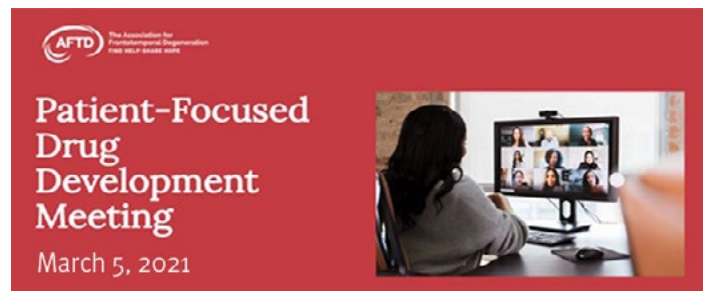
The next session will focus on living with FTD, featuring live and pre-recorded comments from FTD-diagnosed persons, care partners, and others.

There will also be two panel discussions on current and future FTD treatments. One will focus on sporadic FTD and the other will focus on genetic FTD.

The meeting’s agenda was guided by the FTD Insights Survey being conducted in partnership between AFTD and the FTD Disorders Registry. Responses from October were considered when setting the meeting focus, and results from all responses collected through March 31, 2021, will be added to the Voices of the Patient report.

“We have had a tremendous response from the FTD community,” said Registry Director Dianna Wheaton, M.S., Ph.D., CHES. “We are grateful to the more than 1,300 people who have already completed the survey and hope many more will do so before the end of March.”

The survey collects information about the experiences, opinions, and preferences of patients, family members, and caregivers affected by FTD.



The following diagnoses are included:

- **bvFTD** — behavioral variant FTD, frontotemporal dementia, or FTD dementia
- **CBD or CBS** — corticobasal degeneration or corticobasal syndrome
- **FTD-ALS** — FTD with ALS (amyotrophic lateral sclerosis) or FTD with MND (motor neuron disease)
- **PPA** — primary progressive aphasia
  - no subtype given
  - nonfluent agrammatic PPA (nfvPPA) or progressive nonfluent aphasia (PNFA)
  - semantic variant PPA (svPPA) or semantic dementia
  - logopenic variant PPA (lvPPA) or logopenia
- **PSP** — progressive supranuclear palsy or Richardson’s syndrome
- **Pick’s disease**

Survey participants must be 18 years of age or older and a resident of the United States, Canada, or the United Kingdom (UK by invitation only). Participation is voluntary.


The data collected is anonymous; names are not asked, and responses cannot be linked to specific individuals. It takes 20 to 30 minutes and is done through a confidential, secure online platform.


*PFDD Meeting continues on Page 3*

## FTD Disorders Registry LLC

637 Carolina Street  
San Francisco, CA 94107  
888-840-9980

 [www.facebook.com/EndFTDRegistry](http://www.facebook.com/EndFTDRegistry)

 [www.linkedin.com/company/ftd-disorders-registry](http://www.linkedin.com/company/ftd-disorders-registry)

 @EndFTDRegistry

 [bit.ly/YouTubeFTDRegistry](https://bit.ly/YouTubeFTDRegistry)

*PFDD Meeting continued from Page 2*

Following the meeting, the Voice of the Patient report will be published to capture highlights and perspectives from the PFDD meeting and will include highlights from the FTD Insights Survey. This report will become a resource for the FDA, clinicians, researchers, and industry professionals to use when they make decisions about new FTD treatments.

In addition, results of the FTD Insights Survey will be published in a scientific journal.

### [TAKE THE FTD INSIGHTS SURVEY](#)

#### **What is PFDD?**

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. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.

FDA meets with patients to get their perspective of the impact of their condition on their daily life, on the treatments, and on how to choose a treatment. This allows patients' perspectives to play a role in determining how drugs should be developed.

According to the FDA, this includes:

- facilitating and advancing use of systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input to more consistently inform drug development and regulatory decision-making
- encouraging identification and use of approaches and best practices to facilitate patient enrollment and minimizing the burden of

patient participation in clinical trials

- enhancing understanding and appropriate use of methods to capture information on patient preferences and the potential acceptability of tradeoffs between treatment benefit and risk outcomes
- identifying the information that is most important to patients related to treatment benefits, risks, and burden, and how to best communicate the information to support their decision making.

The FDA began using PFDD meetings in 2013 to better understand diseases. These meetings are considered part of the evolution toward patient-centric medical product development and health care. It is driven by patients and caregivers who are willing to speak up.

#### **Why Does FDA Hold PFDD Meetings?**

As the primary benefactors of drugs that treat rare diseases, it is essential that patients, families, and caregivers are involved in the drug development process. These people bring a human perspective to the drug development process and can help guide researchers toward beneficial treatment outcomes. The lessons learned include, but are not limited to, specific experiences that matter most to patients, patient perspectives on meaningful treatment benefits, and how patients want to be engaged in the process.

According to FDA "The patient perspective is critical in helping FDA understand the context in which regulatory decisions are made for new drugs. PFDD meetings give FDA and other key stakeholders, including medical product developers, health care providers, federal partners, an important opportunity to hear directly from patients, their families,

*PFDD Meeting continues on Page 4*

#### **FTD Disorders Registry LLC**

637 Carolina Street  
San Francisco, CA 94107  
888-840-9980



[www.facebook.com/EndFTDRegistry](http://www.facebook.com/EndFTDRegistry)



[www.linkedin.com/company/ftd-disorders-registry](http://www.linkedin.com/company/ftd-disorders-registry)



[@EndFTDRegistry](https://twitter.com/EndFTDRegistry)



[bit.ly/YouTubeFTDRegistry](https://bit.ly/YouTubeFTDRegistry)

*PFDD Meeting continued from Page 3*

caregivers, and patient advocates about the symptoms that matter most to them, the impact the disease has on patients’ daily lives, and patients’ experiences with currently available treatments. This input can inform FDA’s decisions and oversight both during drug development and during our review of a marketing application.”

[LEARN MORE ABOUT PFDD \(Video\)](#)

**PFDD as a Division of CDER**

As part of the U.S. Food and Drug Administration (FDA), the Center for Drug Evaluation and Research (CDER) regulates over-the-counter and prescription drugs, including biological therapeutics and generic drugs. It also ensures that prescription and over-the-counter drugs, both brand name and generic, work correctly and that the health benefits outweigh known risks.

CDER carefully evaluates the benefits and risks of drugs and ensures that consumers have access, as quickly as possible, to promising new treatments. The Center oversees the research, development, manufacture and marketing of drugs. This includes collecting information from persons affected through PFDD meetings.

Drug companies seeking to sell a drug in the United States must first test it. The company then sends CDER the evidence from these tests to prove the drug is safe and effective for its intended use. A team of CDER physicians, statisticians, chemists, pharmacologists, and other scientists reviews the company’s data and proposed labeling. If this independent and unbiased review establishes that a drug’s health benefits outweigh its known risks, the drug is approved for sale. The center doesn’t actually test drugs itself, although it does conduct limited research in the areas of drug quality, safety, and effectiveness standards.

Before a drug can be tested in people, the drug company or sponsor performs laboratory and animal tests to discover how the drug works and whether it’s likely to be safe and work well in humans. Next, a series of tests in people is begun to determine whether the drug is safe when used to treat a disease and whether it provides a real health benefit.

**Benefits of a PFDD Meeting**

When effectively collected, tracked, and analyzed, patient-reported outcomes become a useful tool for making informed decisions and improving the quality of care, treatments, therapies, and interventions. This is even more important for rare diseases like FTD because there is a smaller number of people diagnosed.

A rare disease in the United States affects fewer than 200,000 people. According to AFTD, about 50,000 to 60,000 people in the United States are diagnosed with one of the forms of FTD.

The upcoming PFDD meeting in March benefits the FTD community immediately by sharing stories to enlighten regulators. Persons dealing with a rare disease can learn about their disease from others who are also affected. However, these meetings offer long-lasting benefits through follow-up publishing of the Voice of the Patient report and survey data.

By making your voice heard, you can provide insight to the FDA about FTD symptoms and help them better understand the priorities of families for research to develop treatments targeting this disease.


“Together, we can have a direct impact on the future course of treatments, helping to bring hope

*PFDD Meeting continues on Page 5*

**FTD Disorders Registry LLC**

637 Carolina Street  
San Francisco, CA 94107  
888-840-9980

 [www.facebook.com/EndFTDregistry](http://www.facebook.com/EndFTDregistry)

 [www.linkedin.com/company/ftd-disorders-registry](http://www.linkedin.com/company/ftd-disorders-registry)

 @EndFTDregistry

 [bit.ly/YouTubeFTDregistry](http://bit.ly/YouTubeFTDregistry)

PFDD Meeting continued from Page 4

to individuals and families facing these devastating diseases,” said the Registry’s Dr.Wheaton.

The March 5, 2021, EL-PFDD meeting about FTD is sponsored by the following organizations:

- Ionis Pharmaceuticals
- Lilly
- Alector
- Acadia Pharmaceuticals
- Alzheimer’s Drug Discovery Foundation
- Bluefield Project to Cure FTD
- PassageBio
- Wave Life Sciences
- Alzheimer’s Association
- Prevail Therapeutics
- Biogen
- AbbVie
- Denali Therapeutics

[TAKE THE FTD INSIGHTS SURVEY](#)

[LEARN MORE ABOUT PFDD \(Video\)](#)




**Join the Registry.  
Tell Your Story.  
Advance the Science.**

***“Together, we can make a difference!”***

### FTD Disorders Registry LLC

637 Carolina Street  
San Francisco, CA 94107  
888-840-9980

 [www.facebook.com/EndFTDRegistry](http://www.facebook.com/EndFTDRegistry)

 [www.linkedin.com/company/ftd-disorders-registry](http://www.linkedin.com/company/ftd-disorders-registry)

 @EndFTDRegistry

 [bit.ly/YouTubeFTDRegistry](http://bit.ly/YouTubeFTDRegistry)