

Be Heard by the FDA! Overview of a Patient Focused Drug Development (PFDD) meeting



KrabbbeConnect
CONNECTING PATIENTS & RESEARCH

Guest Speaker Debbie Drell

Director of Membership at NORD



NORD[®]
National Organization
for Rare Disorders



Please Note!

The views and opinions expressed in this presentation are those of the individual presenter and should not be attributed to or considered binding on the U.S. Food and Drug Administration (FDA).



History of PFDD meetings

This could be YOU at the table telling your story to the FDA



Idea behind these unique meetings

- The role of FDA and what they do
- In 2012, the FDA established the PFDD initiative to obtain the patient perspective on diseases
- Are used to gather information from patients on two topics:
 - a. What are the most significant symptoms of the disease and the impact on your daily life
 - b. What are the current approaches to treatment

Purpose of a PFDD meeting



Stakeholders an opportunity to hear from the patients/caregivers



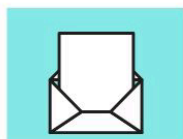
Stakeholders will hear?

- Lessons learned including but not limited to specific experiences that matter most to patients/caregivers
- What a meaningful therapy looks like to patients/caregivers (risk versus benefit)
- How do patients/caregivers want to be involved with the development of new treatments and/or therapies

Lots of Work to be done.....

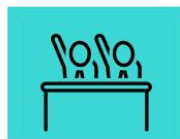
TIMELINE OF EVENTS

October 29th Krabbe disease Patient Focused Drug Development Meeting



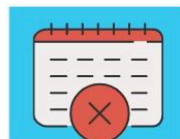
JAN 2020

- NORD Sent LOI to the FDA.



MARCH 2020

- Received approval from FDA, began planning meeting
- NORD invited other stakeholders to participate



APRIL

- Meeting postponed due to COVID-19 health and safety concerns

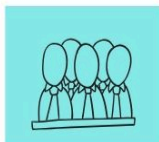


MAY-JULY

- Announce new virtual date-Oct. 29th
- Educate about PFDD meeting
- Register

TIMELINE OF EVENTS CONTINUED

October 29th Krabbe disease Patient Focused Drug Development Meeting



AUG 2020

- Panelist selection and training
- Continue event preparation



SEPT 2020

- Continue to work with panelist, speakers, and alternates
- Moderator
- Live-Polling
- PowerPoint Slides



OCT 29TH 2020

- Join us live 12:30 EST
- Have your voice be heard by the FDA



NOV-DEC 2020

- Post-event survey
- Compile data
- Transcribe video
- Begin work on published report of meeting

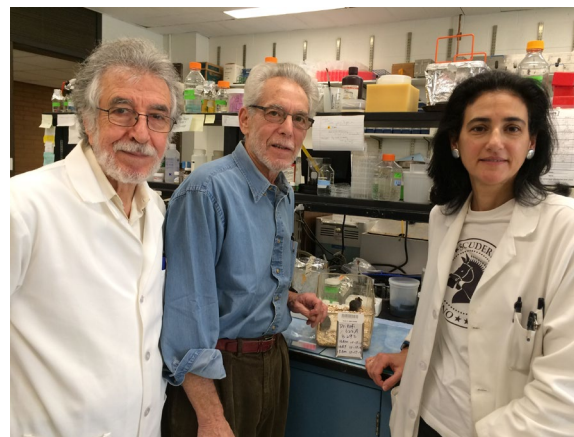


This is your chance!

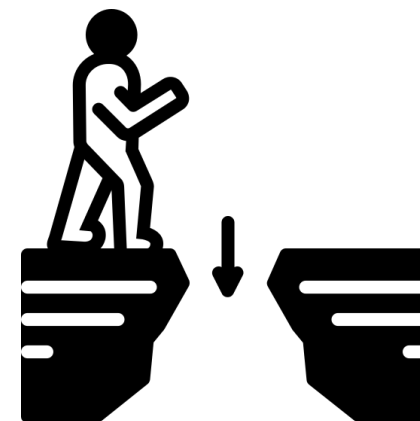
Let your VOICE be heard



Patient Stories



Latest Research



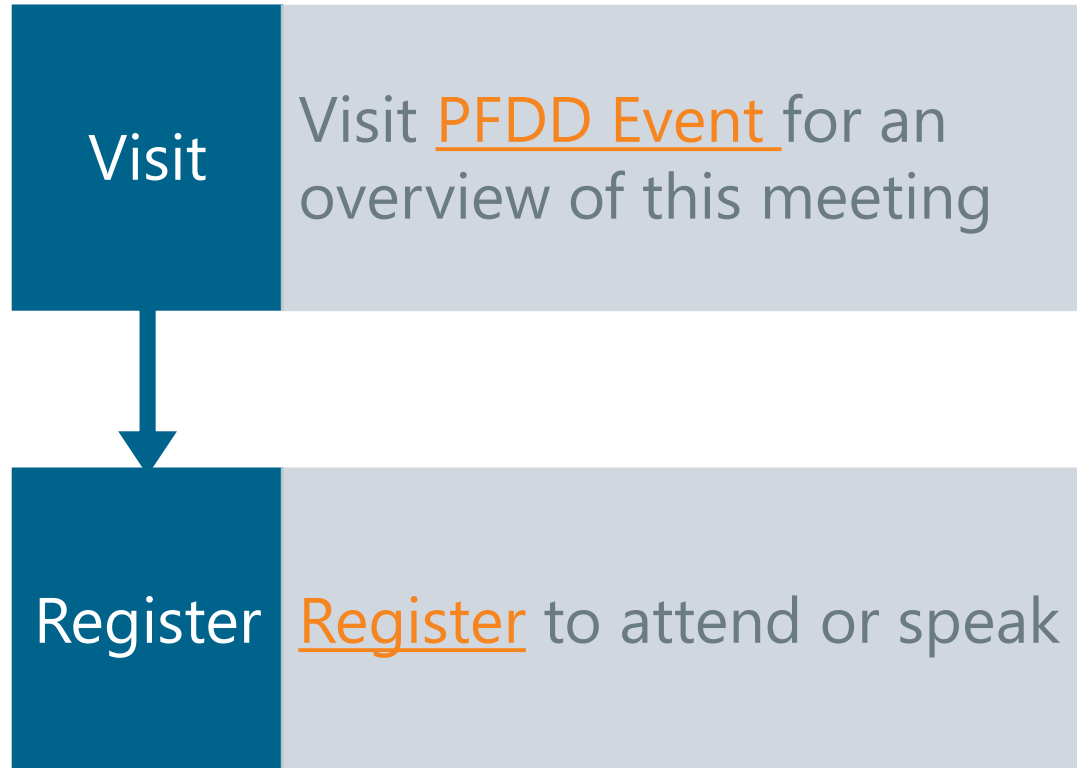
Gaps in Care



The Pipeline and Commitment

Steps to Participate

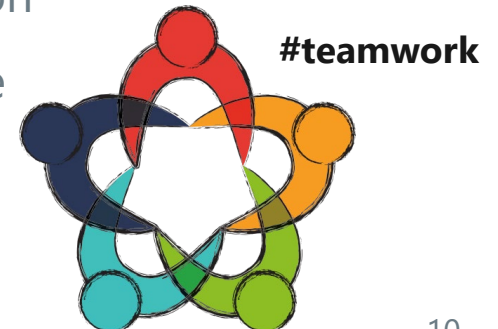
Facilitation by NORD



Please Note:

The following have been invited to participate:

- a. **All patients and caregivers**
- b. Partners for Krabbe Research Foundation
- c. The Legacy of Angels Foundation
- d. The Hunter's Hope Foundation
- e. Krabbe UK Foundation
- f. United Leukodystrophy Foundation
- g. Pharma companies working in the leukodystrophy space
- h. Clinicians and Researchers



Fiscal Sponsors Receive

Be Recognized
by NORD and
KrabbeConnect

Acknowledged in
the Voice of the
Patient Report

Resources to Learn More



SAMPLE EL-PFDD MEETING WITH
[NORD AND PK DEFICIENCY](#)



RECENT ARTICLE ON PFDD
MEETINGS CAN BE FOUND [HERE](#)



[PATIENT VOICE REPORT](#)

Thank You!

Debbie Drell:

ddrell@raredisease.org

Stacy Pike-Langefeld:

stacy.pike@krabbeconnect.org

Anne Rugari:

anne.rugari@krabbeconnect.org



#curekrabbe