Patient-Focused Drug Development Meeting (PFDD) Community Forum
Externally-led Patient-Focused Drug Development (PFDD) meeting for Fragile X Syndrome

• Opportunity to share patient and family perspectives, needs, priorities and experiences with FXS to inform FDA’s decisions and oversight of drug development and review of market applications.

• Wednesday, March 3rd from 10:00AM - 3:00PM ET
Setting the stage for a successful meeting

• The topic is *drug development* in *Fragile X syndrome*.
• No one can read our minds; we have to be clear and vulnerable.
• Four hours is not a lot of time!
• This will make a difference. We need **YOU**.
“... 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.”
What to expect- BEFORE

• Register for the PFDD meeting online
  • If you registered, you have already seen the discussion questions!

• Agenda & discussion questions posted

• Pro tip: Review the agenda and discussion questions, think about how you would answer them.
What to expect- DURING

• Honest, focused conversations moderated by experts. Support and active listening by the audience.

• Live and pre-recorded panelists.

• Raising your hand in Zoom.

• **Pro tip:** Be prepared, write out your talking points and make sure you leave room for others to share.
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Presenter(s)</th>
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<tbody>
<tr>
<td>10:00 – 10:05 am</td>
<td>Welcome</td>
<td>Linda Sorensen, Executive Director, National Fragile X Foundation</td>
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<tr>
<td>10:05 – 10:10 am</td>
<td>Opening Remarks</td>
<td>Randi Hagerman, MD, University of California, Davis MIND Institute and Founder of the National Fragile X Foundation</td>
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<tr>
<td>10:10 – 10:20 am</td>
<td>Overview of FDA’s Patient-Focused Drug Development Initiative</td>
<td>Proposed: Bernard Fischer, MD, Deputy Director (Acting) Division of Psychiatry (DP), Office of Neuroscience (ON), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA</td>
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<tr>
<td>10:20 – 10:30 am</td>
<td>Overview of Fragile X Syndrome and Current Treatment Options</td>
<td>Elizabeth Berry-Kravis, MD, PhD, Rush University</td>
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<tr>
<td>10:30 – 10:40 am</td>
<td>Overview of Clinical Trial Endpoints/Outcome measures</td>
<td>David Hess, Ph.D, University of California, Davis MIND Institute</td>
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<td>10:40 – 10:45 am</td>
<td>Overview of Discussion Format</td>
<td>Linda Sorensen, Executive Director, National Fragile X Foundation</td>
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<tr>
<td>10:45 – 11:15 am</td>
<td>Panel #1 Discussion on Topic 1: Health Effects and Daily Impacts of Fragile X Syndrome</td>
<td>Moderator: Marcia Braden, Ph.D. PC</td>
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<td>11:15 – 12:15 pm</td>
<td>Large-Group Facilitated Discussion Topic 1:</td>
<td>Individuals and caregivers in the audience will be invited to add to the dialogue.</td>
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<td>12: 15 - 12:30 pm</td>
<td>Break</td>
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<td>12:30 – 1:00 pm</td>
<td>Panel #2 Discussion on Topic 2: Current Approaches to Treatment</td>
<td>Craig Erickson, MD, Cincinnati Children’s Hospital Medical Center Chair of the NFXF Clinical Trials Committee</td>
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<td>1:00 – 2:00 pm</td>
<td>Large-Group Facilitated Discussion Topic 2:</td>
<td>Individuals and caregivers in the audience will be invited to add to the dialogue.</td>
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<td>2:00 – 2:05 pm</td>
<td>Closing Remarks</td>
<td>Linda Sorensen, Executive Director, National Fragile X Foundation</td>
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What to expect- Discussion questions

Discussion Topic 1: Health Effects and Daily Impacts of FXS
• Which 1-3 symptoms of FXS have the most significant impact on life? How do this impact daily life on the best days? How about the worst days?
• Are there specific activities or skills that are important to you/your child but that you/your child cannot do at all or as fully as you/your child would like because of FXS?

Discussion Topic 2: Current Approaches to Treatment of FXS
• What symptoms would you most like a treatment to target?
• What specific things would you look for in an ideal treatment for FXS? What would you consider to be a meaningful improvement?
What to expect- AFTER

• Debriefing sessions
• Open docket for comment
• Meeting recording and transcript
• Voice of the Patient Report
• Precompetitive work groups
Let’s look at three communities who had PFDD meetings:

• CLDK5
• Cystic Fibrosis
• Autism
Let’s ask some experts
Remember...

• The topic is drug development in Fragile X syndrome.

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• This will make a difference. We need YOU.
Happy to answer any questions: hilary@fragilex.org