# Agenda for the Public Meeting on Patient-Focused Drug Development for Fragile X Syndrome

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<tr>
<th>TIME</th>
<th>DESCRIPTION</th>
<th>PRESENTER/MODERATOR</th>
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| 10:00 – 10:05 AM | Welcome                                                                     | Linda Sorensen  
Executive Director, NFXF                                                                                                                   |
| 10:05 – 10:10 AM | Opening Remarks                                                            | Randi Hagerman, MD  
University of California, Davis MIND Institute &  
Founder of the National Fragile X Foundation                                                                 |
| 10:10 – 10:20 AM | OVERVIEW: FDA's Patient-Focused Drug Development Initiative           | Bernard Fischer, MD  
Deputy Director for the Division of Psychiatry  
(Office of New Drugs), FDA                                                                                                                    |
| 10:20 – 10:30 AM | OVERVIEW: Fragile X Syndrome and Current Treatment Options             | Elizabeth Berry-Kravis, MD, PhD  
Professor, Pediatrics, Neurological Sciences and Biochemistry, Rush University Medical Center                                                     |
| 10:30 – 10:40 AM | OVERVIEW: Clinical Trial Endpoints/Outcome Measures (or lack thereof) | David Hessl, PhD  
Professor, Department of Psychiatry, UC Davis                                                                                              |
| 10:40 – 10:45 AM | OVERVIEW: Discussion Format                                                  | Linda Sorensen  
Executive Director, NFXF                                                                                                 |
| 10:45 – 11:15 AM | TOPIC 1* Health Effects and Daily Impacts of Fragile X Syndrome        | Marcia Braden, PhD  
Clinical Psychologist                                                                                                                        |

_A panel of individuals and caregivers provide comments to start the discussion._

| 11:15 AM – 12:15 PM | TOPIC 1* Large-Group Facilitated Discussion                               | NFXF Team                                                                                                                                 |

_Individuals and caregivers in the audience are invited to add to the dialogue._

| 12:15 – 12:30 PM | BREAK                                                                      |                                                                                                                                     |
**Topic 1: Health Effects and Daily Impacts of Fragile X Syndrome**

1. Which 1-3 symptoms of FXS have the most significant impact? Are there specific activities or skills that are impacted.
2. How does FXS impact daily life on the best days? How about the worst days?
3. How has your/your child’s FXS changed over time? Is today different than the past? Why or why not?
4. What worries you most about your/your child’s FXS?

**Topic 2: Current Approaches to Treatment**

1. If you had to choose, what are the top symptoms of FXS you would most like a treatment to target?
2. What are you doing now to treat FXS? What factors do you consider when choosing a treatment? How has your treatment regimen changed over the years? Do you consider it effective?
3. What are the biggest gaps in you/your child’s current treatment(s)? What are the biggest downsides?
4. What specific things would you look for in an ideal treatment for you/your child’s FXS? Think about how much of a change/effect you would need to see to use a new treatment assuming there may be mild-moderate side effects.
   a. What impact level would convince you to trial a treatment? For example, a treatment that positively effects what % of patients with FXS.

If you would like to add a comment to the public docket, CLICK HERE.
Comments will be taken for 60 days following the meeting, ending on May 3, 2021.