U.S. FOOD & DRUG ADMINISTRATION

# Clinical Trials: The Patient Experience

Thursday, April 13, 2023, 11:00 a.m. – 3:00 p.m. ET

**Office of Therapeutic Products (OTP)**
**Center for Biologics Evaluation and Research (CBER)**
**U.S. Food and Drug Administration (FDA)**

**Hosted by:**
Anne Rowzee, Ph.D.
Associate Director for Policy, OTP, CBER, FDA

# Workshop Agenda

| | |
|---|---|
| **11:00 a.m.** | Welcome |
| **11:05 a.m.** | Gene Therapy Clinical Trials |
| **11:35 a.m.** | Panel Discussion — Perspectives From Patients and Caregivers (Part 1) |
| **12:35 p.m.** | Break |
| **12:50 p.m.** | Panel Discussion — Perspectives From Patients and Caregivers (Part 2) |
| **1:50 p.m.** | Q&A |
| **2:55 p.m.** | Closing Remarks |

# Virtual Meeting Considerations

- The webinar will be recorded and available online after the event.
- Closed captioning is available in Zoom.
- Use the Q&A box to submit questions throughout the event.
- Use the chat box to share general comments and report technical difficulties.

# RegenMedEd Series

- OTP's event series about regenerative medicine
- Goals of the RegenMedEd Series:

Discuss foundational information about regenerative medicine therapies, including gene therapy and cell therapy

Explore opportunities to engage with FDA and advance regenerative medicine research and drug development

Hear from FDA, patients, advocates, researchers, and other important stakeholders about their experiences

# Gene Therapy Clinical Trials

**Speaker:**
Rosa Sherafat, M.D.
Lead Physician
Office of Therapeutic Products
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

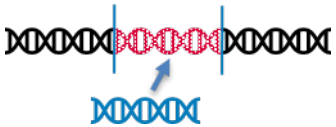
# Gene Therapy

Gene therapies use **genetic material** (DNA) to treat or prevent a medical condition.

- Cells use the information contained in genes to carry out their functions. Different cells express, or use, different genes.
- Scientists can use gene therapy to:

**Turn off a gene** that is causing a medical problem

**Repair a gene** so that it no longer causes a problem



**Replace a gene** that is causing a medical problem

**Add a gene** that helps the body fight a disease

# FDA's Role in Regulating Gene Therapies

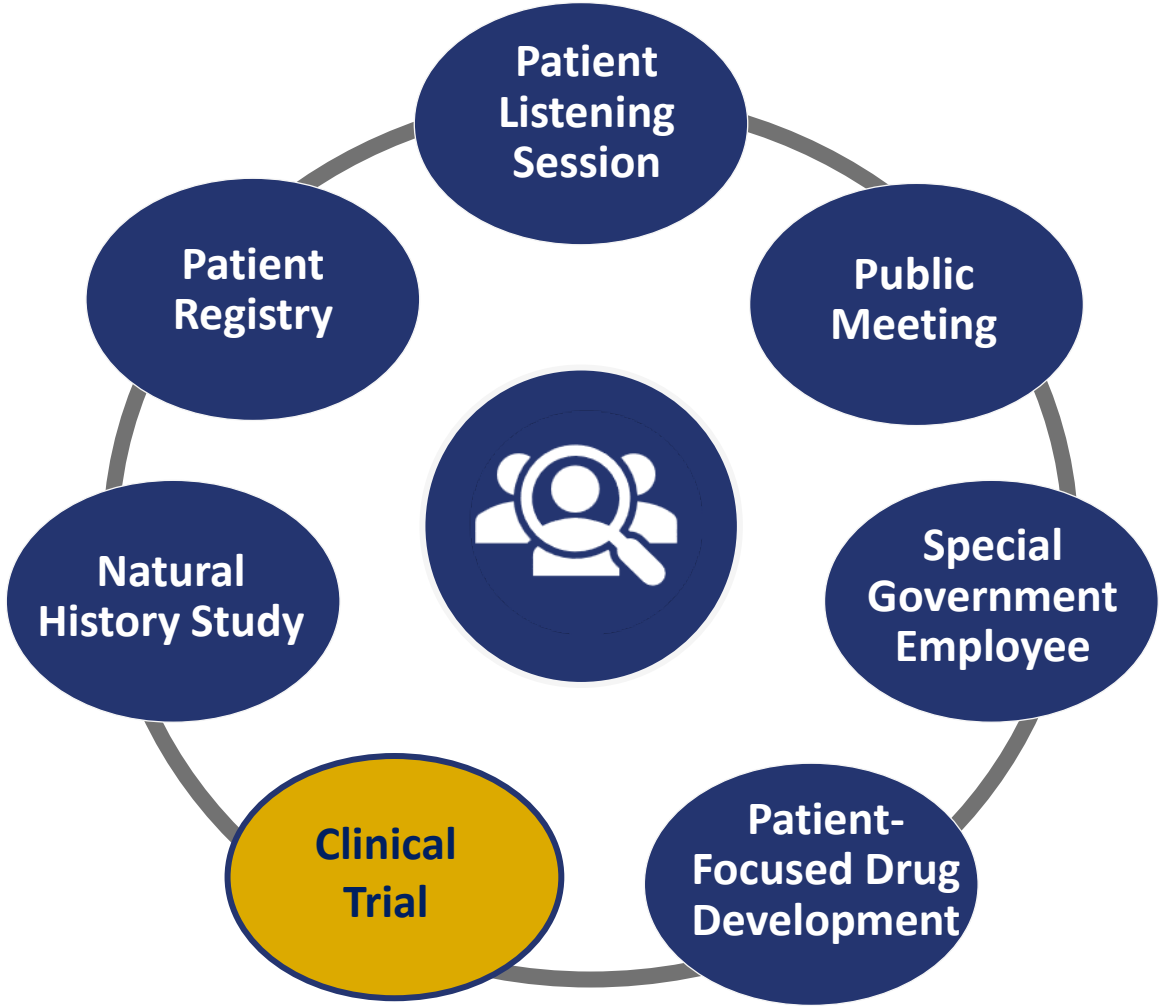
Provide **oversight of clinical trials** to protect **patient safety and rights**

Regulate products over their **entire lifecycle**—during development *and* after approval

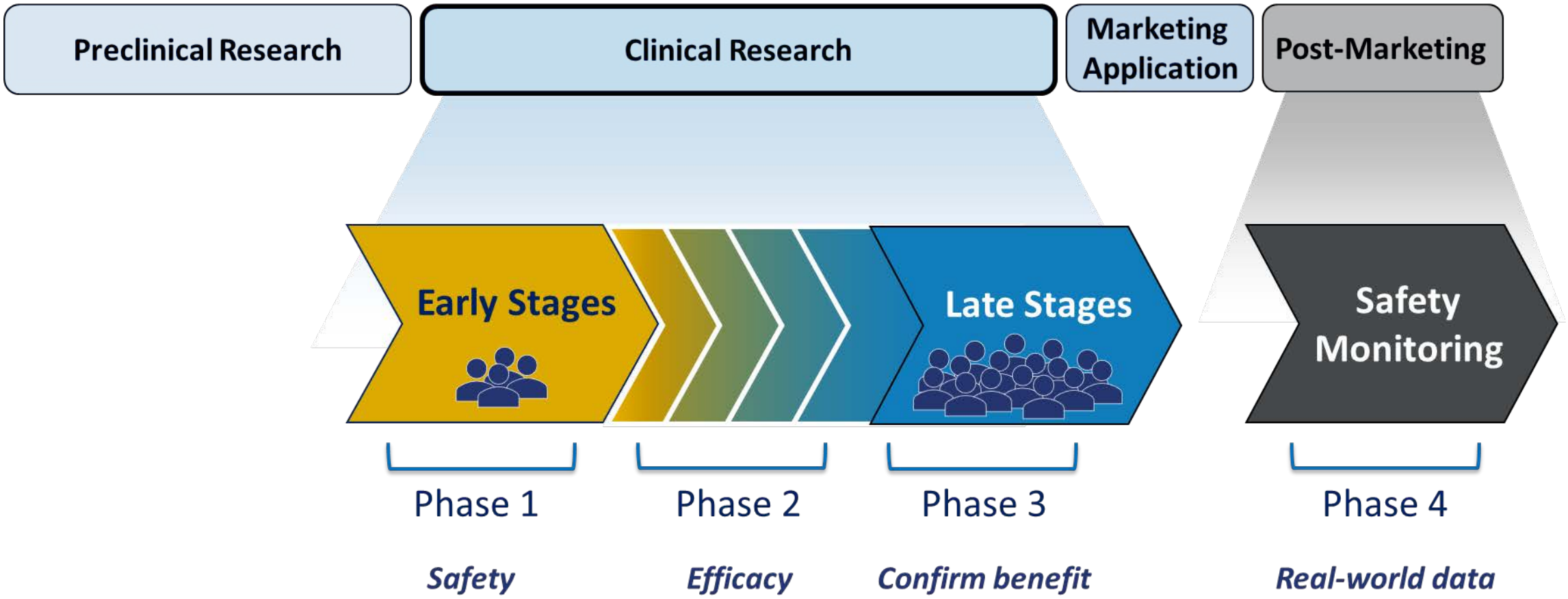
Publish **guidance documents** and **compliance standards** to educate manufacturers

**Engage stakeholders** to facilitate development of **products that meet patient needs**

# How Patients Can Help Advance Medical Research



# Drug Development Overview



# Key Differences in Gene Therapy

Unlike other medical products, gene therapies often involve:

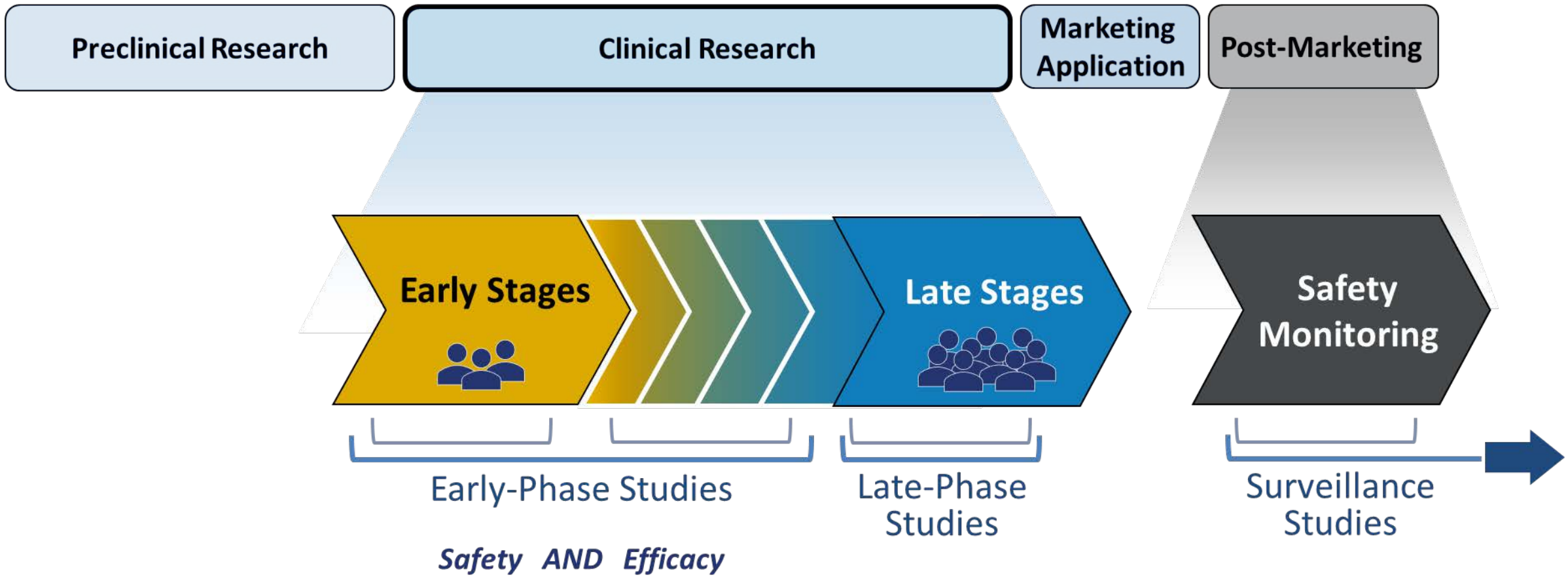
**New technologies** to manufacture the product

**Invasive procedures** to administer the treatment

**Long-lasting effects** that may never go away

**Potential risk** of unintended changes in DNA or rejection by the body

# Gene Therapy Clinical Trials



# Gene Therapies Hold Great Promise

OTP has oversight of over **1,000** investigational gene therapy products in clinical trials.

**80% of rare diseases** are caused by a single-gene defect.

FDA has approved **12 gene therapy products**, **4** of which are for single-gene disorders.

Patient participation and insight is critical for clinical research.

# Patient Perspectives on Gene Therapy Clinical Trials

**Today's panelists will speak about their experiences as patients and caregivers.**

What is it like to find and participate in a gene therapy clinical trial?

What are the risks and benefits of participating in a gene therapy clinical trial?

What are your hopes and expectations for the future of gene therapy?

# Contact Information

- **Regulatory Questions:**
  - OTP Main Line – 240-402-8190
  - Email: OTPRPMS@fda.hhs.gov
- OTP Learn Webinar Series
- CBER website
- **Phone:** 1-800-835-4709 or 240-402-8010
- **Consumer Affairs Branch**: ocod@fda.hhs.gov
- **Manufacturers Assistance and Technical Training Branch**: industry.biologics@fda.hhs.gov
- **Follow us on Twitter:** @FDACBER

*FDA Headquarters*

# Contact Information

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U.S. Food and Drug Administration

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# Panel Discussion — Perspectives From Patients and Caregivers, Part 1

**Moderator:**

Karen Jackler, M.P.H.

Patient Engagement Program Manager

Center for Biologics Evaluation and Research

U.S. Food and Drug Administration

# Meet Our Panelists

**Bobby Wiseman**
Hemophilia patient and advocate

**Suzette James**
Board of Directors, BDSRA Foundation
CLN2 Batten disease parent and advocate

# SUZETTE JAMES

CLN2 BATTEN MOM
MEMBER OF THE BDSRA
BOARD OF DIRECTORS

*2023 FDA GENE THERAPY PANEL*

# Clinical Trials:
## The Patient Experience

- Maya (19), diagnosed at 10
- Xavier (15), diagnosed at 9
- “I once Raced a Whippet” by Maya James
- Disease Progression for Maya and Xavier

“This message is so simple, yet it gets forgotten.
The people living with the condition are the experts.”

**-Michael J Fox**

# Brineura:
## The Journey to Treatment

- Maya did not qualify
- Tested our other children
- #FightingforMaya
- Maya's Mice
- The call to enroll Maya in compassionate use program

# Gene Therapy:
## Accelerated Path and Approvals

- Potential treatments fall off from lack of funding or progress through FDA
- Brineura is only part of the solution, one piece of the puzzle

# Gene Therapy:
## Informed Consent

- We become the experts of our children and their diseases
- We consult our trusted clinical teams, advisors, our scientists, and FDA pre-clinical data

# Fatal Disease:
## Elle Belle and Our Ask

- Parents are a critical part of the equation
- Grateful for Brineura
- Need accelerated pathways for gene therapies
- Maya’s reflection of her friend Elle

# Break

12:35 p.m. to 12:50 p.m. ET

**Stay tuned! Our panel discussion will resume at 12:50 p.m. ET.**

# Panel Discussion — Perspectives From Patients and Caregivers, Part 2

**Moderator:**

Karen Jackler, M.P.H.
Patient Engagement Program Manager
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

# Meet Our Panelist

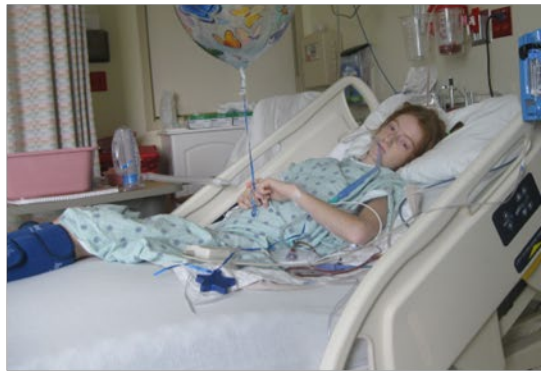

**Shandra Trantham**
Friedrich's ataxia (FA) patient and advocate

# Gene Therapy in Friedreich's Ataxia: A Patient Perspective

Shandra Trantham

# Who I Am

- 25 years old, diagnosed with FA at age 12
- Ph.D. Candidate in Genetics at the University of Florida
- Very active in the FA patient community



# What is FA?

- Progressive neuromuscular disease
- Childhood onset
- Fatal
- 1st treatment that FDA approved in February 2023 slows progression, is not a cure

# What is it like to participate in a clinical trial?

1st dose of the FDA-approved COVID vaccine, January 2020

- Feels like the optimism you experienced getting your first dose of the COVID vaccine nearly a year into the pandemic
- It can also be nerve-wracking and time-consuming, and can force you to become an expert decision-weigher

# Gene Therapy is Different

- Risk-benefit analysis includes new factors:
  - Irreversible dosing/ safety concerns
  - Long trial lengths
  - Individual therapeutic expectations
  - Exclusion from future trials
  - Choice of therapeutic target vs symptom burden

# How is the FA Community Reacting to Gene Therapy Clinical Trials?

Chatter about high expectations for efficacy (hope)

Uncertainty about treatment target

Unfamiliarity with process

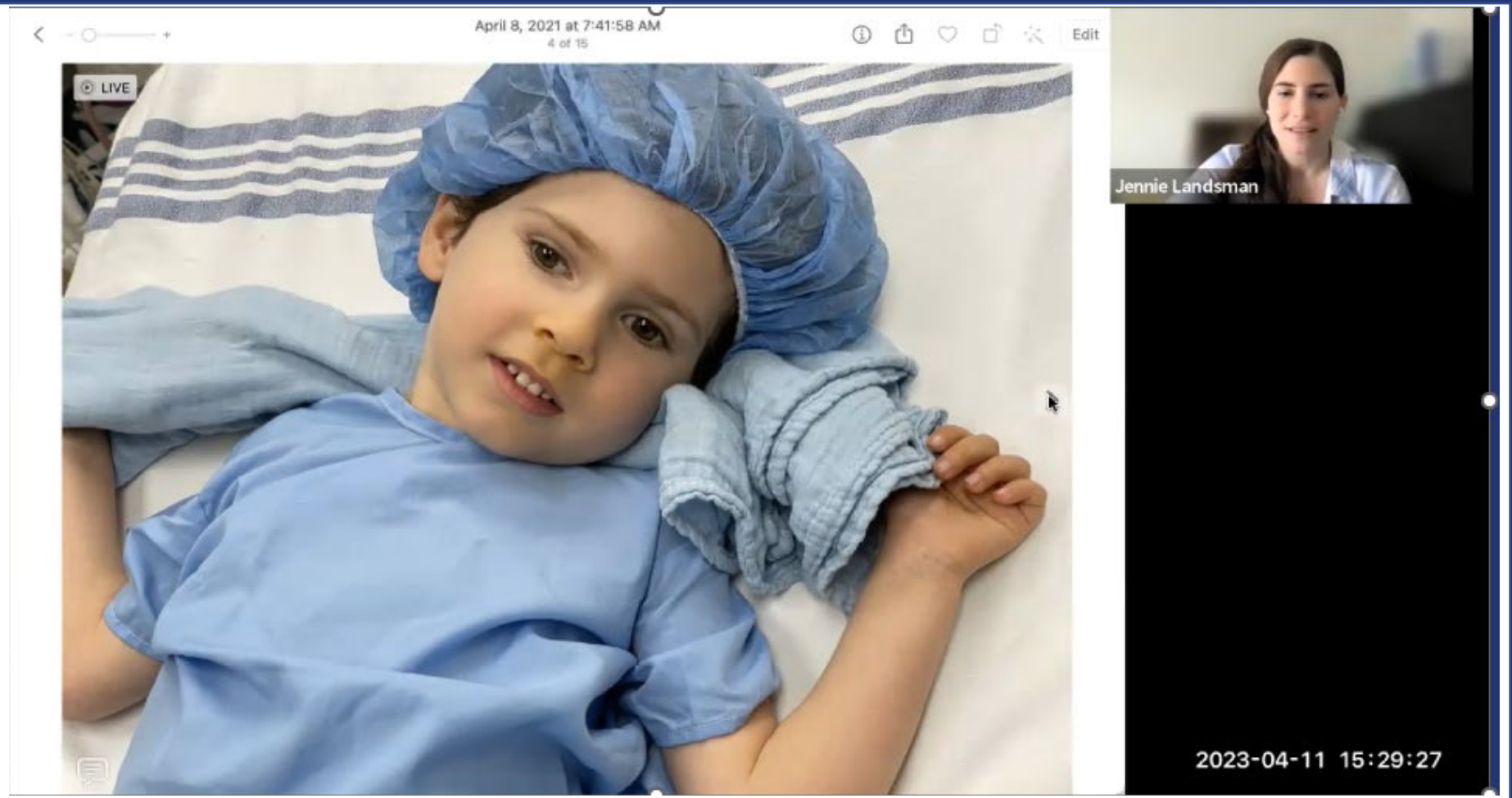
Patient advocacy organizations, like FARA, are pushing education

# Meet Our Panelist

**Jennie Landsman**

Canavan disease parent and advocate

# Jennie Landsman




Jennie Landsman's video is part of the full workshop video and can be viewed from time marker 2:03 to 2:51.

# Stay Connected!

**Helpful Resources:**

- Visit CBER's website
- Sign up for our newsletter, "What's New @ CBER"
- Follow us on Twitter: @FDACBER
- Share your thoughts with us using **#RegenMedEd**

# Thank you!

Workshop materials will be available in the coming weeks on FDA.gov.

Stay tuned for upcoming OTP events:

- *April 25* — Town Hall: Gene Therapy Chemistry, Manufacturing, and Controls
- *April 27* — Public Listening Meeting: Post-Approval Safety and Efficacy Data on Cell and Gene Therapy Products

**#RegenMedEd**