

# Duchenne Citizen Science

## *Conference Polling*

**Parent** JOIN THE FIGHT.  
END DUCHENNE.  
**Project**  
**Muscular**  
**Dystrophy**

Ryan Fischer  
SVP Community Engagement

# Duchenne Citizen Science



# Patient Focused Drug Development (PFDD)

*Moving From Passengers to Co-pilots in Drug Development*

- Food and Drug Safety and Innovation Act of 2012 (PDUFA V) – this established PFDD
- Further improved through 21st Century Cures Act & PDUFA VI



*“The 21<sup>st</sup> Century Cures bill codifies patient focused drug development as a part of the FDA’s mission.”*

*- Dr. Janet Woodcock,  
Director Center for Drug Evaluation and Research*

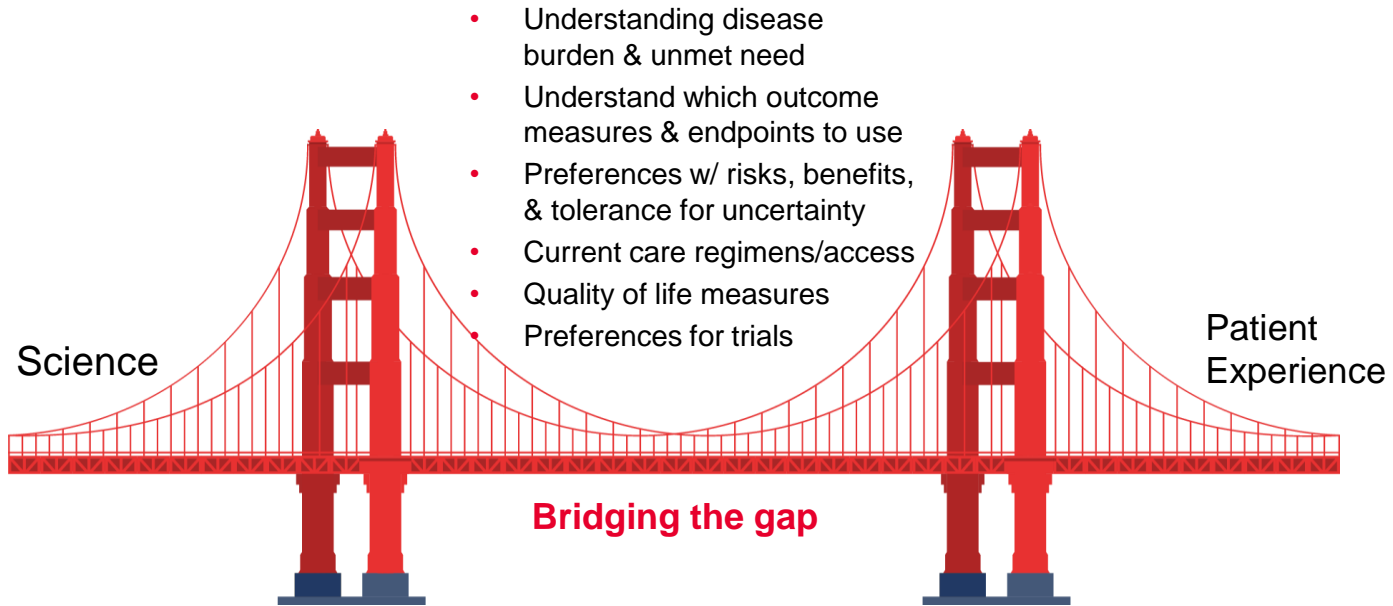
Discovery &  
Preclinical

Development  
(trials)

Regulatory

Post-  
Market

# Advancing the science of patient input



# Conference Polling

# Conference Polling Goals

- **Engage different stakeholders** in one setting
  - Adults with Duchenne or Becker
  - Duchenne/Becker families
  - Healthcare professionals, members of industry, and others invested in Duchenne
- **Gain perspective** on a range of topics
- **Inform** the discussion here and moving forward

# Polling Guidelines

Polling will take place Friday and Saturday in between sessions.  
Those watching the stream can also participate.

To participate visit: [pollev.com/ppmd](https://pollev.com/ppmd)  
Wireless code: **PPMD 2018**

- You will be prompted for when you need to poll on a questions

## Polling Questions

- Questions for everyone
  - Some questions will be for all stakeholder to answer
- Questions for families
  - Some questions multiple families members can participate
  - Some questions we will ask one family member to answer on behalf of family
- Questions for Industry and Clinicians/Healthcare Professionals
  - Separate question sets for both members of Industry and healthcare professionals

Polling warm up