Duchenne Citizen Science

Conference Polling

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SVP Community Engagement
Duchenne Citizen Science

- Trials/Natural Hx
- Duchenne Registry, focus groups, PRO, RWE
- Patient Preferences Surveys
- Direct Advocacy (PFDD)
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 21st 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
Advancing the science of patient input

Bridging the gap

Science

• Understanding disease burden & unmet need
• Understand which outcome measures & endpoints to use
• Preferences w/ risks, benefits, & tolerance for uncertainty
• Current care regimens/access
• Quality of life measures
• Preferences for trials

Patient Experience
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
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