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Title: Community-engaged approaches to explore research priorities in Duchenne and Becker muscular dystrophy

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Propositions
Related to the thesis

“Community-engaged approaches to explore research priorities in Duchenne and Becker muscular dystrophy”

Holly Landrum Peay

1. Though Duchenne (DMD) and Becker muscular dystrophy (BMD) are associated with significant burden, interventions to improve mothers' psychological adaptation should focus on fostering resilience and enhancing benefit finding (*this thesis*).
2. Among DMD worries spanning medical and psychosocial domains, parents prioritized child's symptom progression and access to medical care (*this thesis*).
3. Parents indicated willingness to accept treatment-related risk for a serious or fatal outcome when balanced by slowing progression, even absent lifespan improvement (*this thesis*).
4. Parents who consented children to clinical trials described strong decision making pressures attributed to the progressive, fatal nature of DMD (*this thesis*).
5. Though anticipated benefit to the child was a strong clinical trial motivator, most parents did not display true therapeutic misconception (i.e., misunderstanding the intent of clinical trials) (*this thesis*).
6. Community engagement in research is essential to proposing and answering questions of highest relevance, especially when faced with numerous, compelling unmet needs (*this thesis*).
7. Clinical researchers should engage their disease communities to understand patient/caregiver lived experiences, extending beyond what is seen in clinic.
8. Clinical researchers should appreciate the importance of assessing positive disease appraisals and outcomes.
9. Public health and regulatory policy making should integrate priorities and preferences of patients/caregivers.
10. Severe progressive disorders, where doing nothing is equated with doing harm, requires increased regulatory flexibility in research design and implementation.
11. Researchers exploring clinical trial decision making should advance models integrating and differentiating cognitive and emotional influences.
12. “Decision makers...will make better decisions when they trust their critics to be sophisticated and fair, and when they expect their decision to be judged by how it was made, not only by how it turned out.” (Kahneman D. *Thinking, Fast and Slow*, p. 418. New York: Farrar, Straus and Giroux, 2011.)