Treatment Preferences and Risk Tolerance Study

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Study Goals

- Objective: explore how parents/guardians of individuals with DMD prioritize risk and benefit in the context of new therapies
- Specific Aims:
  - Describe risk tolerance, health-related QoL, and numeracy
  - Explore treatment preferences, risk tolerance and benefit priorities
  - Evaluate the effect of child's disorder progression on treatment preferences
  - Explore Duchenne-related worries

Worry List: In the past 7 days, most/least worried…

A Child focused	My child missing out on new treatments
B Child focused	My child getting weaker
C Child focused	Getting the right care for my child over time
D Child focused	My child feeling happy
E Child focused	My child having good friends
F Child focused	My child feeling like a burden on the family
G Child focused	My child becoming independent from me over time
H Child focused	My child not being able to express deep worries
J External focused	Being a good enough parent for my child
K External focused	Me handling the emotional demands of Duchenne
L External focused	Managing my uncertainty about my child's future
M External focused	Having time for myself
N External focused	Feeling isolated from other families
P External focused	Affording care my child needs within the family budget
Q External focused	Effects of Duchenne on my closest relationships
R External focused	The wellbeing of my other children

Treatment Attributes

LABEL	EXPERIMENT DESCRIPTION
A1 Muscle function	Stops the progression of weakness
A2 Muscle function	Slows the progression of weakness
A3 Muscle function	Does not change progression of weakness
B1 Lifespan	5 year gain in expected lifespan
B2 Lifespan	2 year gain to expected lifespan
B3 Lifespan	No extra gain to expected lifespan
C1 Drug knowledge	2 years of post-approval drug information available
C2 Drug knowledge	1 year of post-approval drug information available
C3 Drug knowledge	No post-approval drug information available

Treatment Attributes Con't

Label	Experiment description
D1 Nausea	No increased chance of nausea
D2 Nausea	Causes loss of appetite
D3 Nausea	Causes loss of appetite with occasional vomiting
E1 Bleeds	No increased risk of bleeds
E2 Bleeds	Increased risk of bleeding gums and increased bruising
E3 Bleeds	Increased risk of hemorrhagic stroke and lifelong disability
F1 Arrhythmia	No increased risk of heart arrhythmia
F2 Arrhythmia	Increased risk of harmless heart arrhythmia
F3 Arrhythmia	Increased risk of dangerous heart arrhythmia and sudden death

Inclusion Criteria & Recruitment

- Recruited from PPMD, DuchenneConnect Registry, and snowball recruiting
- Parents or guardians of at least one living child with Duchenne muscular dystrophy, living in the United States, over 18 years of age, and able to complete an online survey in English
- Study determined to be exempt by the Western Institutional Review Board

Preliminary Results

- 119 parents completed entire survey
- Mean participant age 43.7 (SD 7.7)
- Mean affected child age 12.1 (SD 6.4)
- 80 (67%) biological mothers, 34 (29%) biological fathers, 5 (4%) adoptive parents
- 109 (92%) Caucasian
- 107 (90%) married, 11 (9%) divorced/separated, and 1 (1%) widowed

Affected Children

- 110 (92%) have one affected child; 9 (8%) have two or more affected children
- 101 (85%) have private insurance; 40 (34%) have a state/government program
- 68 (58%) participated in clinical research and 40 (34%) participated in a clinical trial
- 22 (19%) child has experienced a life-threatening emergency that caused parent to worry that the child would die

WORRY PRELIMINARY RESULTS	Utility score
My child getting weaker	-0.637
Getting the right care for my child over time	-0.254
My child missing out on new treatments	-0.245
My child feeling happy	-0.161
Managing my uncertainty about my child's future	-0.127
Affording care my child needs within the family budget	-0.065
My child having good friends	-0.038
My child not being able to express deep worries	-0.025
Being a good enough parent for my child	-0.012
The wellbeing of my other children	0.038
Me handling the emotional demands of Duchenne	0.049
My child feeling like a burden on the family	0.179
Effects of Duchenne on my closest relationships	0.217
My child becoming independent from me over time	0.232
Feeling isolated from other families	0.300
Having time for myself	0.557

Preliminary Conclusions

Participants prioritized protection of muscle function over any other attribute, including longer lifespan and each of two serious risks.

Participants' most significant worries were related to the child's illness progression and care.

The study suggests a parent population that is highly concerned about DMD's effect on their child's strength, and is willing to accept risk and uncertainty for a treatment that would slow or stop muscle weakness.

Collaborators

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