

The Burden of Clinical Trials: One Family's Perspective

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Mother of Garrett, who lives with Duchenne



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

An Honest Informed Consent

- Do you agree to collect a stool sample from your child and carry it in a Ziploc bag in your purse every month?
- Do you agree to work all day, drive to the clinic site through the night and arrive promptly at the hospital at 8am in a time zone an hour earlier than your own?
- Do you agree to make your child do schoolwork while in the hospital with 2 IVs hanging from his arms so he doesn't get behind in school while you try not to feel guilty about it?
- Do you agree to float \$300-\$500 up front each visit to pay for travel and meals while awaiting your reimbursement check from the sponsor?
- Do you agree to entertain your child and his siblings in a 9x9 hospital room for 6 hours each visit with reruns from the Disney Channel while continuously reminding them to sanitize their hands?
- Do you agree to sleep in a lumpy hotel bed under a questionably clean comforter every month while the toilet continuously runs through the night and random strangers run through the halls?

If so, YOU MAY BE READY TO START A CLINICAL TRIAL!

Psychosocial Burden

- Expectations
 - Real Hope vs Misguided Hope
- Emotional strain
 - Child
 - Pressure to perform well on tests
 - Physical discomfort and associated anxiety
 - Siblings
 - At risk for less support from parents
 - Taking on more adult roles at home
 - Parent
 - Increased anxiety and depression
 - Marriages and family life

Financial Burden

- Up-front costs each visit
 - Food
 - Gas/Flight
 - Hotel
- Covered vs. non-covered expenses
 - Siblings
 - Spouses
- Hidden Expenses
- Lost Wages

Logistical Burden

- Jobs that allow for flexibility
- Schools that allow for flexibility
- Coordination of travel and lodging
 - Some sponsors use a 3rd party travel company
- Difficulty of travel with disease progression and growing equipment needs
- Time Commitment
 - Ex: 1x/month for 2 years plus Open Label Extension
 - Factoring in the Wash Out Period

Benefits of Clinical Trials

- Potential therapeutic benefit
- Close monitoring by research team, including doctors
- Potential extended access to a new drug during an open label extension
- Altruistic benefit
- Building of family relationships

“Sometimes in life we become so focused on the finish line that we fail to find joy in the journey.”

-Dieter F. Uchtdorf

