The Burden of Clinical Trials: One Family’s Perspective

Amber Sapp, PT, DPT
Mother of Garrett, who lives with 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 3\textsuperscript{rd} 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