Patient Perspective: The Clinical Trial Process

Pat Furlong

Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

CLINICAL TRIALS

- Opportunity to change the trajectory?
- Words matter (expectations vs reality)
- Inclusion Criteria
- Burden of Participation
- Extended Access Programs?
- Will I learn my (or my child's) response?
- What happens next

Informed Consent (or ticket in?)

- **Nobody can prepare you for the roller coaster your family will ride when you sign up for a trial… simply signing an informed consent doesn't prepare you for the physical and emotional impact of a trial.**

Parent Perceptions of Clinical Trials

- Therapeutic misconception
  - Study of 38 parents involved in the rare disase Phase 2 study
  - *ALL* parents reported expecting some direct benefit of the drug
    - all reported being well informed via multiple sources of information (PI, advocacy, research, etc.)
    - “I did my research so thoroughly that I was convinced it was a cure.”

Peay H., et al. Expectations and experiences of investigators and parents involved in a clinical trial for Duchenne/Becker muscular dystrophy. Clinic Trials. 2014 Feb; 11(1):77-85.

CLINICAL BENEFIT vs MEANINGFUL BENEFIT

6 MWT

4 STAIR CLIMB

Life Span

- Slow/Halt Progression
- Walk/Stand
- Self-Feed
- Touch Head
- Turn Over in Bed
- Wrist and Finger Function
- Breathing

Clinical Trials: Burden of Participation (in real time!)

- **The difference between being a patient and being a subject**.
  - When you are a patient, you are treated with urgency. You don't have to wait months on end for people to tell you what's happening with your condition. People don't sit on paperwork that delays your care for weeks at a time.
  - When you're a subject, you have no right to expect urgency. the research wheels slowly churn was very difficult, and the timelines are constantly moving out further and further.
  - Care: One example - an echocardiogram isn't supposed to hurt, right? when you're a subject, the tech tells you, "I've got to get the pictures for the study." Tough luck for your kid, in other words.
  - I did not understand that companies would not feel any obligation towards the kids who sacrificed so much for clinical trial participation. I thought that once our kids had given their pound of flesh, so to speak, that there would be a sense of obligation in return

Emotional and Physical Burden

- I **did not know how hard it would be on my son**. I was so desperate to get him on some drug, any drug, that I did not consider the fact that we were medicalizing his life at a time where he would ordinarily only be going to the hospital once or twice per year. He was a much sadder kid during that year on placebo - so sick of the airport, so sick of the hospital, so tired of being away from home. I don't think that I respected his opinion and desires enough in weighing our decisions.
- Less important than his feelings is that I did not expect how many logistics would be added to our lives - it was like having another part-time job. I had to make travel arrangements each week, arrange childcare each week, submit expense receipts each week for taxis, etc., I underestimated the toll that the simple logistics would take, and the fun family times that they replaced. I also underestimated the toll on my other children, who missed both their brother and me in the frequent travel, and whose needs always got usurped by the trial.

TIME and Sacrifice

- we have spent a total of 28 nights in hospital and 52 days as inpatients
- give blood, urine, sweat, tears and access to every part of his body and life…
- complied with every test and measure with military precision
- stopped him drinking and eating the things he loves to adhere to strict protocols, foods that actually give him pleasure and make him enjoy his life like chocolate and lemonade

Cost of Participation

- **Required to be patient when 3 month trials have turned into over five months,**
- **changed our whole families diets,**
- **sacrificed holidays, cut back on luxuries to afford to take part in trials because although the company covered travel costs, they don't cover childcare costs for siblings,**
- **loss of earnings through taking unpaid leave**
- **cost of the number of presents I have had to get him for taking part is probably in the thousands.**

Financial Burden

- **I did not know expensive it would be**. The costs vary per trial, I had to go part time with work, pay for child care once per week for my other children, etc. At one point, the hospital was so behind in reimbursement that I was out an additional $6,000 in covered expenses. I was not prepared in advance for the financial toll on my family.

Uncertainty

- **countless nights I laid on a rock hard camp bed watching him sleep, watching him take every breath, watching the minutes of the clock tick by, seeing them turn into hours and then days praying he will be ok and that he won't encounter an adverse event.**
- **Who knows what could happen to the first children exposed to this drug?**

Placebo

- Use of Placebo:
  - – I thought that if we really were able to identify who was on drug and who wasn't, that they would just use an untreated control group alongside the treated group, not put them through the pain and damage of a year's worth of fake injections and needless surgery.

Rolling with the Punches

On Friday, Sam had surgery to place an internal port for his weekly clinical trial infusions. It did not go as planned. The surgeon saw the port they had placed was too small and had to remove it**.** Unfortunately, and unbeknownst to us, the hospital did not stock larger ports. The surgeon spoke with our doctor's team (who had spoken with her, as she was out on vacation), and his new recommendation was an external catheter.... We ok'ed this believing our doctor thought it was the best option. The problem with this was we were not informed of the restrictions an external catheter would place on Sam. Our doctor told her team to make sure the surgeon told us, but someone dropped the ball. These restrictions have to do with swimming and showering. Swimming is restricted with it, and showering is difficult at best. There are also major care differences. The internal port requires us to do no extra care at home. The external catheter requires daily flushing with heparin and cap and dressing changes. It's also far more prone to infection. Because Sam is immune-suppressed, this could be quite the issue. Another major issue was no care instructions or supplies given to us upon discharge. We were told what to watch for regarding post surgery infection, but that's it. And since we had not been informed of the care differences, we did not know we should have been given supplies and further instruction.

More Punches…and still

So we arrived home after discharge thinking all was well. I decided to Google information on his external catheter. That's when I discovered the restrictions and extra care. We phone the surgery department to try to figure out what was going on. Now after speaking with our doctor, and agreeing with us that swimming, Sam's only form of exercise, is of utmost importance, we are needing to schedule him for another surgery to remove the external catheter and place an internal port of the correct size. Putting Sam under for surgery is never an easy decision. He has more risks than the average child. But he's healthy, and we all believe the benefits are worth it. We don't know yet when the surgery will be. We're trying for the soonest possible so he can get back in the pool

DATA

- What was my response?
- Communication = Study and Results?
- Label
- Access and reimbursement

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

ENDDUCHENNE.ORG