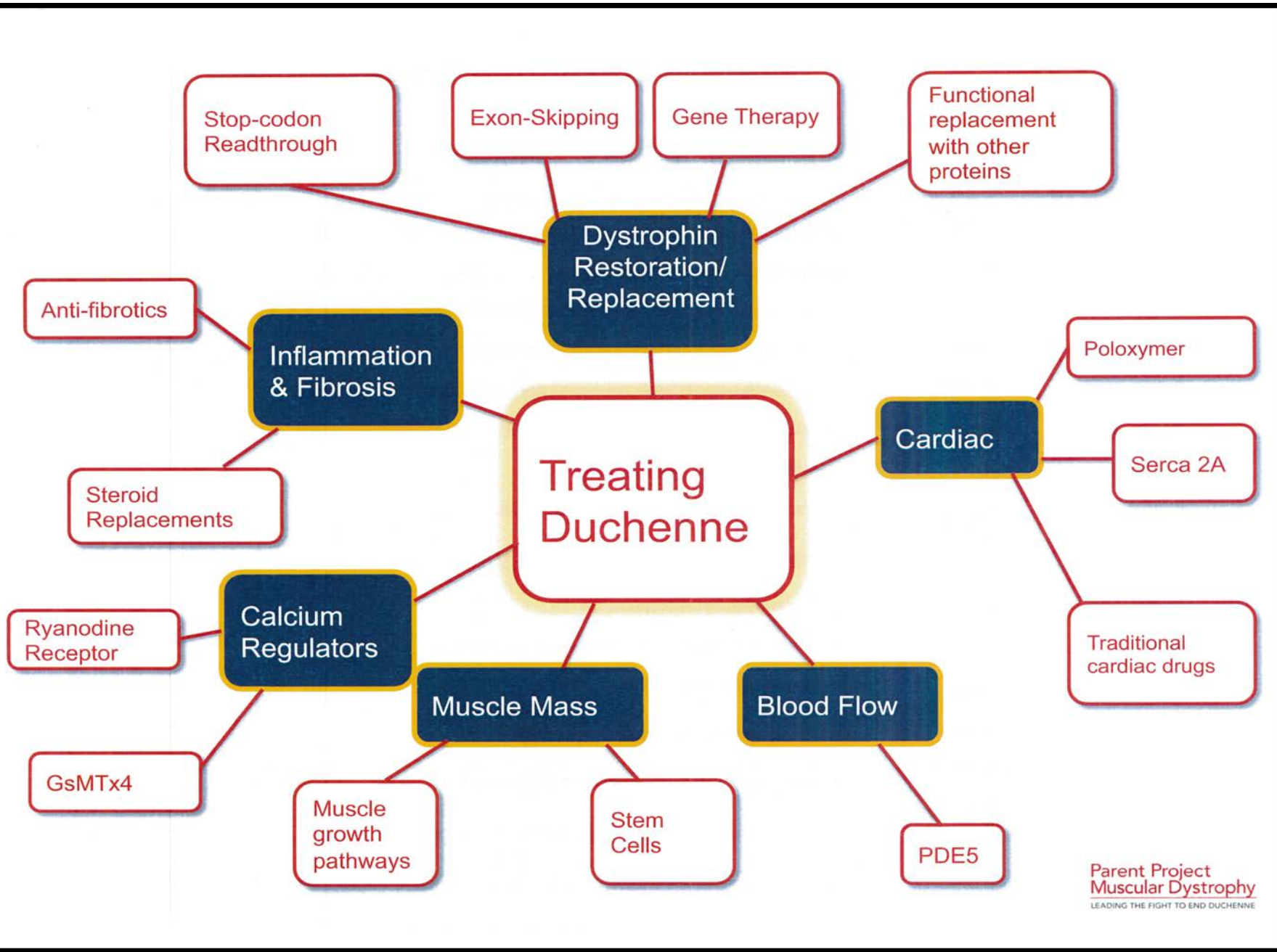


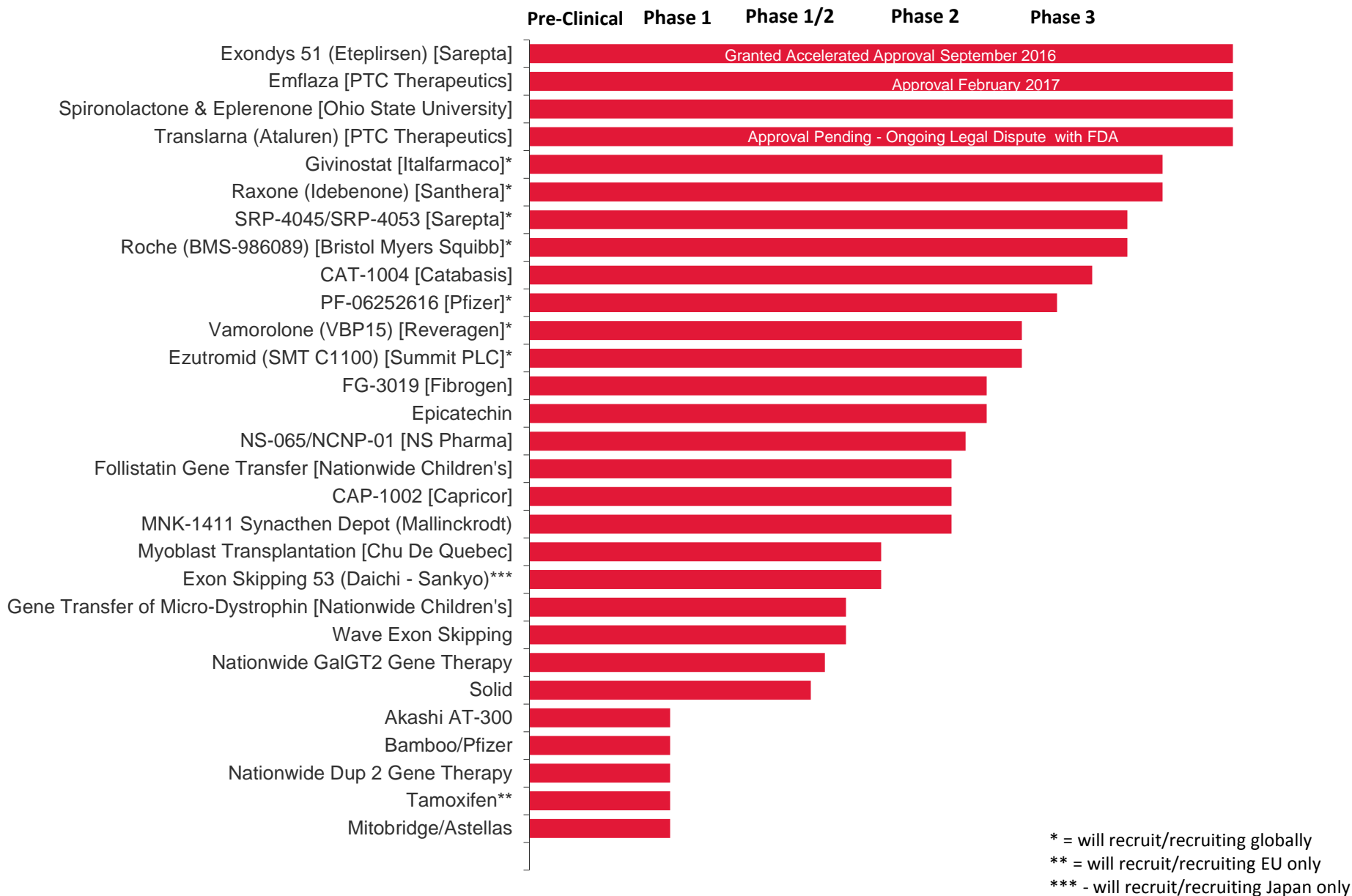
# Clinical Trial Decision Making: Helping Families Navigate the Current Clinical Trial Landscape

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



# DUCHENNE THERAPEUTIC PIPELINE





# The “Problem”



# “So which trial should we choose, doc?”

- Lots of factors to consider (MD and family):
  - Likelihood of benefit (if so, how much?)
  - Likelihood of harm (from therapy, anxiety, invasive procedures)
  - Logistical demands of the study
    - Travel distance
    - Frequency and duration of visits
- Make sure parents don't feel “guilty” about saying “no” to trials
- Make sure parents understand that trials are “experiments”, not “new treatments”
- All studies share one common denominator: ***an already stressful life will become more complicated***

# “So which trial should we choose, doc?”

- Some trials require boys to be steroid-naïve
  - What if boy is 5 yo, family waiting for study and start-up lags?  
And then, he screen fails?
- Will participation in Trial A exclude me from Trial B?
- Can I drop out of Trial A (Yes) in order to be eligible for Trial B (Maybe)?
  - If I do that, and don't get into Trial B, where does that leave me?
- Will the fact that I'm taking FDA-approved Drug A exclude me from Trial B?
  - If so, should I stop taking Drug A?
  - If I do that, I still might not get into Trial B
  - Maybe I get into Trial B, but it turns out the therapy in Trial B is ineffective (and it took them 3 years (with me off of Drug A) to figure that out).

# “So which trial should we choose, doc?”

- Ironic that now with more choices, life for our families/patients has become more difficult in some ways
- I think the best we (HCPs) can do is offer open, honest, ongoing communication
- No way to anticipate every scenario
- Help families that are motivated to pursue clinical trials access accurate, updated information (DuchenneConnect)
- Make sure all of your patients are genotyped AND that the genetics report is accessible



# Questions/Comments?

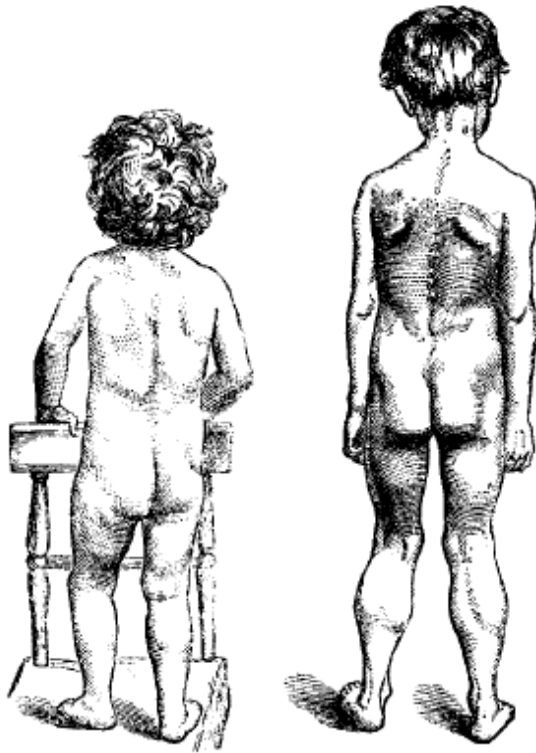


FIGURE 8. Two brothers aged 4 (Harvey S, Case 3) and 7 (William S, Case 4) from Gowers. "The youngest. . .would not suggest to you the idea of disease. There is no obvious muscular wasting or enlargement, and yet. . .his movements were greatly impaired. He could only just succeed in rising from the floor. . .The other boy. . .as his photograph indicates, [has] very distinct enlargement of the calves. His thighs are small, the back thin, hollow in the lumbar region, the angles of the scapulae prominent, the muscles of the upper limbs thin, except the deltoids, which are rather large."<sup>27</sup>

The disease is one of the most interesting, and at the same time most sad, of all those with which we have to deal: interesting on account of its peculiar features and mysterious nature; sad on account of our powerlessness to influence its course, except in slight degree, and on account of the conditions in which it occurs. It is a disease of early life and early growth. Manifesting itself commonly at the transition from infancy to childhood, it develops with the child's development, grows with his growth—so that every increase in stature means an increase in weakness, and each year takes him a step further on the road to hopeless infirmity, and in most cases to an early and inevitable death.<sup>26,27</sup>

Tyler K. *Muscle & Nerve* 2003

## 4:00 PM – Wrap-Up & Adjourn