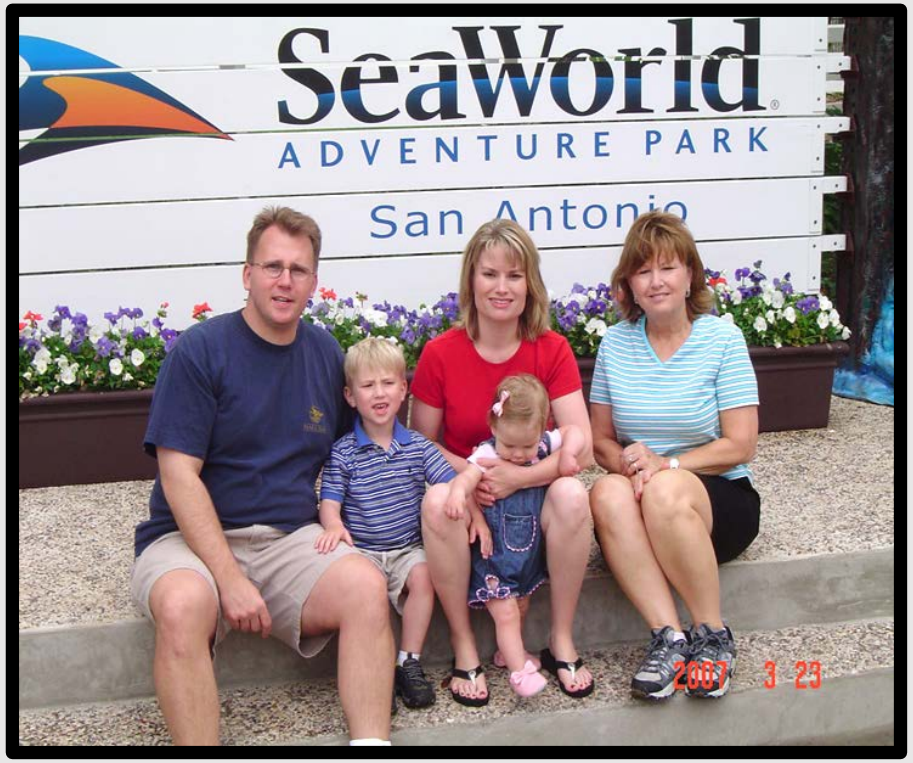
The Patient and Parent Perspective

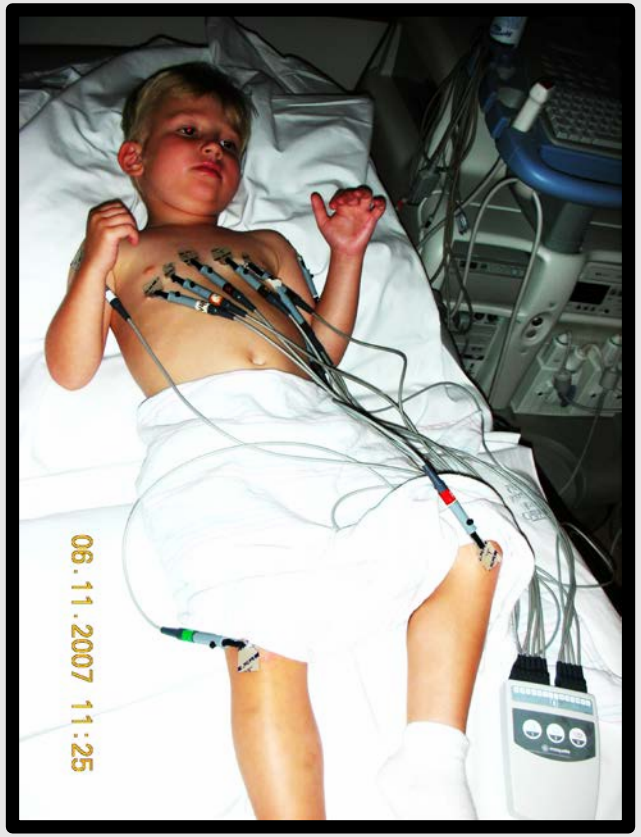
Joint Meeting of the Pediatric Advisory Committee and
the Pediatric Ethics Subcommittee

Thursday, May 18, 2017

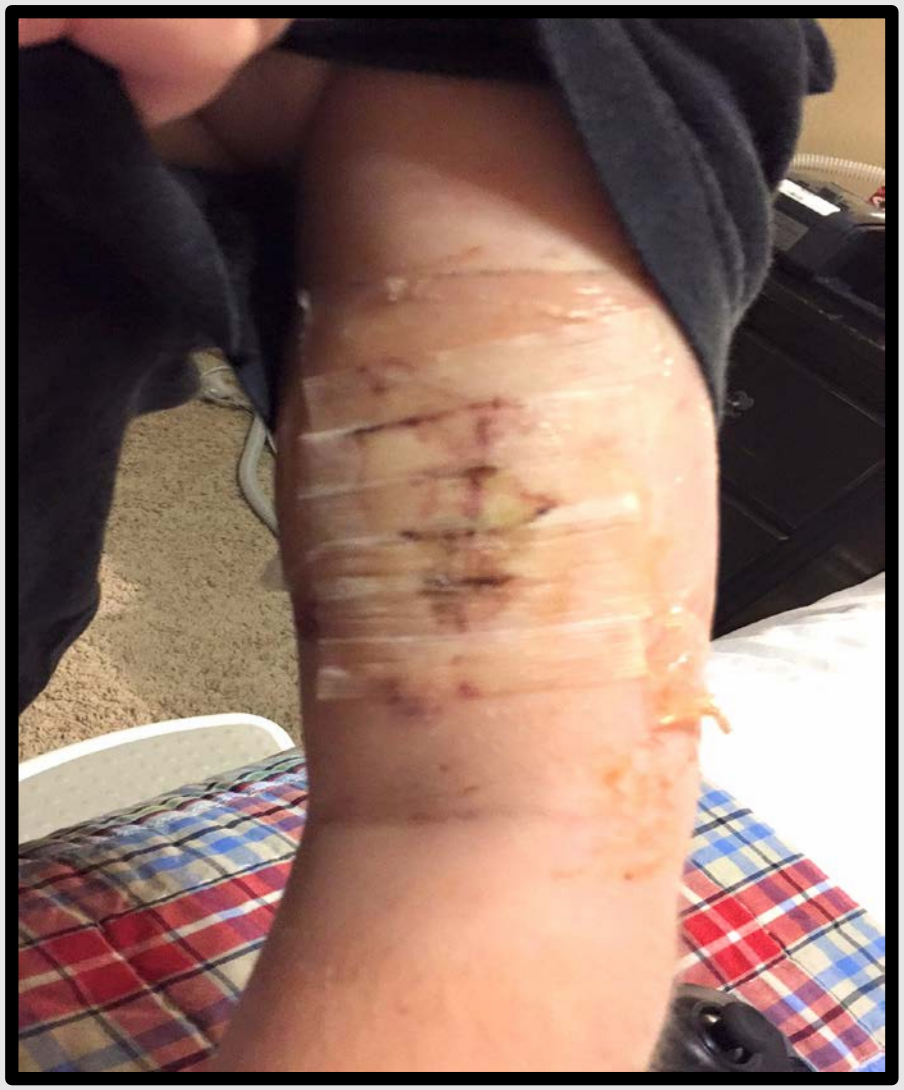
Essence Trial

Our Diagnosis Story





UCLA Trial Site

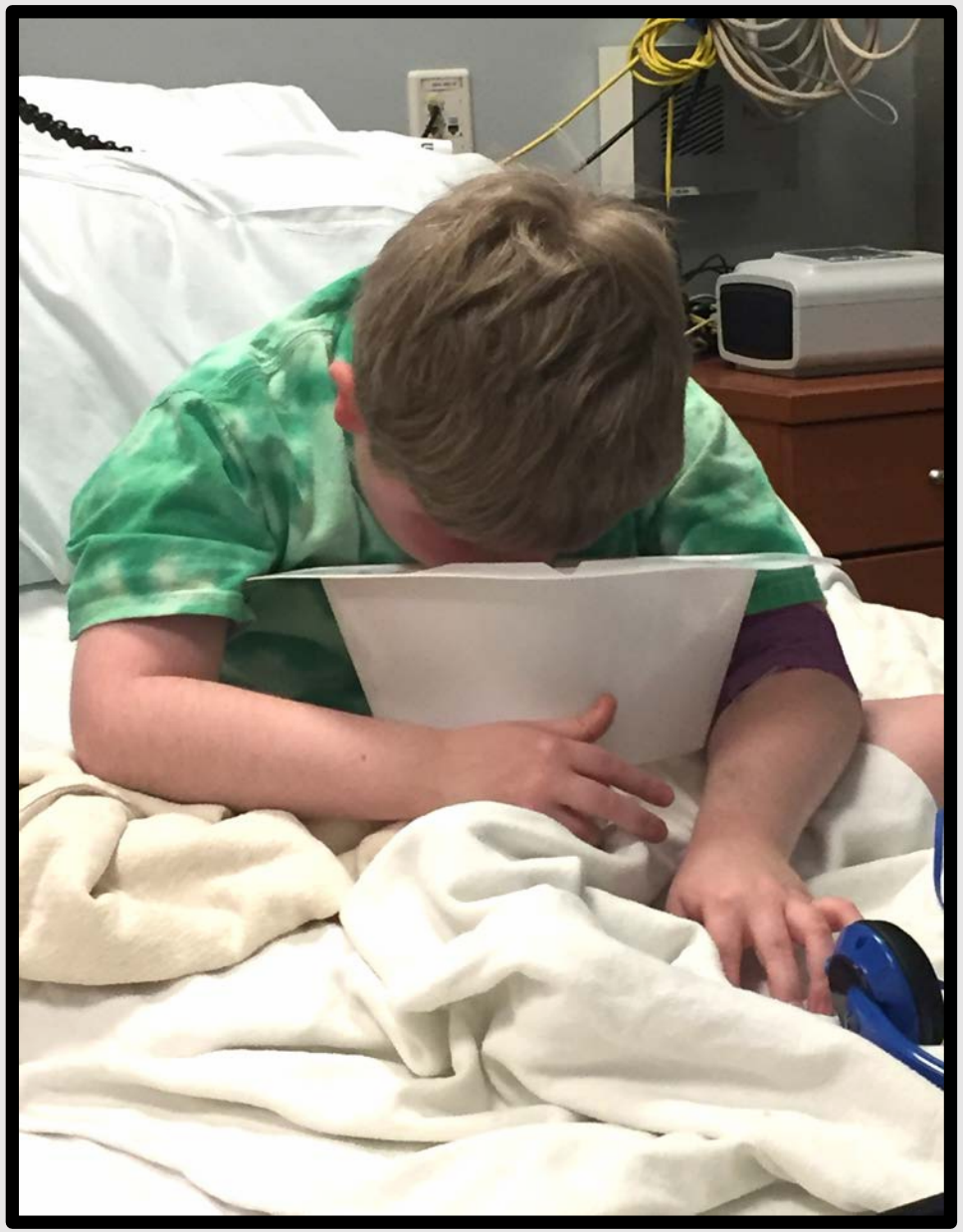


The Burden of Clinical Trials

- Extensive Travel
- Time Away From Work and School
- Placebo Arms
- Invasive Procedures

Peripheral Venous Access in DMD

- For the entire 96-weeks of the ESSENCE study, weekly infusions must be administered via IV
- Many Duchenne patients suffer from peripheral access difficulties that cause problems during clinical trials or hospital visits
- The ESSENCE protocol dictates that patients CANNOT be infused through a portacath




UCLA

Infusion Complete

Benefit-Risk of Port Placement

- Risks of port placement is clear
  - Risk of infection
  - 3rd trial related procedure performed under anesthesia
  - Recovery time

- What is at risk without a port?
  - 2 years of emotional and physical turmoil for participating patients
  - Risk of being “dropped” from the study or put on clinical hold

The Importance of Options

- Not ALL Essence patients need a port, but infusion through a port should be an option available for those who cannot tolerate weekly peripheral venous access

- The decision to infuse via port should be made only by:

    1. a Duchenne expert or PI in consultation with the family and;

    2. who appreciates the risks associated with port placement surgeries and can use their medical judgment to make the best decision for the patient and their family

Nicholas and Dawson, 2007

Nicholas and Dawson, 2016

Thank you!

- Thank you Dr. Nelson, and the Office of Pediatric Therapeutics for the opportunity to share our experience with the panels