



The Intersection of Physical Therapy and Research

Physical therapists (PTs) provide beneficial clinical care for many patients with neuromuscular disorders, and they can play an important role in advancing clinical research for neuromuscular diseases. Jenna Lammers, MSR/PT, a clinical specialist in pediatric physical therapy and research therapist at the Powell Center for Rare Disease Research and Therapy in the Department of Pediatrics in the College of Medicine at the University of Florida, fills us in on how PTs contribute to neuromuscular disease research.

PT in clinical trials

Jenna serves as a Clinical Evaluator administering functional outcome assessments in clinical trials for adults and children with neuromuscular diseases. These assessments occur at specific time points in the protocol, such as screening, baseline, and every 3-6 months for a specified number of years.

Examples of validated outcomes used in these rare disease trials are the NorthStar Ambulatory Assessment (NSAA), Performance of the Upper Limb (PUL 2.0), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-Intend), Neuromuscular Gross Motor Outcome (GRO), and Friedreich's Ataxia Rating Scale (FARA).

In her experience with clinical trials, Jenna has seen the sacrifices parents make so their children can participate. In one trial, the children were too medically fragile to travel by car or plane, so they had to be transported via medical RV with backup generators. They stayed in the RV outside the research center until the three-day visit was completed.

Many parents use PTO or take unpaid leave to bring their children to frequent research center appointments. These trips take them away from their spouse and other children at home. In addition, kids involved in trials may miss school.

The individuals and families who participate in trials often bear these burdens because of the promise of potential medical advances. "They're not just doing it for themselves but also for other people who could benefit in the future," Jenna says. "At their initial visit, they sign an informed consent form that says the trial may not help the study participant. They participate because they want things to change for the rare disease community."

Using PT to address challenges

During the pandemic, Jenna saw more patients than ever being cut off from getting the PT they needed. This led her to invent the GiggleFIT™ Therapeutic Play Gym, a portable frame structure with a kit of 31 cuffs and straps that a caregiver can set up at home to train and improve a patient's strength. Jenna's paper entitled "Therapeutic Play Gym: Feasibility of a Caregiver-Mediated Exercise System" was accepted for publication in Volume 1 2024 of the "Pediatric Physical Therapy Journal."

In her research on the therapeutic play gym (TPG), Jenna observed that physical therapy research conducted in the home environment has advantages over testing taking place in a clinical setting. "By going into the home, we tested technology-dependent children in their own environment, where they could be well rested and were not impacted by anxiety that comes with attending onsite research or clinic appointments where they know they will get a blood draw," Jenna says. "We saw firsthand the configuration of the space where parents would set up the TPG and gained insight into caregiving situations that may affect the ability of the caregiver to work with the child on an exercise regimen. Examples of this include a presence or lack of home nursing, additional siblings in the training space, or how the TPG fit in the medical crib."

Jenna also found that it's valuable to try to understand the patient's surrounding experience.

"It's so different when a family tells you they live out in the country as opposed to driving out there, taking the roads they take, and really seeing how rural it is and the access issues they face," she says. "I would encourage clinical research teams to build in opportunities to experience the participant in their own environment. If home visits are not possible, Zoom visits are another way to better understand the participants."

The changing role of PT in research

Despite the limitations, Jenna has seen a great deal of growth in the involvement of physical therapy in research for neuromuscular disorders over the years and feels optimistic about its future.

Partly, this comes from her recollections of being in physical therapy school when she wasn't aware that physical therapists could be a part of pharmaceutical trials.

"I didn't know there could be joint research between doctors, clinicians, therapists, and researchers," she says. "That knowledge is there today." She sees greater awareness and encouragement of these disciplines working together in research.

"For me, it took working in a pediatric intensive care unit and meeting a researcher involved with MDA," Jenna recalls. "I had an interest in babies with Pompe disease, and the researcher encouraged me to be a part of Pompe studies. Over the past 13 years, I have participated in over 60 trials in the neuromuscular disease space and love working on a multi-disciplinary clinical research team."

Jenna says that while she was fortunate that an MDA-affiliated researcher opened the door for her to be involved in research in 2010, those opportunities are easier to find now.

Physical therapy resources:

- Share [MDA's Recommendations for School Accommodations: Physical and Occupational Therapy](#) with families and educators.
- Patients can use [MDA's disease-specific At-Home Physical Therapy Exercises guides](#). Guides for DMD, Pompe, and SMA are available now, with more to come.