

**Scott Schliebner**

**Abstract Title**

Patient-focused and virtual trial models to reduce the burden of clinical trial participation for patients and families suffering from CNS diseases.

**Methodological questions being addressed:**

How can clinical trial designs evolve to be more patient-focused? What methodological approaches can be utilized to ensure that future clinical trials are designed and conducted around patients' lives? Can patient-focused and virtual trials democratize clinical research and provide access to clinical trials for any patient that wants to participate?

**Introduction (aims)**

Clinical drug development is an incredibly difficult and challenging endeavor in most disease states. Developing new medicines and therapies for patients with complex CNS diseases poses an additional set of logistical hurdles and complexities to overcome. These patients are often not located near major academic medical centers and rely on parents and/or caregivers for support. Participating in a clinical trial can require traveling great distances, sometimes to other countries, for extended periods of time. In addition to time away from work or school, there can be additional financial considerations, intense time commitments, and uncertainty and anxiety. This is not a realistic drug development paradigm, and ultimately, this approach is unsustainable for the future development of new treatments for CNS diseases.

The challenges and complexities noted above require a new approach to CNS drug development where clinical trials are adapted to the daily life and experience of the CNS patients we serve. By viewing and treating patients as partners, and ultimately end-consumers, we thereby prioritize the patient's perspective, interests, and motivations. Our colleagues in the technology sector, for example, have embraced a customer-focused approach with great success, resulting in countless new products and solutions that are aligned with what consumers want. Unfortunately, clinical trials have historically targeted and recruited patients, but have not wholeheartedly engaged with patients as partners. A patient-focused, forward-thinking paradigm that centers on the patient experience will disrupt the current traditional clinical trial approach and result in clinical trials that are more appealing and feasible for all CNS patients.

**Methods**

Assessment of patient attitudes, concerns, and barriers to participating in clinical trials were assessed in 482 individual patients and caregivers who have recently participated in clinical research. Further assessment on the patient experience in clinical research will examine the use of traditional clinical trial paradigms as well as exposure to evolving virtual paradigms to clinical research.

**Results**

Findings from our patient assessment project helped to support and confirm prior anecdotal findings and also provided feedback directly from patients and caregivers related to their experience with clinical research. Of the 482 respondents, 463 were female (96%), the far majority (93%) were over the age of

40, and only 47 (10%) had the opportunity to participate in a clinical trial. Of those patients that had an FA-approved therapy for their disease (82%), most patients were currently receiving this therapy (75%). The most common challenges cited as barriers to participating in clinical trials were travel to clinical sites, financial burden, and overall inconvenience.

### **Conclusions**

We examined the most prevalent barriers to participating in clinical research, as reported by our patient and caregiver outreach. Patients shared the barriers that they found most significant in their clinical trial experience as well as those barriers that prevented clinical trial participation altogether. From our collective experience, as well as the summary of patient findings, it is clear that clinical trials require an incredible amount of time, travel, financial, and emotional burden for families and caregivers. Solutions to overcoming these challenges and changing the clinical trial paradigm are discussed.

### **Disclosures**

None.