Science 37®

# **Case Study:** Fragile X Syndrome

**Phase 3 | Rare Disease, Neurology**

## Challenge

Families of Fragile X patients often find themselves overwhelmed with caregiving responsibilities. Additionally, Fragile X patients are sensitive to overstimulation, so they often avoid social situations and struggle to leave home, making clinical visit travel especially burdensome. The complex protocol requiring frequent specimen collection and caregiver-administered dosing was especially taxing for this pediatric population, increasing irritability and overstimulation, thus risking high attrition rates at traditional sites.

## Solution

Science 37's Direct-to-Patient Site enabled in-home trial participation tailored specifically to Fragile X patient needs. Pediatric-trained, research-grade mobile nurses ensured patient comfort and protocol adherence in the home, reducing stress for patients and caregivers, while also improving engagement and data quality. The sponsor met its goals and aligned with families' strong preference for in-home care, proving especially effective for this rare, debilitating condition.

## Lessons Learned

Collaboration with advocacy groups built trust and raised awareness, directly supporting patient inclusion. Provider networks served as key referral sources for this rare patient population. For pediatric patients with rare, debilitating conditions, bringing the study to the patient significantly improves recruitment, retention, and the overall experience—demonstrating the unique value of a direct-to-patient model.

## Science 37 Results

**Enrolled**
**25%**
**of Total Study Participants**

**98%**
**Retention**
in a difficult rare disease population

**Top Enrolling Site**
**in the Trial**

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FDA INSPECTED

**The First & Only FDA-Inspected Direct-to-Patient Site**