

Case Study: Rare Genetic Neurological Disorder Causing Severe Impairments in Pediatric & Young Adult Females

Rare Disease Program Demographics:

Precision is running multiple studies for Sponsor

- Ages 5–20-year-old trials
 - Phase III, pivotal
 - Open-label extension
 - Long-term exposure study, including compassionate use until drug approval
- Ages 2–5-year-old trials
 - Open label (Phase II/III)

Services provided by Precision for Medicine:

- Project Management
- Site Start-up and Management
- Clinical Monitoring
- Medical Monitoring

Challenges Encountered & Overcome:

Flexibility, creativity, and decisive action taken to solve unforeseen problems and evolving challenges:

- COVID-19's impact forced a conversion to remote and decentralized strategies, including
 - Home nursing visits and safety assessments
 - Direct IP shipment and drug accountability
 - Remote assessments & in-home visits
 - Deployment of a remote monitoring/document-sharing platform
 - Modification of diaries and completion guidelines

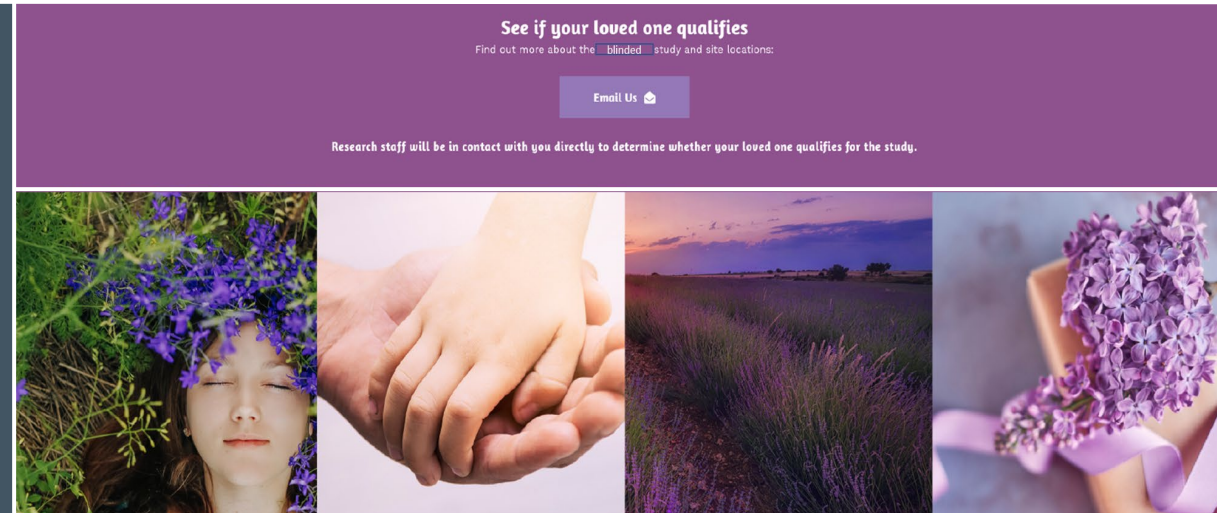
Pivotal Phase III Statistics:

- 21 sites, all USA
- 220+ recruited
- 180+ enrolled



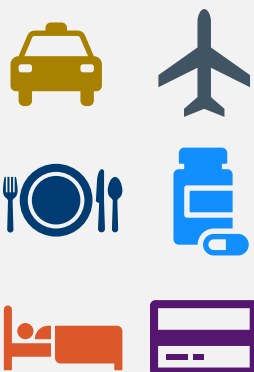
Patient Recruitment Tools

- Study website brought in >100 interested patients and required unblinded CRO staff for triage and site referrals
- Collaboration with patient advocacy group for awareness
- Identification of the right investigators and pediatric neurologists and leveraging of their patient databases



“We as a team are so invested in this drug and in these girls. I personally push to solve issues with [the sponsor] to keep these patients. I’ve never met any of them, but I’m their biggest advocate.” – Study Clinical Trial Manager

Patient & Caregiver Concierge Services:



- Full support for booking & reimbursement: *e.g., car, plane, shuttles, lodging, and food*
- These services were vital for supporting and retaining the young patients fighting a difficult neurological disease and the stressed parents and caregivers trying to support the study while taking care of their children

Thank you to the sponsor for their thoughtful support in extending these critically important services!

