RARE DISEASE

First-of-its-kind Platform Trial for ALS

Eases Participation for Patients with Challenging Disease Progression

TRIAL DETAILS

TYPE:
Platform Trial

DURATION:
24-Week Double-Blind
Optional Indefinite Open-Label Extension

CONTRACTED SITES:
54

CONTRACTED PATIENTS:
80

LOCATIONS:
US

Amyotrophic lateral sclerosis (ALS), or Lou Gehrig's disease, presents a particular challenge for study teams. Disease progression weakens muscles and significantly impacts participants' mobility, making site visits nearly impossible in some cases. With a mission to find an effective treatment for people living with ALS as quickly as possible, a nationwide network of research centers and a leading Center for ALS partnered to conduct a platform trial.

The platform trial was designed to test multiple investigational products simultaneously to accelerate the path to new therapies. **Firma was engaged to complete in-home visits for the 24-week double-blind portion of the study, additional variable visits for up to a year, and then an optional open-label extension that would continue indefinitely** as long as patients were seeing benefit and were willing and able to participate.

STUDY CHALLENGES

Firma was contracted for 80 participants to the master protocol, which would then be randomized across four different treatment regimens (20 per regimen) for a total of 320 visits within the 24-week double-blind portion. Visits for all regimens consisted of:

- Review of changes in health and medication
- Vital signs (including weight, blood pressure, respiratory rate, temperature and heart rate)
- Blood collection (chemistry and hematology assessments) including processing of blood samples in the home
- Urine collection
- Receipt of Investigational Product (IP) and assessment of IP condition (when IP was shipped directly to participant's homes)

While the study included a fairly straightforward protocol, the **disease progression, semi-sporadic schedule of assessments, widely distributed nature of the patient pool, and an ongoing nursing shortage due to the COVID pandemic all created unique challenges.**

#1. Disease Progression

As a progressive neurodegenerative disease, ALS itself was a significant study challenge even for in-home study visits. Tremors made blood draws more difficult to complete, and patients with tracheostomy tubes for impaired respiratory function or those who were bed-bound made capturing weight challenging. Because some participants suffered from limited mobility due to their worsening disease, traveling to the site for visits became increasingly difficult and these participants were very grateful to be seen in the comfort of their home.

#2. Distributed Patient Pool

There were a total of 80 participants enrolled in the study but the patient pool was highly distributed across the nation. There were also a number of individuals in rural areas. These two factors all but eliminated the option of utilizing one in-home healthcare provider for more than one patient, and also made the process of finding the highly skilled providers especially challenging.

#3. Sporadic Visits

After an initial site visit, Firma would begin in-home visits at Week 4, followed by Week 8, Week 16, Week 24, Week 28, and Week 40. Additional unscheduled visits could be completed starting around Week 16 - 24 depending on how each patient was progressing through the disease and responding to the treatment protocol. While the scheduled visits every month were straightforward, sourcing and training a reliable team of highly skilled in-home nurses for the unscheduled, variable visits and an indefinite number of visits for the open-label extension was not an easy ask.

#4. COVID Nursing Shortages

On top of these already challenging requirements, the pool of home healthcare providers was significantly diminished due to the lingering impacts of the COVID-19 pandemic. A number of pandemic-related trends were contributing to the low number of available providers: Many nurses had to quarantine after falling ill or being exposed to the latest variant. Others were moving away from home health and to hospitals or other brick-and-mortar healthcare settings due to the overwhelming need for bedside nurses throughout the pandemic. Some were leaving healthcare entirely due to burnout or shifting desires as part of the nationwide workforce shift.

RESULTS

In short, the study required skilled in-home nurses with adequate protocol training to ensure sample collection was efficient and accurate—and that patients and their caretakers felt as comfortable as possible with the entire process. Additionally, those in-home providers needed to be thoroughly vetted and willing to agree to an unknown schedule to suit participant needs—and Firma needed to be ready to provide trained replacement providers should any of the original group drop out.

Firma's home trial services team was able to not only meet all of the aforementioned challenges but exceeded the expectations of the collaborative study team and participating patients. Firma's agile and effective sourcing and vetting process ensured that in-home visits were attended by highly skilled nurses with the right rapport. Each provider received individualized and in-depth protocol-specific training and ongoing support from a Firma study coordinator to answer questions, troubleshoot, or provide guidance.