ON THE FOREFRONT OF BIOMEDICINE

# How WCG Statistics Collaborative Helped Breakthrough Therapy Obtain Regulatory Approval

WCG Statistics Collaborative, Inc., provides guidance and statistical services to sponsors—including biotech firms with little or no in-house statistical capabilities. WCG Statistics Collaborative was involved in one of the most remarkable developments of this century: A first-of-its-kind therapy for a genetic condition.

## BACKGROUND

The WCG Statistics Collaborative team became involved in the development process early. WCG Statistics Collaborative helped design the study evaluating the therapy, prepared the final analysis and represented the sponsor in discussions with the FDA.

The researchers completed a Phase 1/2 trial and needed biostatistical expertise as they moved into Phase 3. Like most small biotech companies, they didn't have a large statistical department. Thus, they turned to WCG Statistics Collaborative.

## THE CHALLENGES

### A Small Population, Small Studies

The indication being treated is an exceptionally rare disease: There were only 12 patients in the Phase 1/2 study.

### B Unique Metrics

Researchers faced several challenges, including the following:

- In this rare disease population, typical metrics assessing functional outcomes were not directly applicable.
- The primary assessment involved a carefully designed functional course that assessed the ability to navigate obstacles related to the disease state. **But how do you translate such findings into a measurable outcome for use in a clinical trial?**

# THE SOLUTION

WCG Statistics Collaborative came in before the Phase 3 trial, consulted with team members and investigators, analyzed data from the Phase 1/2 participants, and helped create a novel functional primary endpoint for Phase 3.

## HELPING WITH TRIAL DESIGN

WCG Statistics Collaborative provided insights on Phase 3 protocol design and developed a statistical analysis plan. Additionally, WCG Statistics Collaborative worked with the FDA as needed, incorporating agency feedback. It was also involved with the biopharma's interactions with the FDA at the advisory committee panel.

## STANDARDIZING RESEARCHER METRICS

Even though the researchers had developed a method for assessing participants, the FDA required a functional measure of how the therapy would impact patients. WCG Statistics Collaborative developed a scale to quantify the results corresponding to successful mobility.

## Lessons Learned

**A**

### Size doesn't have to matter:

When a disease is very rare and a treatment highly effective, a small sample size doesn't have to be an impediment to winning approval. It's challenging, but with careful planning, design and statistical considerations, it can be done well.

**B**

### The earlier, the better:

It is important to involve statisticians as early as possible, including in protocol design.

## Outcomes

The FDA approved the first-of-its-kind biomedicine for a genetic condition and WCG Statistics Collaborative participated in the presentation of the trial's receipt of the prestigious David Sackett Trial of the Year Award.