TRUE CNS OUTCOMES

Expert Analysis of Rater Discrepancies Allows Sponsor to Demonstrate Efficacy—Even with a Missing Endpoint

THE CHALLENGE

A small neuroscience pharmaceutical company conducted a 28-day Phase II proof-of-concept clinical trial in 95 patients with a movement disorder.

But there was a problem: The primary study endpoint using remote video assessment failed to meet significance at Day 28. However, using the same scale, investigators who observed the patients in person did find significant improvement in treated patients at Day 28 when compared to placebo. They used the same scale.

The company turned to WCG MedAvante-ProPhase for guidance and expertise, asking us to re-analyze the primary endpoint data from all sources.

THE RESULTS

A

WCG verified that the site-rated data was accurate; this gave the sponsor confidence to proceed with development of the compound, which will enter a Phase IIb study this year (2021).

B

WCG's independent analysis of the video rating process uncovered unexpected complications. After addressing these issues, the sponsor was able to show compelling evidence of efficacy, despite missing the primary endpoint. The drug worked; the problem was they didn't have the right protection to defend their primary endpoint.

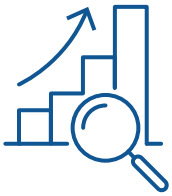
C

Because of WCG MedAvante-ProPhase's analysis, the sponsor is now working closely with the scientific community to enhance central video rating for use in interventional studies.

OUR SOLUTION

WCG SERVICES FOR CNS:

WCG MedAvante-ProPhase conducted post-hoc analysis and comparison of the assessment data from the site raters and the central reviewers to:



Identify data-quality issues and/or discrepancies.

Shed light on implementation issues that may have contributed to the discrepancies.

The analysis uncovered unexpected complications in implementation and use of the assessment tool. Specifically, it identified a clear and significant shift in distribution between site raters and central reviewers:

The expert scorers were neither carefully calibrated, nor subject to any data analytics during the study.