transparency life sciences

The Challenge

A small drug development company was exploring an orphan disease with no approved therapies as a new indication for an investigational drug it owns. This new chemical entity (NCE) had been tested in a Phase III trial for a different indication but was not approved for marketing because of insufficient efficacy data. The sponsor's key concern for the new indication was achieving adequate patient enrollment. They knew that orphan indication trials often have difficulty reaching their enrollment targets, resulting in timeline and budget overruns. The novelty of the indication and need to recruit scarce patients made this project a prime candidate for protocol design crowdsourcing and digital execution. The Principal Investigator (PI) and company sponsor's team had assembled a synopsis for the proposed clinical trial for the new indication, but it had many unanswered questions regarding clinical endpoints, patient monitoring, and other program features.

TLS Solution

Our first step was to transform the study synopsis document into a crowdsourcing survey. In conjunction with the PI and a foundation focused on the rare disease being addressed, the TLS team created two surveys that would be presented to a "crowd" of patients and researchers. These surveys asked respondents to review and assess the assumptions in the current study plan, and to react to many open-ended questions, allowing participants to offer new ideas that would enhance the approach. We received responses from approximately 250 patients and 50 researchers and clinicians who study or treat this disease.

Once the crowdsourcing input was received, TLS's next task was to interpret the data and provide detailed suggestions to the PI and medical writing team. To achieve this, TLS conducted a statistical analysis of the quantitative and qualitative data and assembled an advisory team of standout researcher and physician participants in the process to review the analysis. The team used the crowdsourcing-generated analysis to propose modifications to the proposed therapeutic approach, endpoints and telemonitoring program. For example, the study's steroid taper period was refined based on patient response to a proposed dosing schedule, and the parameters of the endpoint were narrowed after many researchers expressed concerns. The PI and sponsor's team agreed with these suggestions, and the changes were incorporated into the protocol.

The Benefit

To encourage study enrollment and manage costs, TLS aimed to reduce the need for patient site visits in the trial. We presented plans to the study team and the FDA that primarily relied on home and community-based measurements and monitoring. After receiving positive preliminary input from the FDA, the study team concluded that 80% of study patients can be enrolled and monitored without visiting a clinical study site. Recently, the FDA cleared the study protocol to proceed, granting what we believe to be

the first IND approval for a fully digital study administered from patients' homes and offices. This study is expected to begin enrollment shortly, using the crowdsourced protocol and TLS's virtual trials platform for data collection. By making the changes in scope and methodology, TLS aided the sponsor in optimizing a protocol to reflect the best thinking from the research community and the needs of those diagnosed with this disease.