

MJFF Practices Proactive Quality Design to Effectively Deliver Input that Speeds Parkinson's Progress

MJFF Applies CTTI's Quality by Design Recommendations

SUMMARY

The Michael J. Fox Foundation (MJFF) wanted to use its extensive clinical trials knowledge to address or remove common challenges Parkinson's disease trials face early on in the design stage of studies, with the hope that their input could ultimately speed new insights and therapies to patients. Here is how the Foundation applied CTTI's Quality by Design (QbD) recommendations to accomplish this goal.

GOAL(S)

MJFF knows Parkinson's disease. Once lauded in the *New York Times* as "the most credible voice on Parkinson's research in the world," the Foundation is the world's largest non-profit funder of Parkinson's drug development, and it has galvanized the search for a cure. In 2018, MJFF began using its extensive clinical trials expertise, which includes former study coordinators, in house neuroscientists, and other individuals who work closely with the patient community, to consult with sponsors on trial designs for Parkinson's research in an effort to pre-emptively identify and address the challenges these studies frequently face.

CHALLENGES

As MJFF began building up its protocol consultation service, it sought a more systemic way to package advice and recommendations to sponsors. The goal was to make it easier for sponsors to review and understand the feedback the Foundation provided, as well as what areas of the protocol MJFF was reviewing and what rubric it was using to inform its review.

SOLUTION(S)

Fortunately, one colleague at MJFF is also a member of CTTI's steering committee, and she had become aware of CTTI's work on QbD. The idea of designing a clinical trial in the most efficient, effective, patient-centric manner as possible to increase a study success aligned perfectly with what the Foundation was trying to achieve, so [CTTI's QbD recommendations](#) became the Foundation's rubric to evaluate Parkinson's disease protocols.

TAKING ACTION

MJFF advises sponsors to provide Parkinson's study protocols as early as possible in the design phase. Once the protocol is in hand, the Foundation gathers its team's expertise and introduces a QbD evaluation that outlines specific sections, including feasibility, recruitment, retention, and return of results. Individually, the expert contributors on the team review the protocol, assessing it through the lens of each section listed in the QbD metric. After individual review, the group comes together and discusses thoughts related to each section. Many times, the team identifies areas the sponsor has overlooked, potential challenges inherent to the protocol to flag, or opportunities to highlight that may help facilitate research or ease the burden on the study coordinator. These comments are listed under each outlined section for ease of review and sent to sponsors via e-mail, along with the QbD rubric to help sponsors understand the rationale. A broad variety of expertise to holistically assess protocols is a cornerstone of the QbD approach CTTI recommends and fundamental to MJFF's evaluation. For each protocol, expertise from clinical operations, academia, recruitment and retention, and the patient perspective is applied to deliver a 360-degree evaluation that looks at the protocol from multiple perspectives -- true study feasibility, after all, requires that all of these voices be taken into account.

IMPACT

Sponsors have responded positively to MJFF's protocol consultation offering. In follow-up surveys deployed by the Foundation, each sponsor was asked how likely they are to request another QbD review from the Foundation, and 100 percent of respondents said they are likely to request similar consult for another Parkinson's study. The consultations have also made a meaningful difference in study protocols to date. For example, in one protocol with a genetic testing component, the sponsor had listed different vendors that the participant could use for test results to show they were study-eligible. One piece of feedback the Foundation had in looking at the list of vendors was that one of the more common vendors was not on that list. So, the Foundation flagged it with questions: If the patient were to bring a result from this common consumer genetic testing company, how would the sponsor react? Would they accept those results? If not, how many individuals might this exclude? This helped the sponsor to think ahead, and they eventually agreed to add the additional vendor. As a result, burden on the patient was reduced for the study and the sponsor avoided a potential protocol amendment (which is both time-consuming and costly) later down the road if enrollment was low due to the omission.

ADVICE

The cost and time of amendments is huge, so the ability to have early feedback on how a study will be executed is impactful. Using CTTI's QbD recommendations, the Foundation is able to offer easy-to-digest upfront expertise that translates to a significant value for sponsors. At the same time, the process furthers MJFF's mission of not only funding research and educating the patient community, but also helping research teams who are conducting clinical trials execute efficiently to help advance treatments to patients in need.

ORGANIZATION

The Michael J Fox Foundation for Parkinson's Research

CONTACT

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ORGANIZATION TYPE

Patient

IMPLEMENTATION DATE

2018

TOPIC

Quality

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