FRIENDS of CANCER RESEARCH

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Annual Meeting

Incorporating Pragmatic Elements in Study Designs to Enhance Oncology Randomized Clinical Trials

EXECUTIVE SUMMARY

Given calls for trial modernization, the FDA Oncology Center of Excellence (OCE) launched Project Pragmatica to seek opportunities to introduce functional efficiencies and enhance patient centricity by integrating aspects of clinical trials with real-world routine clinical practice through appropriate use of pragmatic elements. Introduction of pragmatic approaches into clinical trials, where appropriate, can streamline trial conduct and data collection. Trials incorporating pragmatic elements may reduce the burden on patients and providers and facilitate enrollment of more diverse trial populations, ultimately enabling an improved understanding of medical product effectiveness and safety in a population more representative of those affected by the disease. The integration of pragmatic elements into clinical trials will vary depending on the regulatory and clinical context of the trial, and a framework for when and how it may be appropriate to incorporate pragmatic elements into clinical trial designs is needed. Friends of Cancer Research (*Friends*) convened a multi-stakeholder group of experts including members from the FDA and National Cancer Institute (NCI), drug developers, patient advocates, and academicians representing the NCI National Clinical Trial Network (NCTN) to layout considerations for determining the appropriateness of incorporating pragmatic elements into randomized clinical trials and to outline potential innovative trial designs that may support a shift to streamlining data collection and study design.

As efficacy and safety evidence accumulate through the lifecycle of a drug, this expanded knowledge base may allow for added flexibility to modernize evidence generation through novel methods of data collection and data sources, including introduction of pragmatic elements. The specific scientific question, intent (e.g., inform regulatory decision or practice guidelines), indication, and drug, as well as the composite evidence previously generated from clinical trials and observational studies, are factors to evaluate when assessing which elements are appropriate for a pragmatic clinical trial design. For example, a trial may incorporate broader eligibility criteria, select patient centric outcome measures, or streamline safety data collection to enhance trial efficiency and generalizability of results. Considerations for incorporating these pragmatic elements are outlined in the white paper. To encourage the inclusion of pragmatic elements in randomized clinical trials, select case studies highlight pragmatic elements amenable to each scenario. Trials with pragmatic elements require custom approaches, and thoughtful consideration, as well as early discussion with regulatory authorities, should be taken when developing a clinical trial including specific pragmatic elements.

Click or access the full white paper with the QR code here:

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Thank you to our working group collaborators for informing the development of this white paper.

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