

Regulatory, Financial and Access Considerations

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METHODS FOR GATHERING PATIENT-RELEVANT CONCEPTS: THE USE OF THE PATIENT QUALITATIVE ASSESSMENT OF TREATMENT (PQATV2) IN EARLY ONCOLOGY DEVELOPMENT

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Background The importance in measurement and analysis of patient-reported outcomes (PROs) in early oncology development as toxicity and efficacy endpoints— particularly with the emergence of targeted therapies and immunotherapies, has been recognized among researchers.¹⁻⁶ Recently, more attention has been directed towards considerations for applying PROs to early oncology development by the FDA in public workshops.^{7,8} Despite their recognized value, methods for collecting, analyzing, and interpreting early phase PRO data are complex, leading to infrequent use in early oncology trials. The aim of this abstract is to conduct a scoping review of PRO usage in early oncology trials and highlight the novelty of a Sanofi-developed PRO measure, Patient Qualitative Assessment of Treatment version 2 (PQATv2).

Methods To validate our approach to PQATv2 implementation, we sought to identify early phase industry-sponsored oncology trials reporting PRO endpoints registered with ClinicalTrials.gov between January 1, 2009, to July 20, 2022—a start date to coincide with the publication of the 2009 FDA PRO Guidance for Industry. The PQATv2 is a 6-item, generic PRO measure. The PQATv2 includes: two 11-point numeric rating scales assessing participant-perceived treatment benefits and disadvantages (0 = not at all beneficial/disadvantageous to 10 = extremely beneficial/disadvantageous); an item assessing participants' willingness to continue treatment after the trial (yes/no); a 7-point Likert-type item assessing participants' overall benefit/risk ratio perceptions (-3 = 'disadvantages of the drug I received significantly outweighs the benefits' to 3 = 'the benefits of the drug I received significantly outweigh the disadvantages'); and three free-text items assessing participant-perceived treatment benefits, disadvantages, and reasons why the participant would be willing or unwilling to continue the treatment after the trial.

Results Of the 78 early phase studies reporting PRO endpoints and registered with ClinicalTrials.gov, Phase 1 oncology trials accounted for 33.3% (n=26) and Phase 1|2 at 66.7% (n=52). The PQATv2 is being included in several of our early oncology studies. Recognizing the need to enrich traditional efficacy and safety endpoints, Sanofi believes the PQATv2 is a novel and important approach to identifying salient and important concepts that guide the identification of future PRO measures in later phases.

Conclusions The PQATv2 is a novel exploratory measure aimed at generating additional hypotheses to guide selection of fit-for-purpose PROs in later phases. Further analysis of the utility of PROs in early phase oncology, and their impact in guiding preparation of PROs for later stage studies will be conducted as we gain more experience.

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