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Clinical Trials in Oncology and Defining Benefit

In the editorial "The Evolution of Clinical Trials in Oncology: Defining Who Benefits from New Drugs Using Innovative Study Designs" [1], Goldberg et al. nicely discuss many changing aspects of clinical trial design in oncology. However, I believe their title is misleading. No study will define "who benefits" but only who *might* benefit. For example, human epidermal growth receptor 2 overexpression or gene amplification in breast cancers epitomizes a remarkable example of a predictive marker validated to show benefit. Still, adjuvant trastuzumab benefits less than half the patients who receive it compared with chemotherapy alone.

Also, the authors write "there must be extensive deliberation among clinicians, biostatisticians, and regulatory experts . . . and they must reach consensus on setting appropriate efficacy and futility boundaries." However, one basic tenet of personalized medicine is that efficacy or futility should largely be defined by the patient. What might be considered efficacy to one patient might be considered futility to another. For example, response duration might be of great importance to a patient, even if there is no survival benefit or progression-free survival benefit seen for the average patient.

As William Osler wrote, "It is more important to understand what sort of a person has a disease than what sort of disease a person has." The promise of precision oncology rests not only with improved markers of efficacy used in well-designed and innovative trials but also in understanding the values and nature of the particular patient and how that patient defines efficacy or futility.

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Disclosures

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Reference

1. Goldberg RM, Wei L, Fernandez S. The evolution of clinical trials in oncology: Defining who benefits from new drugs using innovative study designs. ***The Oncologist*** 2017;22:1015–1019.

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