Surrogate Markers and Endpoints

Frank W. Rockhold, PhD
Professor of Biostatistics and Bioinformatics
Duke University Medical Center

The Food and Drug Administration's Accelerated Approval Process for New Pharmaceuticals A Virtual Workshop
January 30-31, 2023

Duke Clinical Research Institute | FROM THOUGHT LEADERSHIP TO CLINICAL PRACTICE

The challenge of using novel biomarkers as "reasonably likely" surrogate endpoints

"Reasonably likely surrogate endpoints are supported by strong mechanistic and/or epidemiologic rationale, but the amount of clinical data available is not sufficient to show that they are a validated surrogate endpoint ."

In fact, for a biomarker to qualify as a validated surrogate endpoint, the biomarker must, at a minimum, not only be correlated with the outcome, but the change in the in the biomarker must "explain" the change in the clinical outcome.

https://www.fda.gov/drugs/development-resources/surrogate-endpoint-resources-drug-and-biologic-development

Expert Panelists

David L. DeMets, University of Wisconsin, Madison
Peter Stein, Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration
Scott S. Emerson, University of Washington
Steve Pearson, Institute for Clinical and Economic Review (ICER)