MEDICAL UNIVERSITY OF VIENNA

Wiener Biometrische Sektion
der Internationalen Biometrischen Gesellschaft
Region Österreich – Schweiz

Please join the Biometric Colloquium

SCOTT EVANS, PHD, MS

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Founding Chair and Professor, Department of Biostatistics and Bioinformatics

President, Society for Clinical Trials

Milken Institute School of Public Health

George Washington University

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A PATIENT-CENTRIC PARADIGM FOR CLINICAL RESEARCH: THE DOOR IS OPEN

April 10th, 2025 at 9:30-12:00

Informatikbibliothek, Center for Medical Data Science,
Spitalgasse 23, Room 88.03.806

Medical University of Vienna, 1090 Wien

Host: Franz König

Agenda:

9.30: You are kindly welcome to a joint coffee

10-12: Presentation

Abstract:

Randomized clinical trials (RCTs) are the gold standard for evaluating the benefits and harms of interventions. However commonly used design and analysis approaches to RCTs often are not suited to answer the most important research questions to inform medical decision-making. Typically efficacy and safety are evaluated in silos, one outcome at a time. However this approach: fails to incorporate associations between or the cumulative nature of multiple outcomes in individual patients, suffers from competing risk complexities during interpretation of individual outcomes, fails to recognize important gradations of patient responses, suboptimally evaluates treatment effect heterogeneity based on a single endpoint rather than benefit:risk considerations, and since efficacy and safety analyses are often conducted on different populations, generalizability is unclear.

The Council for International Organizations of Medical Sciences (CIOMS) will soon be releasing "Benefit-risk balance for medicinal products". Included within the report are two new points of emphasis: (1) transitioning benefit-risk evaluation as a post-hoc exercise to incorporating benefit-risk considerations into clinical trial design, and (2) a pragmatic patient-centric approach to benefit-risk assessment reflecting how benefits and harms are experienced by patients to better align with the goals of informing clinical practice.

The desirability of outcome ranking (DOOR) is a paradigm for the design, analysis, and interpretation of clinical trials and other research studies based on patient-centric benefit-risk evaluation, developed to address these issues and advance clinical trial science. In this paradigm outcomes are used to analyze patients rather than patients being used to analyze outcomes. The experiences of trial participants in different treatment arms are compared by the desirability of the overall patient outcome, increasing pragmatism and addressing the most important "real world" question to aid clinical decision-making: how do resulting patient experiences, when comprehensively considering benefits and harms, compare between therapeutic alternatives? We describe the DOOR paradigm; define guiding principles to maximize replicability, robustness, objectivity, transparency, and pragmatism; outline a recommended statistical analysis plan; illustrate application with examples; and demo a freely-available online application for implementing the recommended DOOR analyses and the design of studies implementing DOOR.