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| Nota di contenuto | Preface -- Part I. Real-World Data and Evidence to Accelerate Medical Product Development -- The need for real world data/evidence in clinical development and life cycle management, and future directions -- Overview of current RWE/RWD landscape -- Key considerations in forming research questions -- Part II. Fit-for-use RWD Assessment and Data Standards -- Assessment of fit-for-use real-world data sources and applications -- Key variables ascertainment and validation in real-world setting -- Data standards and platform interoperability -- Privacy-preserving data linkage for real-world datasets -- Part III. Causal Inference Framework and Methodologies in RWE Research -- Causal Inference with Targeted Learning for Producing and Evaluating Real-World Evidence -- Framework and Examples of Estimands in Real-World Studies -- Clinical Studies Leveraging Real-World Data Using Propensity Score-Based Methods -- Recent statistical development for comparative effectiveness research beyond propensity-score methods |

-- Innovative Hybrid Designs and Analytical Approaches leveraging Real-Word Data and Clinical Trial data -- Statistical challenges for causal inference using time-to-event real-world data -- Sensitivity Analyses for Unmeasured Confounding: This is the way -- Sensitivity analysis in the analysis of real-world data -- Personalized medicine with advanced analytics -- Use of Real-World Evidence in Health Technology Assessment Submissions -- Part IV. Application and Case studies -- Examples of applying causal-inference roadmap to real-world studies -- Applications using real-world evidence to accelerate medical product development -- The use of real-world data to support the assessment of the benefit and risk of a medicine to treat spinal muscular atrophy -- Index.

Sommario/riassunto

This book provides state-of-art statistical methodologies, practical considerations from regulators and sponsors, logistics, and real use cases for practitioners for the uptake of RWE/D. Randomized clinical trials have been the gold standard for the evaluation of efficacy and safety of medical products. However, the cost, duration, practicality, and limited generalizability have incentivized many to look for alternative ways to optimize drug development. This book provides a comprehensive list of topics together to include all aspects with the uptake of RWE/D, including, but not limited to, applications in regulatory and non-regulatory settings, causal inference methodologies, organization and infrastructure considerations, logistic challenges, and practical use cases.