1.Patient. 2025 Jul;18(4):303-315. doi: 10.1007/s40271-024-00695-6. Epub 2024 Apr 25.

An Overview of Data Collection in Health Preference Research

This paper focuses on survey administration and data collection methods employed for stated-preference studies in health applications. First, it describes different types of survey administration methods, encompassing web-based surveys, face-to-face (in-person) surveys, and mail surveys. Second, the concept of sampling frames is introduced, clarifying distinctions between the target population and survey frame population. The discussion then extends to different types of sampling methods, such as probability and non-probability sampling, along with an evaluation of potential issues associated with different sampling methods within the context of health preference research. Third, the paper provides information about different recruitment methods, including web-surveys, leveraging patient groups, and in-clinic recruitment. Fourth, a crucial aspect addressed is the calculation of response rate, with insights into determining an adequate response rate and strategies to improve response rates in stated-preference surveys. Lastly, the paper concludes by discussing data management plans and suggesting insights for future research in this field. In summary, this paper examines the nuanced aspects of survey administration and data collection methods in stated-preference studies, offering valuable guidance for researchers and practitioners in the health domain.

2.Eur J Cardiovasc Nurs. 2025 Jul 21;24(5):808-812. doi: 10.1093/eurjcn/zvae158.

Challenges and strategies for effective recruitment and retention of participants in clinical research studies

Effective recruitment and retention of participants in clinical research studies are critical to be able to draw meaningful and valid conclusions in research studies. However, there are multiple challenges related to communication, generalizability, and logistics. Researchers must address and overcome these challenges to ensure robust research outcomes. Effective strategies include honest and clear communication, awareness of reasons for (non)-participation, incentivization, and reimbursements of expenses as well as co-designing interventions and research protocols. This paper outlines common issues in participant recruitment and retention and provides practical strategies to overcome challenges.

3.Dis Model Mech. 2025 Jul 1;18(7):dmm052401. doi: 10.1242/dmm.052401. Epub 2025 Jul 28.

Global partnerships in rare disease research

Rare diseases collectively impact hundreds of millions worldwide, yet the genetic causes of many remain unknown or poorly understood. Model organisms (MOs) - such as yeast, fly, zebrafish and mouse - provide powerful experimental systems for functional validation of candidate genes and variants, elucidation of gene function and disease mechanisms, and identification of potential therapeutic targets and treatments. However, gaps persist between clinical gene discovery and MO-based research. The Canadian Rare Diseases: Models and Mechanisms (RDMM) Network was established in 2014 to address this gap by linking clinicians with MO researchers through a scientist registry and peer-reviewed funding process. Over the past decade, the RDMM Network has funded over 160 collaborative projects, enabled insights into numerous rare conditions, and led to sustained partnerships and external funding. The RDMM Registry software has been adopted internationally, forming a network of interoperable registries that enable cross-border collaborations and expand access to MO expertise worldwide. Going forward, the Canadian RDMM Network remains committed to sharing its tools, processes and experience to help establish new RDMM-like networks worldwide and invites the global research community to join efforts to accelerate rare disease research.

4.J Exp Anal Behav. 2025 Jul;124(1):e70040. doi: 10.1002/jeab.70040.

Research synthesis in behavior analysis I: An introductory guide to conducting systematic reviews

As a data-driven science, the field of behavior analysis necessitates accumulating evidence for research and theory development and clinical intervention. The most comprehensive evidence will come from systematic review and meta-analysis of a given topic. Systematic reviews comprise an established set of methods for collecting and synthesizing a body of research to identify trends, examining the strength of evidence and potential sources of bias, and identifying areas in need of further investigation. Despite their utility and widespread use in other disciplines, systematic reviews are underused in many behavior analysis domains. This technical report is part of a series on research synthesis methods in behavior analysis, with Part 1 focusing on systematic reviews and Part 2 focusing on meta-analysis. In Part 1, we provide a step-by-step guide to conducting systematic reviews using current best practices and adhering to international guidelines. Examples of tables and figures commonly included in these types of reviews are also provided. We conclude by emphasizing the importance of these reviews for behavior analysis research, practice, and theory and calling for increased numbers of published systematic reviews in behavior analysis. Finally, we provide annotated references to additional in-depth methodology resources for the interested behavior analyst.

5.Biometrics. 2025 Jul 3;81(3):ujaf097. doi: 10.1093/biomtc/ujaf097.

Covariance-on-covariance regression

A covariance-on-covariance regression model is introduced in this manuscript. It is assumed that there exists (at least) a pair of linear projections on outcome covariance matrices and predictor covariance matrices such that a log-linear model links the variances in the projection spaces, as well as additional covariates of interest. An ordinary least square type of estimator is proposed to simultaneously identify the projections and estimate model coefficients. Under regularity conditions, the proposed estimator is asymptotically consistent. The superior performance of the proposed approach over existing methods is demonstrated via simulation studies. Applying to data collected in the Human Connectome Project Aging study, the proposed approach identifies 3 pairs of brain networks, where functional connectivity within the resting-state network predicts functional connectivity within the corresponding task-state network. The 3 networks correspond to a global signal network, a task-related network, and a task-unrelated network. The findings are consistent with existing knowledge about brain function.

6.Stat Med. 2025 Jul;44(15-17):e70180. doi: 10.1002/sim.70180.

An IPCW Adjusted Win Statistics Approach in Clinical Trials Incorporating Equivalence Margins to Define Ties

In clinical trials, multiple outcomes of different priorities commonly occur as the patient's response may not be adequately characterized by a single outcome. Win statistics are appealing summary measures for between-group difference at more than one endpoint. When defining the result of pairwise comparisons of a time-to-event endpoint, it is desirable to allow ties to account for incomplete follow-up and not clinically meaningful difference in endpoints of interest. In this article, we propose a class of win statistics for time-to-event endpoints with a user-specified equivalence margin. These win statistics are identifiable in the presence of right censoring and do not depend on the censoring distribution. We then develop estimation and inference procedures for the proposed win statistics based on inverse-probability-of-censoring weighting adjustment to handle right censoring. We conduct extensive simulations to investigate the operational characteristics of the proposed procedure in the finite sample setting. A real oncology trial is used to illustrate the proposed approach.

7.NEJM Evid. 2025 Aug;4(8):EVIDctw2400393. doi: 10.1056/EVIDctw2400393. Epub 2025 Jul 22.

Multiplicity Control in Clinical Trials

Statistical testing of more than one hypothesis has the potential to increase the risk of wrongly concluding that the result for a given end point is statistically significant (false discovery). This review is designed to acquaint nonstatisticians with traditional approaches for controlling type I error and with the seemingly complex procedure known as graphical testing.

8.Value Health. 2025 Jul;28(7):1126-1135. doi: 10.1016/j.jval.2025.02.001. Epub 2025 Feb 13.

Statistical Methods for Analyzing EQ-5D in Randomized Clinical Trials: A Systematic Literature Review

Objectives: We conducted a systematic literature review to summarize the application of statistical methods for analyzing treatment effect on EQ-5D in randomized clinical trials (RCTs).
Method: We searched 2 electronic databases (MEDLINE and EMBASE, from inception through 2021) and www.Clinicaltrial.gov. Eligible studies were RCTs that analyzed postbaseline EQ-5D data by treatment group. Information on trial characteristics, EQ-5D data characteristics, and statistical methods were extracted. Descriptive statistics were used to summarize results by dimension response, EQ visual analog scale (EQ VAS), and EQ-5D utility.
Results: A total of 2125 trials met the eligibility criteria. EQ-5D was commonly considered a secondary (n = 1219, 57.4%) or exploratory (n = 775, 36.5%) endpoint in RCTs. EQ-5D utilities were the most analyzed. Both utilities and EQ VAS were primarily analyzed in numerical format. The most common statistical models for analyzing utilities were the linear fixed-effect model for single postbaseline (192/589, 32.6%) and the linear mixed-effect model for multiple post-baselines (338/984, 34.3%). Of the 2054 studies that analyzed numerical EQ-5D, 221 (10.8%) examined model assumptions and 438 (21.3%) adjusted for the baseline score. Missing data were explicitly assessed in 661 trials, among which 347 (52.5% of 661) applied imputations, with the 2 most used imputation methods being multiple imputations (n = 200, 57.6% of 347) and last observation carried forward (n = 106, 30.5% of 347).
Conclusions: This review found that health utilities are the most frequently analyzed EQ-5D data collected in clinical trials, followed by EQ VAS. Significant variation was observed in the selection of models, with most trials lacking adjustments for baseline data and appropriate methods for handling missing data.

9.Br J Psychiatry. 2025 Jul 21:1-2. doi: 10.1192/bjp.2025.113. Online ahead of print.

Diagnostic accuracy for multiple categories: statistical advice

10. Stat Methods Med Res. 2025 Jul 30:9622802251362644. doi: 10.1177/09622802251362644. Online ahead of print.

Efficient randomized adaptive designs for multi-arm clinical trials

In clinical trials, response-adaptive randomization (RAR) has gained increasing attention due to its ability to assign more patients to better-performing treatments. Consequently, several RAR methods have been proposed in recent years. Among them, the efficient response adaptive randomization design (ERADE), proposed by Hu et al. (2009), stands out as an optimal approach, with the asymptotic variance of the allocation proportion achieving the Cramér-Rao lower bound, demonstrating its statistical efficiency. However, the original ERADE is limited to trials with only two treatment arms. Given the growing prevalence of multi-arm trials in modern clinical development, the original ERADE design no longer meets all practical needs. In this paper, we extend ERADE for use in multi-arm clinical trials, proposing the multi-arm ERADE algorithm. We establish the asymptotic properties of this generalized design and demonstrate its effectiveness in finite sample settings through simulations and a real-world trial redesign.

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