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## APPENDIX: ABSTRACTS OF ARTICLES BASED ON THIS PCORI-FUNDED RESEARCH

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### **beanz: An R Package for Bayesian Analysis of Heterogeneous Treatment Effect With Graphical User Interface**

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In patient-centered outcomes research, it is essential to assess the heterogeneity of treatment effect (HTE) when making health care decisions for an individual patient or a group of patients. Nevertheless, it remains challenging to evaluate HTE based on information collected from clinical studies that are often designed and conducted to evaluate the efficacy of a treatment for the overall population. The Bayesian framework offers a principled and flexible approach to estimate and compare treatment effects across subgroups of patients defined by their characteristics. In this paper, we describe the package *beanz*, which facilitates the conduct of Bayesian analysis of HTE by allowing users to explore a wide range of Bayesian HTE analysis models and produce posterior inferences about HTE. The package *beanz* also provides a web-based graphical user interface (GUI) for users to conduct the Bayesian analysis of HTE in an interactive and user-friendly manner. With the GUI feature, *beanz* can also be used by analysts not familiar with the R environment. We demonstrate *beanz* using data from a randomized controlled trial on angiotensin-converting enzyme inhibitor for treating congestive heart failure (N = 2569).

## Bayesian Analysis of Heterogeneous Treatment Effects for Patient-centered Outcomes Research

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Evaluation of heterogeneity of treatment effect (HTE) is an essential aspect of personalized medicine and patient-centered outcomes research. Our goal in this article is to promote the use of Bayesian methods for subgroup analysis and to lower the barriers to their implementation by describing the ways in which the companion software **beanz** can facilitate these types of analyses. To advance this goal, we describe several key Bayesian models for investigating HTE and outline the ways in which they are well-suited to address many of the commonly cited challenges in the study of HTE. Topics highlighted include shrinkage estimation, model choice, sensitivity analysis, and posterior predictive checking. We present a case study in which we demonstrate the use of the methods discussed.

## Considerations When Assessing Heterogeneity of Treatment Effect in Patient-Centered Outcomes Research

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When baseline risk of an outcome varies within a population, the effect of a treatment on that outcome will vary on at least 1 scale (eg, additive, multiplicative). This treatment effect heterogeneity is of interest in patient-centered outcomes research. Based on a literature review and solicited expert opinion, we assert the following: (1) Treatment effect heterogeneity on the additive scale is most interpretable to health care providers and patients using effect estimates to guide treatment decision making; heterogeneity reported on the multiplicative scale may be misleading regarding the magnitude or direction of a substantively important interaction. (2) The additive scale may give clues about sufficient-cause interaction, although such interaction is typically not relevant to patients' treatment choices. (3) Statistical modeling need not be conducted on the same scale as results are communicated. (4) Statistical testing is 1 tool for investigations, provided important subgroups are identified a priori, but test results should be interpreted cautiously given nonequivalence of statistical and clinical significance. (5) Qualitative interactions should be evaluated in a prespecified manner for important subgroups. Principled analytic plans that take into account the purpose of investigation of treatment effect heterogeneity are likely to yield more useful results for guiding treatment decisions.