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Enhancing Generalizability in Patient Preference Studies: Addressing Sample Skewness in the associated Covariate Distribution

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Brett Hauber
Please provide a brief biography for the Presenting author(s)
I am a trained economist specializing in individual utility and preference analysis. My early work was in environmental economics. For the past 20 years, I have been working in health and pharmaceuticals conducting surveys to understand patient, physician, and decision-maker preferences for health interventions. After a long career as a researcher at RTI Health Solutions, I joined Pfizer in 2021.

Marco Boeri
Please provide a brief biography for the Presenting author(s)
Marco Boeri, PhD, is a Director of preference research and scientific lead for the Patient centered outcomes group at OPEN Health and honorary professor in practice in health economics at Queen's University Belfast. Dr. Boeri has over 15 years of experience in preference assessment in environmental and health economics. He has extensive knowledge and expertise in experimental design, survey development, and modelling data from preference studies in health, food, and environmental economics. Dr. Boeri’s research focuses on the econometric analysis of heterogeneity in discrete-choice experiments and on comparing various stated preference analysis methodologies at the individual, household, and sample level.
Dr Boeri has coauthored the first applications of the Random Regret Minimization model in both environmental and health economics and published over 50 peer-reviewed papers, including several in applied economics journals across different disciplines, such as Pharmacoeconomics, Journal of Health Economics, Health Economics, Patient Prefer Adherence, Social Science and Medicine, Value in Health, Medical Decision Making, Preventive Medicine, Environmental and Resource Economics, Energy Economics, Transportation Research Part A, the Journal of Economic Behavior and Organization, and Journal of Choice Modelling, demonstrating the applicability of his methodological tool at top levels in different topics and fields.

Divya Mohan
Please provide a brief biography for the Presenting author(s)
Divya Mohan is a Senior Scientist in the Patient-Centered Outcomes team, based in London. She has a PhD in Health Economics from the University of Aberdeen. Her PhD focused on integrating preference heterogeneity into economic evaluations in healthcare. Prior to joining OPEN Health, she worked as a Postdoctoral Research Fellow in the Preference and Value team at the University of Aberdeen, and before that as a Manager in charge of economic modelling for the legal services team at a consulting firm. Divya’s research and publications focus on DCEs and economic modelling.

Byron Jones
Please provide a brief biography for the Presenting author(s)
Until the end of 2022, Dr Byron Jones was a Senior Biometrical Fellow and Executive Director in the Statistical Methodology and Consulting Group at Novartis Pharma AG in Basel, Switzerland. He is now partly retired and works as an external Novartis employee, specializing in the design and analysis of Patient Preference Studies. He is a Fellow of the American Statistical Association and the co-author of nine statistical textbooks.

For the twenty-five years before joining the pharmaceutical industry he worked in academia, ultimately holding the position of Professor of Medical Statistics at De Montfort University, UK. After leaving academia, Byron held Honorary Professorial positions at four UK universities: University College London, the London School of Hygiene and Tropical Medicine, University of Leicester and Queen Mary, University of London. Prior to joining Novartis in 2011 he held senior positions at GSK and Pfizer.

He has been the Chairman of the External Advisory Board to the joint University of Oxford and Imperial College London, Center for Doctoral Training and before that he was an advisor to the Department of Statistics at Oxford University. Byron was a member of the ICH Expert Working Group that revised the ICH E8 guidance on "General Considerations for Clinical Studies".

Byron was a Series Editor for the Chapman and Hall/CRC Press' Biostatistics book series, a Founding Editor-in-Chief of the PSI journal Pharmaceutical Statistics, formerly an Associate Editor of JRSS Series B and a Regional Editor of the Journal of Biopharmaceutical Statistics. He has been a Board of Directors' member of PSI (Statisticians in the Pharmaceutical Industry) and in 2016 led the successful campaign to save PSI from being overtaken by a larger statistical society.

He is passionate about Patient-Focused Drug Development and the use of patient preference studies to understand the needs of patients. He was a member of the PREFER consortium and is now a member of its follow-up body, the Patient Engagement Network.

Josh Coulter
Please provide a brief biography for the Presenting author(s)
As a Director of Preference Elicitation at Pfizer, I bring a wealth of experience and expertise in conducting preference studies, analyzing data, and interpreting results. My strong analytical skills, attention to detail, and ability to communicate effectively make me an asset to any project team. I am a skilled problem-solver and am able to adapt to changing circumstances and priorities. I have a track record of success in leading and managing projects, collaborating with team members, and building positive relationships with subcontractors. In addition to my technical abilities, I am a strong communicator with excellent presentation skills. I am confident in my ability to present findings and recommendations to both technical and non-technical audiences. Overall, my diverse background in research and data analysis has given me a well-rounded skill set and the ability to contribute value to any organization.

Conny Berlin
Please provide a brief biography for the Presenting author(s)
Conny Berlin is an Executive Director Patient Experience Data Science at Novartis. She holds a diploma in mathematics and has been working in the pharmaceutical industry for 30 years. Conny is a scientific leader with significant experience in drug development and talent development. Between 2012 and 2021, she led the Quantitative Safety & Epidemiology group at Novartis, which supports clinical teams with analytical strategies to best assess patient safety. Since 2022, Conny has been leading the Patient Engagement Science group at Novartis, a team of scientists who drive and support the implementation of patient-focused drug development. This includes the design and conduct of patient preference studies to better understand what is important to patients, as well as the set up of concepts to involve patients in the design of clinical development programs. During her career, Conny successfully led several initiatives at Bayer and Novartis to develop and implement cutting edge signal detection methods and tools, new safety analysis approaches for specific safety risks, and structured benefit risk. Between 2016 and 2022, she was the industry lead of IMI PREFER, a public private consortium that developed the PREFER Recommendations including a framework and points to consider for method selection for patient preference studies.

Single topic, multi-speaker session, Workshop or Single presentation submission

A single presentation/poster

Single presentation or poster submission

The growing demand for quantitative patient preference information (PPI) from regulators and health technology assessment bodies underscores the critical need for accurate and generalizable results. Challenges in achieving representativeness and generalizability in PPI samples are well known. Traditional approaches often rely on oversampling specific subsamples (underserved populations) or setting specific target sample sizes to explore preference heterogeneity. However, PPI samples frequently exhibit skewness towards specific patient demographics, such as white, women or more highly educated individuals.

This can be relevant when the distribution of the covariates in the study sample does not match the distribution of these same covariates in the target population (i.e., general population, patients with a specific condition, or trial population). Various approaches can be used to redress the skewness of the study sample covariates so that the distribution of covariates in the sample population matches that in the target population. Weighting adjustment techniques, including iterative proportional fitting and post-stratification, are effective when the discrepancies between the target population and the study sample are minimal. However, these methods become less reliable as the divergence increases, leading to suboptimal results. Consequently, alternative approaches, such as matching with a simulated target population or employing individual conditional parameters generated from a mixed logit or latent class logit model, are being explored to enhance the robustness of the analysis.

Using data from two completed preference studies, the presentation will describe and illustrate the application of these various techniques to enhance generalizability. The empirical application of these techniques will be critically assessed.