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Assessing the Readiness of the Patient Preference Study Landscape for Meta-Analyses and Benefit Transfers: Do We Always Need a New Preference Study?

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Michael Bui
Please provide a brief biography for the Presenting author(s)
Michael Bui is a PhD candidate at the Health Technology and Services Research section of the University of Twente. His research focuses on the development of methods and guidelines for performing benefit transfers of patient preference information.

Janine van Til
Please provide a brief biography for the Presenting author(s)
Dr. Janine van Til (female), PhD is an associate professor in health preference research at the department of Health Technology and Services Research at the University of Twente since 2007. She received her Master degree as a health and movement researcher from the Vrije Universiteit Amsterdam in 2001 and her PhD degree for her work in supporting shared decision making in the treatment of stroke patients in 2009. Dr. Janine van Til's current research is focused on supporting shared decision making with the use of quantitative value elicitation techniques. Moreover, she aims to increase patient and public involvement organizational and societal decision making in health care. Currently, she is involved in projects related to the improvement of patient care after post-anoxic coma, cardiovascular disease, and cancer. She is a member of the International Academy of Health Preference Research, the society of Medical Decision Making and the special interest group for health preference research at the Professional Society for Health Economics and Outcomes Research (ISPOR). Her methodological expertise includes the design, analysis and interpretation of health preference studies (conjoint analysis, best worst scaling) and multi-criteria decision analysis.

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.

Karin Oudshoorn
Please provide a brief biography for the Presenting author(s)
I'm a biostatistician with an research interest in obtaining, in a methodologically sound way, patients', physicians' and other stakeholders' preferences, values and needs to support decisions in health care.
My main areas of expertise are the design, analysis and interpretation of preference studies (conjoint analysis, best worst scaling) and multi-criteria decision analysis studies.

My other interest is in the emerging field of statistical learning (or alternatively machine learning). I have a lot of experience in developing and analyzing predictive (regression) models and handling missing data with multiple imputation.

Since January 2020 I am a member of the Behavioural Data Science Incubator at the BMS faculty of the University of Twente (https://www.utwente.nl/en/bms/research/support/news/2020/4/575342/behavioural-data-science-incubator).
Additionally, I have more than 15 years of broad experience as a registrated biostatistician in health services research at several research institutes on a diverse applications in the biomedical field. This includes all steps of designing clinical and observational studies, analyzing and modelling clinical data and reporting (https://www.researchgate.net/profile/Catharina_Groothuis-Oudshoorn). I am an enthusiastic R user and teacher.

Cecilia Jiminez-Moreno
Please provide a brief biography for the Presenting author(s)
Leveraging from my experience as HCPC, at Kielo Research my focus as a Senior Research Associate encompasses patient involvement in the drug development pathway, particularly within the realm of rare diseases.

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 setup 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

Although patient preference (PP) studies are costly, time-intensive, and burdensome on patients, their findings are rarely used beyond the purpose of the original study. If PP study findings could be transferred to other contexts through meta-regression (benefit transfers), resources could be better utilized and potentially novel drug development speeded up. We conducted a scoping review to assess the readiness of the current PP study landscape for evidence synthesis and transferability research.

Quantitative PP studies examining risks and benefits of treatments were identified through a systematic search on PubMed, Scopus, and Web of Science. Based on benefit transfer guidelines from environmental economics, prospects for transferring PP study findings were judged based on: the number of studies across indications, consistency in elicitation methods, consistency in treatment attributes, and consistency in reported preference information.

In total, 645 studies were included. Of these, 72.2% were discrete choice experiments (DCEs) and most studies were conducted in the USA. Indication-wise, most research was concentrated in type 2 diabetes (T2D): 43 DCEs, 8 non-DCEs.

The landscape of PP studies is dispersed across various indications and therapeutic focus areas, which generally limits inter-study comparisons. However, numerous DCEs on T2D exhibited a high consistency in reported outcome measures, and a moderately high degree of overlap in studied attributes (hypoglycaemia, glycaemic control, weight change, out-of-pocket costs). Thus, benefit transfers seem feasible in T2D.

This presentation will discuss the issues involved in the transferal of PP results and present a way forward for exploring evidence synthesis and transferability research in general.