118

Published patient preference studies can influence the choice of endpoints in clinical trials: An example from Atopic Dermatitis

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Seren Phillips
Please provide a brief biography for the Presenting author(s)
Seren Phillips is currently a Senior Director in Patient Engagement Data Science at Novartis with a particular focus on the design and application of patient preference studies to support development. With a career spanning roles at NICE and in industry Seren has a deep understanding of study design, evidence generation and how evidence will be perceived by healthcare decision makers. She has held positions in clinical research, health economic and outcomes research and market access. As head of NICE scientific advice she advised companies on how to optimize their clinical and economic development programmes in order to meet the needs of a future health technology assessment. She has Masters degrees in Clinical Pharmacology and Health Economics.

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 employee of Novartis, 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 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 has been a Series Editor for the Chapman and Hall/CRC Press' Biostatistics book series, a Founding Editor-in-Chief of the 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.

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

A single presentation/poster

Single presentation or poster submission

Although it is widely accepted that the inclusion of patient relevant outcomes is important in medicines development, in practice, difficult choices need to be made regarding which outcome measures it is feasible to include in clinical trials.

Atopic dermatitis (AD) is a chronic inflammatory skin disease affecting approximately 15-20% of children and up to 10% of adults. Traditionally, the physician administered endpoint Eczema Area and Severity Index (EASI) has been included as a primary or co-primary endpoint in regulatory AD trials.

A targeted literature review (TLR) of published patient preference studies (PPS) in AD was undertaken to learn what endpoints are actually preferred by patients. This was challenging, because even if all PPS address more or less the same research question, they are still slightly different in terms of the number of attributes, their levels and descriptions.

The presentation will discuss the way the TLR was conducted and discuss its conclusion that the traditionally used endpoint EASI does not capture what patients see as most important. In terms of symptoms, itching was consistently seen as one of the most troublesome symptoms. We will show how the results of the TLR were used to support and prioritize recommendations on a patient-centred outcome strategy for a new medicine in development for the treatment of AD.

In conclusion, the TLR showed that PPS can provide robust data on the relative importance of different treatment attributes which can be used to guide decisions on the selection and prioritization of outcomes to include in trials.