

Three new methodologies for calculating the effective sample size when performing population adjustment

Landan Zhang¹, Dan Jackson², Sylwia Bujkiewicz³

¹Bayer PLC, Reading, United Kingdom. ²AstraZeneca, Cambridge, United Kingdom.

³University of Leicester, Leicester, United Kingdom

Landan Zhang

Please provide a brief biography for the Presenting author(s)

Landan Zhang is currently a Senior Medical Affairs Statistician at Bayer. She provides statistical insights to the Medical Affairs and Market Access teams and contributes to health technology assessment (HTA) submissions, life-cycle management strategies, and methodology publications.

Before joining Bayer, Landan worked as a Postdoctoral Research Fellow in the Statistical Innovation Group at AstraZeneca, specializing in methods for population-adjusted indirect treatment comparisons.

Dan Jackson

Please provide a brief biography for the Presenting author(s)

Dan Jackson is Statistical Science Director in the Statistical Innovation Group at AstraZeneca; his primary research interests are methods for meta-analysis, survival analysis and population adjustment.

Sylwia Bujkiewicz

Please provide a brief biography for the Presenting author(s)

Sylwia Bujkiewicz is Professor of Biostatistics in the Biostatistics Research Group at the University of Leicester; her primary research interests are in the area of Bayesian methods for evidence synthesis, with a main focus on multi-parameter evidence synthesis for combining data from diverse sources.

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

A single presentation/poster

Single presentation or poster submission

The concept of population is fundamental in epidemiology and statistics, represented as the 'P' in PICOS statements and as a key attribute of an estimand. Direct sampling from the population of interest is not always feasible, leading to the use of weighting as a statistical method for making inferences when samples are unrepresentative of the population of interest. The effective sample size (ESS) estimates the sample size required for an unweighted sample to achieve the same precision as a weighted sample. ESS reflects the information retained after weighting and is easy to interpret. Traditional ESS calculations rely on strong assumptions, such as homoscedasticity of outcome data. We propose three new approaches to compute ESS that are valid for any data type and weighted analysis. The first method compares the variances of weighted and unweighted estimates by calculating variances in a valid way, regardless of data type or statistical model. The second method uses re-sampling to find the smallest unweighted sample size that produces variance at least as large as that of the weighted analysis. The third method uses existing closed-form variance expressions for unweighted estimates, applying scale factors to determine the necessary sample size for equivalent precision. We illustrate these methods with a numerical example involving a matching-adjusted indirect comparison analysis. Our findings highlight the importance of accurate ESS calculations for valid statistical inferences.

in weighting techniques for population adjustment. This research aims to improve the conventional calculation and application of ESS for statistical inference.