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Statistical Challenges in Health Technology Assessment (HTA) for Rare Diseases

Samadhan Ghubade
University of Pune, Pune, India. Symbiosis International University, Pune, India. ICON PLC, Bangalore, India. Cytel Inc., Pune, India

Samadhan Ghubade
Please provide a brief biography for the Presenting author(s)
Samadhan Ghubade holds a master's degree in Statistics. He is currently working as a Senior Biostatistician at ICON PLC and pursuing a Ph.D. in the field of drug safety and Statistics. With a decade of experience as seasoned biostatistician, he has made significant contributions to healthcare industry, playing a vital role in ensuring the accuracy and reliability of statistical analyses in drug safety research, addressing crucial questions related to drug development, clinical trials, pharmacovigilance and regulatory compliance. His commitment to advancing scientific knowledge was highlighted by his presentations of groundbreaking research papers at in international conferences such as PSI, PhUSE etc. where he shared insights with global experts in the field. Outside the professional realm, he is deeply committed to social, particularly in education sector where he actively contributes to initiatives benefiting students. His passion for both statistical research and social impact reflects a well-rounded and dedicated professional who strives to make a positive difference in both the scientific and societal domains. Also, contributing as Top Statistics Voice on various topics on professional platforms.

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Health Technology Assessment (HTA) for rare diseases create unique challenges, including inadequate data, diverse patient profiles, and high levels of uncertainty. To deal with these problems, this study presents a framework for the future that uses advanced statistical methods. By mixing Bayesian hierarchical models with meta-analytic tools, the method makes HTA decisions better by bridging the gap between data from clinical trials and evidence from the real world.

The use of the concept will be demonstrated by a case study on an innovative gene therapy for a rare metabolic condition. This study assesses cost-effectiveness by combining data from sources and then using probabilistic sensitivity analysis to adjust for uncertainty. By identifying patient characteristics linked to results, subgroup analyses provide personalized treatment alternatives.

This innovative framework will address the intrinsic complexities of HTA for rare diseases and also demonstrates how statisticians can drive impactful policy decisions. By delivering actionable insights and supporting resource allocation for high-need populations, this work highlights the critical role of statistical attention in shaping the future of healthcare for rare diseases.

This new way of thinking not only solves the problems that come with HTA for rare diseases, but it also shows how researchers may be the ones making policy decisions that affect a lot of people. This research highlights the critical role that statistical accuracy will play in

determining the future of healthcare by providing insights that can be put into action and by providing support for the allocation of resources to populations with high needs.