

Title: Enhancing Medical Product Development and Review with Bayesian Quantitative Benefit Risk Methods

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ABSTRACT:

Establishing the benefit-risk profile of a new medicinal product or intervention is a fundamental requirement for sponsors and regulators. It is also one of the most complex tasks payers, physicians, and patients face. Therefore, communicating the trade-off of benefits and risks in a clear and transparent manner, and using all available evidence, is critical to ensure that the best decisions are made for patients. Bayesian inference allows for integration of different sources of information and uncertainty, along with its links to optimal decision theory, providing a natural framework to perform quantitative assessments of the benefit-risk trade-off.

In this talk, I will introduce ASA BIOP Bayesian Scientific Working Group's Benefit-Risk Subteam, a group of individuals from pharmaceutical companies, regulatory agencies, and academia, with a special interest and expertise in benefit-risk evaluation. I will highlight some of our achievements in the past few years for furthering the science of quantitative benefit-risk assessments (QBRA). As a team, we have organized and participated in numerous conference sessions, developed methodological innovations and software for Bayesian benefit-risk analysis, and disseminated our research findings in scientific publications. Some of the software we have developed include an R Shiny App for Bayesian Multi-Criteria Decision Analysis / Stochastic Multi-Criteria Acceptability Analysis, an R Shiny App and package for Hierarchical Bayesian Benefit-Risk, and an R packages for holistic benefit-risk analysis. I'll walk through some of these using a few case studies.

The impact of our work can be seen in the increased ability of stakeholders to make more informed decisions regarding benefit-risk assessments, ultimately contributing to improved patient safety and healthcare outcomes. My talk will end with some reflections on recent regulatory thoughts on leveraging QBRA and general recommendations for planning and conducting Bayesian QBRA with a look toward the future.