Bayesian Statistics and Real-World Evidence: A Successful Example from CBER

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Abstract:

Despite consensus among statisticians that Bayesian methods are valid, there are few successful regulatory examples that use Bayesian methods. In this talk, I will discuss in detail a recent example where Bayesian methods were successfully used to update the label of a pertussis vaccine to include an indication for immunization during pregnancy to protect the infant. In this example, a Bayesian meta-analysis of previously published studies was used as the prior for a Bayesian analysis of real-world data (RWD) to generate real-world evidence (RWE) in support of the indication, including an estimate of vaccine efficacy that was included in the product labeling. I will give a brief description of the regulatory history of this file, to highlight the regulatory perspective on the use of Bayesian methods in this case. After this introduction, I will discuss the statistical methods used in detail, including the statistical issues highlighted during the review related to both Bayesian analyses, such as the review of the prior and the sensitivity analyses performed for each Bayesian analysis. Finally, I will discuss the unique challenges presented by communicating a Bayesian result in product labeling, given the lack of precedent.