Title: Augmenting clinical trial data with external controls through energy balancing weighted power prior.

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ABSTRACT:

In recent years, there has been growing interest in utilizing real-world data (RWD) in drug development to enhance the precision of treatment effect estimates, reduce sample size requirements, expedite development timelines, and lower costs. However, integrating RWD with clinical trial data can introduce biases, inflate type I errors, and decrease power. Traditional Bayesian dynamic borrowing methods like power prior and meta-analytic-predictive prior address these issues by down-weighting external data in the presence of heterogeneity, but they mainly adjust for differences in outcomes, not covariates. We present a novel hybrid approach that augments clinical trial data with external controls using an energy balancing weighted power prior. This method adjusts for differences in both outcomes and covariates without requiring tuning parameters or specifying the treatment assignment mechanism.

The presentation will cover:
1. The motivation for using RWD in clinical trials and limitations of current methods.
2. The theoretical framework of the novel hybrid approach.
3. Performance comparison with other methods through a simulation study.
4. A case study in rare disease research.
5. Regulatory considerations for using external control data and implementing this methodology.

This approach promises to enhance the robustness and reliability of integrating RWD in clinical trials, offering a more comprehensive adjustment for confounding factors and ultimately improving the credibility of trial outcomes.

Presenting author short bio: Shaoming Yin is currently an Associate Director of Statistics at Takeda Pharmaceuticals in Cambridge, MA. He assumes statistics leadership in various drug development programs across phases I through IV, focusing on rare diseases and neuroscience. At Takeda, he co-authored a Bayesian methodology paper for rare disease applications and successfully designed a Bayesian dynamic borrowing post-marketing commitment study, achieving FDA alignment.
Previously, Shaoming served as Senior Director of Biometrics at Pharma Medica Research Inc. and Principal Statistician at Cytel Inc. He holds a Master's degree in Statistics and a Bachelor's degree in Statistics and Biochemistry from the University of Toronto. Shaoming is currently pursuing a PhD in Data Science (Biostatistics) at Worcester Polytechnic Institute, MA.