Case Study

Bayesian Extrapolation

Bayesian Extrapolation to Evaluate Efficacy in Pediatric Subjects using Adult Data

KerusCloud®

KerusCloud® is a revolutionary simulation-guided study design tool that ensures clinical trials are designed effectively to collect the right data, in the right patients, in the right way. Its use supports evidence-based design decisions to extensively de-risk real clinical studies, reducing development time, costs and patient burden.

The Challenge

A pharmaceutical company's pediatric clinical trial was stopped early due to slow recruitment, enrolling only 68% of the planned participants. The EMA's Pediatric Committee recommended an extrapolation analysis using a Bayesian approach that incorporated adult trial data to improve the precision of pediatric efficacy results, under a tight timeline. Because the original sample size was not achieved, the sponsor also reassessed the study's probability of success if it were adapted to include fewer patients than initially planned.

The Approach

KerusCloud® was first used to quickly assess whether the complex, multi-stage Bayesian analysis was feasible and likely to succeed, confirming a good chance of generating strong pediatric efficacy evidence. The subsequent work involved identifying relevant adult studies, checking that pediatric results were consistent with adult findings using frequentist methods, and then performing a Bayesian extrapolation analysis. This used adult data to form informative priors, which were combined with weak priors in a dynamic borrowing framework that automatically determined how much adult information to incorporate based on its consistency with the pediatric data.

The Results

KerusCloud® showed that the Bayesian dynamic borrowing approach was feasible and had a good probability of success before any work was undertaken. The subsequent analysis went on to show:

- The available efficacy data for the pediatric population appeared consistent with that for the larger adult population, as there were no observed differences in treatment effect across the adult and pediatric populations.
- A significant increase in the primary endpoint in pediatric subjects could be supported through the extrapolation analysis with only a relatively small (25%) prior confidence in the pediatric treatment effect being similar to the adult treatment effect.

The Impact

Extrapolation of drug efficacy in adults to pediatric subjects could be supported in this case as part of a robust evidence package for Regulators.

"MMS has provided excellent service on the Bayesian extrapolation of efficacy in pediatric patients. This is a highly technical expertise that several preferred vendors could not provide when we sought bids from them for this project. I can't say enough it was an absolute pleasure to work with the team at MMS!"

Project Statistician and Statistical Manager, Large Pharmaceutical Company

Let's talk!

If you'd like to discuss this case study further or learn more on how our technology enabled services can support your development project, please visit **mmsholdings.com** or get in touch **info@mmsholdings.com**