Aligning Estimands and Their Estimators
– A Case Study

Elena Polverejan, Ph.D.
Associate Director, Statistical Modeling and Methodology
Janssen R&D, Johnson & Johnson

Abstract:
Draft ICH E9(R1) Addendum on “Estimand and Sensitivity Analysis in Clinical Trials” provides different strategies for addressing intercurrent events in defining the estimand and describing the treatment effect that is targeted. Clinical trials are often faced with more than one intercurrent event and the set of intercurrent events for consideration will depend on the specific therapeutic setting and trial objectives. This presentation will consider a case study of a trial investigating treatment effect of a new drug in subjects who are asymptomatic at risk for developing Alzheimer’s dementia to illustrate the definition of different estimands which correspond to different scientific questions of interest. The potential intercurrent events will be identified and the case study will highlight how the selection of various strategies for intercurrent events translates into different estimators. A simulation investigation will also be presented, illustrating how the selection of the estimators for an estimand could have a strong impact on the estimates of the treatment effect.