SESSION IV: HOW TIGHTLY SHOULD INVESTIGATORS ATTEMPT TO CONTROL OR RESTRICT TREATMENT QUALITY IN A PRAGMATIC OR REAL-WORLD TRIAL?

National Pharmaceutical Council®

On One Hand: Tight Control Needed to Support Valid Inference and Patient Safety

On The Other Hand: Loose Control Needed to Support Valid Inference and Patient Safety

It Depends: Control to Support Valid Inference and Patient Safety

Assumptions:

- Study question clearly defined (decision and decision-maker the study should inform)
- Data are of adequate quality to assess eligibility, key prognostic factors, treatment exposure, and outcomes.
- Treatments are assigned randomly or by some other method that supports valid inference.

In scope:

- *Treatment, setting, provider control*
- *Participant safety*

Decision Aid for Discussion

How much would the effectiveness or safety of the study treatment(s) vary among providers or care settings? How is this variability related to different levels of resources, experience, or expertise?

What level(s) of resources/experience/expertise are now present in the care settings in which results of this trial will be applied?

What level(s) of resources/experience/expertise are now present in the care settings in which this trial could be conducted?

What special vulnerabilities or risks are anticipated in the study population?

Is there some minimal or floor level of treatment quality necessary for valid inference regarding the study question?

Is there some minimal or floor level of treatment quality necessary to assure participant safety?