

Gary Cutter - *Adaptive trial designs in rare disorders*

Abstract:

Adaptive designs are not new and may not even be innovative. They are done for efficiency. In rare diseases sample sizes are always an issue. Longer trials don't compensate for lack of sample size and the desire to move faster is always present. Use of biomarkers and preferably surrogate outcomes can speed the time of investigation. Examples of older adaptive and futility designs are discussed as well as the risk and benefits of platform designs. Some general pitfalls of entrance criteria, ethical decisions in design of trials and the use of medical records to get around trials are also presented