2nd European Congress on

Pharmaceutical Science & Research

February 23, 2022

A Small Data Perspective on Trial Design: Concepting for Rare Disease Trials

Steven M. Schwartz
Tridiuum Health Individuallytics Inc,. USA

Rare diseases in the U.S. are defined as having a patient population of <200,000. Although each disease impacts a small population, there are an estimated 7,000 documented rare diseases. Since 1983, more than 600 agents treating rare diseases have reached the market and more than 500+ are currently in development. Low prevalence rates for rare diseases run contrary to tradition drug development processes. Rigorous testing the pipeline for rare conditions continues to be an industry challenge.

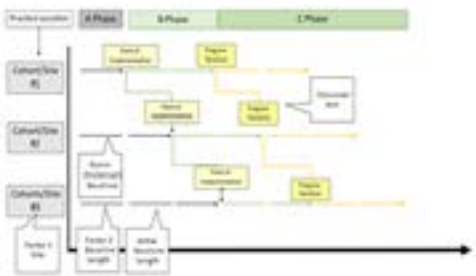
The objective of this presentation is to bring a best practice framework to the evaluation of pharmaceutical products for rare disease. This presentation covers 3 approaches, discuss general principles for when and how they should be applied, and hat limitations should be considered when developing methods. The presentation discusses the following:

1. N of 1 Trials. Trials designed, conducted, and evaluated at the level of the individual patient [8].

1. Adaptive Research Design. Trial allowing for prospective modifications based on accumulating data feedback on trial subjects [9, 10].

2. Multiple Baseline. Staggers the baseline length and onset of intervention with repeated measures across treatment conditions (each consecutive individual serves as both control and treatment) [11].

A fictionalized 3 X 2 X 2 mixed model factorial design (see Figure) example is presented to illustrate. Qualifying subjects are initially randomized into one of three varying baseline periods. At the end of baseline (A), patients are randomized a second time to condition (i.e. test article vs. a true placebo/standard of care). In the C Phase, subjects cross over to the condition not assigned in Phase B. At predetermined points, interim analyses are conducted, with a priori decision rules. The final phase is "open label" and offered only if the final analysis is supportive.



e: steve@individuallytics.com