

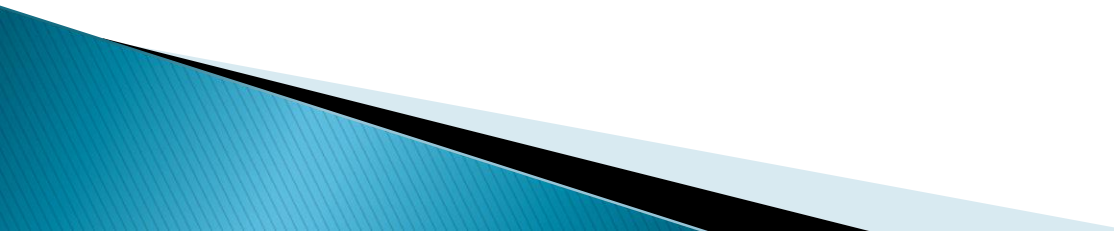
**Increasing the efficiency and
success of drug re-purposing**

**Utilising multiple-compound
study designs in rare diseases**

Joanna Segieth PhD, MTOPRA

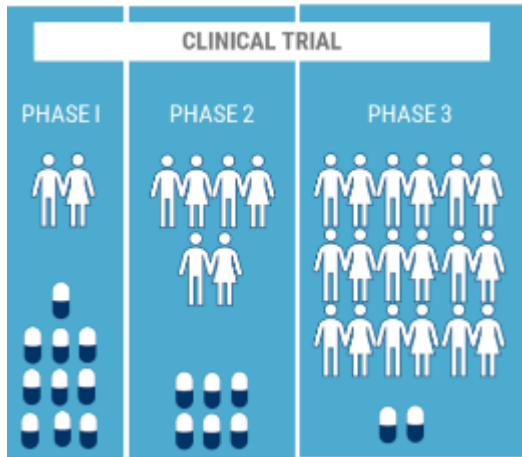


Platform Development

- Experimental infrastructure to evaluate multiple treatments and/or combination of treatments in heterogeneous patient populations
 - The concept of multiple-compound studies is usually applied to diseases with biomarkers e.g., oncology
- 

Innovative Designs

Typical Design



Multi-Arm Design



Basket Design

Single Treatment

Single DA

One Question

Multiple Treatments

Single DA

Series of Questions

Single Treatment

Multiple DA or Subtypes

Series of Questions

Drug Re-purposing

- ▶ Why might the platform approach be of interest in the area of unmet medical need and drug re-purposing?
 - Rapid comparison of existing therapies
 - Reduces clinical testing cycle
 - Reduces the required number of patients for testing
 - Rapid delivery of effective therapies

Success



Connect

Collaborate

Cure

We will Connect and Collaborate
as we all work towards a Cure

Thank you !