

Clinical Development 301: Phase II/III

55-MINUTE ONLINE COURSE | LEVEL 3

SUGGESTED PREREQUISITES: CLINICAL DEVELOPMENT 101, CLINICAL DEVELOPMENT 201

OVERVIEW

Clinical Development 301: Phase II/III considers the purpose, design, and conduct of Phase II and III clinical trials. Learn the various trial design approaches, endpoint choices, statistical considerations, and special regulatory designations.

Five Takeaways:

1. Key differences between early stage (Phase I) and late-stage (Phase II/III) clinical trials.
2. Regulatory significance of clinical endpoint, primary endpoint, secondary endpoint, and surrogate endpoint.
3. Fluency in Phase II and Phase III clinical trial nuances.
4. Basic statistical analysis completed in late-stage trials.
5. Description of specialized and expedited development cycles for rare disease, orphan drugs, and therapies for unmet medical needs.

AGENDA

- **Phase II/III Introduction** identifies the principle elements of a well-controlled Phase I/II clinical trial with an in-depth look at study design, endpoints, and statistical analysis. The concepts of a null hypothesis, p-value, type 1 error, type 2 error, power, variability, and treatment size effects are explained.
- **Phase II/III Objective and Design** compares the general characteristics of Phase II and Phase III clinical trials. Defines pivotal study, adaptive trial, basket trial, and umbrella trial. Discusses the function and types of recommendations the Data Safety Monitoring Boards provides.
- **Phase II/III Special Designations** focuses on rare disease and serious unmet medical need designations in both the US and Europe. Explains the challenges with clinical studies associated with rare disease and provides examples of flexible clinical development approaches for application to rare diseases. Lastly, the relationship between clinical trial endpoints and approved labeling claims are explained.