Course Syllabus

Visiting Professor: David Wypij

Course	Biostatistics: Principles of Clinical Trials		
Credit	1	Method of Teaching	Lecture

Outline (subject to change)

This short course is designed to introduce basic principles in the design, conduct, and analysis of clinical trials including scientific and management aspects. Topics include trial design (different study designs, the study cohort, study treatments, treatment allocation methods, study outcomes, and sample size calculations), trial documents (protocols, statistical analysis plans, data monitoring plan, consent documents, trial registration, case report forms, and manual of operations), and data analysis (interim trial monitoring, adverse events, final data analyses, subgroup analyses, multiple comparisons, handling of missing data, and manuscript preparation). Use of the SPIRIT guidelines for protocol development and the CONSORT guidelines for trial manuscript preparation will be an important component of the course. The course should be of benefit to statisticians and quantitatively-oriented clinicians interested in working on clinical trials. Background in basic biostatistics (comparisons of two groups, hypothesis testing, confidence intervals) and regression methods (linear and logistic regression) is strongly recommended.

Class Schedule (90 minutes each) (subject to change)

Day 1 (January 6, 2025)

1. **Introduction to Clinical Trials (Overview, basics of drug development, basic study design, ethics)** (9:00-10:30)
2. **Protocol Development (Study objectives, study outcomes, surrogate and composite endpoints, repeated measurements, study population, SPIRIT guidelines)** (11:00-12:30)

Day 2 (January 7, 2025)

3. **Treatment Allocation and Blinding (Randomization methods, blocking, stratification, adaptive randomization, types of blinding)** (9.00-10.30 am)
4. **Sample Size Considerations (Type I error and power, adjustment for multiple comparisons, nonadherence, noncompliance, and dropouts)** (11:00-12:30)

Day 3 (January 8, 2025)

5. **Statistical Analysis Plan and Operational Issues (Multiple endpoints and hypotheses, data analysis plan, unadjusted vs. adjusted analyses, exploratory analyses, case report forms, manuals of operation)** (9:00-10:30am)

6. **Study Monitoring and Interim Analyses (Data Monitoring Committees, adverse events and safety data, outcome data, early study stopping, group sequential methods, conditional power)** (11:00-12:30)

Day 4 (January 9, 2025)

7. **Example of a Clinical Trial from Start to Finish, including the CONSORT guidelines** (9:00-10:30)

8. **Modern Developments (Novel designs, personalized medicine, and the future of clinical trials)** (11:00-12:30)

Written Exam (January 9, 2025): (13.30-15.00pm)

We may add seminars by Japanese teachers for each to assist students with difficulty in language/background knowledge

Recommended Text (subject to change)

Fundamentals of Clinical Trials, Fifth Edition, Lawrence M. Friedman, Curt D. Furberg, David L. DeMets, David M. Reboussin, Christopher B. Granger, Springer, 2015.

Clinical Trials: A Methodologic Perspective, Third Edition, Steven Piantadosi, Wiley, 2017.

Related readings

Handout of lecture slides will be made available prior to the lecture.

Achievement evaluation (subject to change)

There will be a written final exam about the course contents scheduled in the class upon completion of the course.