

## Comparative Effectiveness and Patient-Centered Outcomes Research

### University of Cincinnati (UC) Course Director:

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**Class Time:** Wednesday 1-2:50 pm, Kettering 121

**Office Hours:** By appointment, please contact Dr. Huang, Bowers, and/or Altaye

### Course Objectives:

- To gain knowledges of current recommendations for the best practices in CER and PCOR
- To recognize strengths and limitations of common study designs for conducting comparative effectiveness research (CER)
- To acquire knowledge of the different statistical methodologies and recognize their strengths and limitations common in CER
- To learn qualitative study designs that can be applied to studies in CER and patient-centered outcomes research (PCOR)
- To effectively critique and summarize relevant literature
- To understand the use of available data sources and their strength and limitations for CER studies
- To describe patient-relevant outcomes for PCOR studies
- To understand the importance of subgroup analysis and identification in PCOR studies

### Class format:

The first hour of class will consist of a presentation by either the course directors or guest lecturers from Cincinnati Children's Hospital Medical Center, the University of Cincinnati, or other institutions in the region. The second 50 minutes will be a discussion of a paper which utilizes the methods discussed in class that day. The discussion will be co-led by one to two students (who also turn in their written critical appraisal report on that day) and guided by our expert discussant (the presenter from the first hour).

### Course Requirements:

1. Readings: All readings are made available electronically through blackboard at UC. No textbook is required. All materials will be available via blackboard.
2. Homework: A robust discussion is fundamental to learning in this class. Therefore, to facilitate discussion, students are asked to prepare at least one question or point of discussion based on the class topic and reading for that class.
3. Class Participation: To receive full credit for class participation, it is necessary to **verbally** participate in discussion. We recognize this can be challenging for some students and appreciate any effort students put forth towards discussion. Students should contact the course directors regarding missed classes.
4. Student Presentation of Journal Article: At the beginning of the course, each student will choose two pre-selected articles for journal club presentation during the manuscript discussion portion of the class. Students can use the Critical Appraisal form (end of this document) as a template (as a guide as not all points are relevant to all articles) and provide the instructor a written appraisal about the article the day of discussion. Each appraisal will be worth 30% of the student's grade.

### Course Grade:

Discussion question for each class	10%
Face-to-face class participation	30%
Critical Appraisals (30% each)	60%

**Academic Ethics:** All students in this course will be expected to conduct themselves with complete integrity, in accordance with the respective student codes of conduct put forth by each university. All work by the student will be the work of that student, unless otherwise referenced. All work presented and shared by students will be the considered their work and not used by another student unless a collaboration is determined.

## Class Schedule

Journal articles not listed will be posted on blackboard prior to class!

No	Date	Topic	Lecturer	Journal Article for Class
<b>Part I: CER/PCOR Introduction</b>				
1	8/28	Historical perspective and causal modeling in CER and PCOR	Bin Huang/ Katherine Bowers	<p>Reading for first class:  <a href="https://www.nytimes.com/2018/08/20/upshot/medical-treatments-compared-important-everyday-questions.html">https://www.nytimes.com/2018/08/20/upshot/medical-treatments-compared-important-everyday-questions.html</a></p> <p>Meyer A-M, Wheeler SB, Weinberger M, et al. An Overview of Methods for Comparative Effectiveness Research. Seminars in Radiation Oncology 2014; 24: 5–13</p> <p>Reference for course:  <a href="https://www.pcori.org/sites/default/files/PCORI-Methodology-Standards.pdf">https://www.pcori.org/sites/default/files/PCORI-Methodology-Standards.pdf</a></p>
<b>Part II: Study Designs and Data Sources</b>				
2	9/4	Randomized Controlled Trials and Pragmatic Clinical Trials	Mekibib Altaye	Intramuscular versus intravenous therapy for prehospital status epilepticus. NEJM; 366 (7); 2012
3	9/11	Conventional and emerging methods in the design of trials –	Changchun Xie	Adaptive Randomization of Veliparib-Carboplatin Treatment in Breast Cancer. NEJM; 375 (1); 2016
4	9/18	Observational studies for CER using administrative data Part I	Katherine Bowers	Comparative Effectiveness of High-Dose Versus Standard-Dose Influenza Vaccines Among US Medicare Beneficiaries in Preventing Postinfluenza Deaths During 2012–2013 and 2013–2014; The Journal of infectious Diseases 2017; 215: 510-515.
5	9/25	DAG	Katherine Bowers	TBD
6	10/2	Design considerations in observational studies for CER and PCOR Part II	Maurizio Macaluso	Comparative effectiveness of radical prostatectomy and radiotherapy in prostate cancer: observational study of mortality outcomes. BMJ 2014; 348.
7	10/9	Sources of information: Health Care Claims; HER	Jareen Meinzen-Derr	Leucht et. Al. Comparative efficacy and tolerability of 15 antipsychotic drugs in schizophrenia: a multiple-treatments meta-analysis. Lancet Vol 382; 2013
<b>Part III: Statistical Analysis Considerations of CER/PCOR</b>				
8	10/16	Propensity Scores Theory and Methods	Bin Huang	
9	10/23	Causal Inference methods beyond Propensity Score	Bin Huang	

10	10/30	Methods for Preventing and Handling Missing Data	Nanhua Zhang	Multiple imputation with large data sets: a case study of the Children's Mental Health Initiative. <i>AJE</i> ; 169 (9); 2009
11	11/6	Systematic Reviews and Meta-Analysis	Bin Zhang	Murad M, Montori VM, Ioannidis JA, et al. How to Read a Systematic Review and Meta-analysis and Apply the Results to Patient Care: Users' Guides to the Medical Literature. <i>JAMA</i> . 2014;312(2):171-179. doi:10.1001/jama.2014.5559.
12	11/13	Decision Science Modeling and Analysis	Mark Eckman	Transplanting Hepatitis C Virus–Infected Versus Uninfected Kidneys Into Hepatitis C Virus–Infected Recipients. <i>Annals of Internal Medicine</i> . 169 (4); 2018
13	11/20	Instrumental Variables,	Rhonda Szczesniak	Rassen et al. IV II: IV application-25 variations, the physician prescribing preference generally was strong and reduced covariate imbalance. <i>Journal of Clinical Epidemiology</i> 2009; 62:1233-1241
	11/27	Thanksgiving Break		No reading!
14	12/4	Health Economic Analyses and Cost-Effectiveness	Monir	Cost-effectiveness of Screening for Chronic Hepatitis C Infection in the United States. <i>CID</i> 2013; 56. Eckman et al.
15	12/11	Finals- no class		

## **Approach to Critical Appraisal of Published Medical Research**

- A. Research Hypothesis and Study Design
  - 1. What is the rationale for study (i.e., do the authors use the existing literature to justify the importance or uncertainty of the study)?
  - 2. Is there a clear statement of the hypothesis?
  - 3. What is the study design?
  - 4. Is it appropriate for the hypothesis?
- B. Study Sample
  - 4. How many subjects were included?
  - 5. Were recruitment efforts and inclusion / exclusion criteria described?
  - 6. Were informed consent and human subjects protections described?
- C. Outcome Variable
  - 7. What criteria are used to define the presence of disease (case definition, time period)?
  - 8. Is the determination of the presence or absence of disease accurate?
- D. Methods of Analysis
  - 9. Are the statistical methods employed suitable for the types of variables (categorical vs ordinal vs continuous) in the study?
  - 10. Have the levels of type I and type II errors been discussed appropriately?
  - 11. Is the sample size adequate to answer the research question?
  - 12. Have the assumptions underlying the statistical tests been met?
  - 13. Has chance been evaluated as a potential explanation of the results?
- E. Exposure Variable(s)
  - 14. How many exposures or risk factors are being studied?
  - 15. How is the presence or absence of exposure determined?
  - 16. Is the assessment of exposure likely to be precise and accurate?
  - 17. Is there an attempt to quantify the amount and duration of exposure?
  - 18. Are biologic markers of exposure used in the study?
- F. Possible Sources of Bias
  - 19. Is the method of selection of subjects likely to have biased the results?
  - 20. Is the measurement of either the exposure or the disease likely to be biased or misclassified?
  - 21. Have the investigators considered whether confounders could account for the observed results?
  - 22. In what direction would each potential bias influence the results?
- G. Interpretation of Results
  - 23. How large is the observed effect? (Is this a meaningful effect?)
  - 24. Is there evidence of a dose-response relationship?
  - 25. Are the findings consistent with laboratory methods?
  - 26. Did they examine the possibility of effect modification?
  - 27. Are the effects biologically plausible?
  - 28. If the findings are negative, was there sufficient statistical power to detect the effect?
- H. How can study results be used?
  - 29. Are the findings consistent with other studies of the same question?
  - 30. Can the findings be generalized to other human populations?
  - 31. Do the findings warrant a change in current practice?
  - 32. If further research is warranted, how would you proceed?
- I. Other details:
  - 33. Is this a peer-reviewed journal? How reputable is the journal?
  - 34. Are the findings timely? When was the article submitted? Accepted?