

M19-5252 Spring 2017
Comparative Effectiveness Research

Course Description

This course will provide a comprehensive introduction to comparative effectiveness research (CER). Topics include an overview of CER, stakeholder engagement in CER, designing CER studies, methodologic challenges in doing CER, and recent developments in PCORI and Federal policy. Students will be expected to review and evaluate comparative effectiveness studies as well as actively participate in class discussions. Nearly every class session will include a case study. Through examples, class discussion and homework students will become familiar with the methods for Comparative Effectiveness Research. They will prepare a set of specific aims for a CER project and identify gaps in CER in their field of study.

Course Activities:

Lectures and discussions, reading assignments, critique studies, in-class case-studies.

All registered students are expected to attend the PCOR symposium on April 26 held at WUSM in the Eric P. Neuman Educational Center (EPNEC). <http://pcor.wustl.edu/PCOR-Symposium/2017-Symposium>.

Evaluation:

Evaluation will be based on class participation (20%), a journal article critique (30%), and a final presentation and paper (50%). All students are expected to come prepared to actively participate in class discussions and demonstrate their knowledge of CER as well as complete in-class case studies. Details on the journal article critique and final paper/presentation will be handed out on the first day of class and posted on blackboard.

Competencies:

1. Define CER and its basic tenets.
2. Understand the broader context of CER
3. Identify common methodologies used in CER
4. Be able to identify the appropriate research design for the CER question
5. Identify methodologic challenges of CER

Time and Place: J. B. Richmond Teaching Room, 2nd Floor, Taylor Ave Building, Fri 1 to 3 PM

Instructors

Anke Winter, MD, MSc
wintera@wudosis.wustl.edu

Office hours

By appointment

BLACKBOARD

We will use Blackboard to manage our class and access assignment instructions. Blackboard can be accessed at <https://bb.wustl.edu/>. Log in with your WUSTL Key, and the course should appear on the homepage.

Attendance

Class attendance is required. As a courtesy to other students, you are expected to arrive on time. More than two absences from class may result in a lowered grade. Do not enroll if you have more than two absences already planned. The value of the class stems from the quality of the dialog and conversations with peers and course instructors.

Grade

Your grade will be based on:

- Class Participation (20%)
- Journal article critique (30%)
- Class project paper and presentations (50%)

Grading Scale

A+: 97-100; A: 93-96; A-: 90-92; B+: 87-89; B: 83-86; B-: 80-82; C+: 77-79; C: 73-76; C-: 70-72

Policy on Late Assignments

Late assignments will result in a deduction of one grade point (A+ down to A) for each day late (including weekends) unless prior approval is obtained from the instructor or a compelling situation prevents prior approval (i.e. documented health issues or family emergencies).

Date	Topic	Instructor
Jan 20	Overview of the Course and CER	Dr. Anke Winter
Jan 27	Consumer Engagement in CER Trials	Dr. Mary Politi
Feb 3	Designing Prospective Observational CER Studies	Dr. Anke Winter
Feb 10	Overview of Large Databases for Retrospective CER Studies	Dr. Margie Olsen
Feb 17	Stakeholder Engagement Methods	Dr. Aimee James
Feb 24	Patient-Reported Outcome Measures	Dr. Amy McQueen
Mar 3	Effectiveness and Pragmatic Trials	Dr. Anke Winter
Mar 10	Examples and Applications of a Pragmatic Trial in Asthma	Dr. Kaharu (Cajal) Sumino
Mar 17	CER Systematic Reviews & Meta Analyses	Dr. Su-Hsin Chang
Mar 24	Examples and Applications in Thoracic Surgery	Dr. Varun Puri
Mar 31	No class due to symposium	
Apr 7	Patient Centered Outcomes in Anesthesia (PROMIS)	Dr. Michael Avidan
Apr 14	PCOR in Treatment-Resistant Depression in Older Adults	Dr. Eric Lenze
Apr 21	No class due to symposium	
Apr 26	PCOR Symposium 8 am – 4 pm	
Apr 28	Student Presentations	
May 5	Student Presentations	

Class Background Material:

IOM initial priorities for CER

<http://www.iom.edu/Reports/2009/ComparativeEffectivenessResearchPriorities.aspx>

PCORI national priorities (adopted May 21, 2012, latest update August 2014):

<http://www.pcori.org/research-results/research-we-support/national-priorities-and-research-agenda>

<http://www.pcori.org/assets/PCORI-National-Priorities-and-Research-Agenda-2012-05-21-FINAL1.pdf>

PCORI methods standards (latest update Nov 2013):

<http://www.pcori.org/assets/2013/11/PCORI-Methodology-Report.pdf>

Jan 20: Overview of the Course and CER (Dr. Winter)

Clancy C, Collins FS. Patient-Centered Outcomes Research Institute: the intersection of science and health care. *Sci Transl Med* 2010;**2**(37):37cm18.

Fleurence RL, Curtis LH, Califf RM, et al. Launching PCORnet, a national patient-centered clinical research network. *J Am Med Inform Assoc* 2014;**21**(4):578-82.

Gabriel SE, Normand SL. Getting the methods right--the foundation of patient-centered outcomes research. *N Engl J Med* 2012;**367**(9):787-90.

Price-Haywood EG. Clinical Comparative Effectiveness Research Through the Lens of Healthcare Decisionmakers. *Ochsner J* 2015;**15**(2):154-61.

Selby JV, Beal AC, Frank L. The Patient-Centered Outcomes Research Institute (PCORI) national priorities for research and initial research agenda. *JAMA* 2012;**307**(15):1583-4.

Selby JV, Forsythe L, Sox HC. Stakeholder-Driven Comparative Effectiveness Research: An Update From PCORI. *JAMA* 2015;**314**(21):2235-6.

Sox HC. Defining comparative effectiveness research: the importance of getting it right. *Med Care* 2010;**48**(6 Suppl):S7-8.

Methodological Standards and Patient-Centeredness in Comparative Effectiveness Research: The PCORI Perspective (2012). *JAMA*; 307:1636-40

Jan 27: Consumer Engagement in CER Trials (Dr. Politi)

Hoffman A, Montgomery R, Aubry W, Tunis SR (2010). How best to engage patients, doctors, and other stakeholders in designing comparative effectiveness studies. *Health Affairs*, 10, 1834-41.

Daverka, PA, Lavalley, DC, Desai, PJ, Esmail, LC, Ramsey, SD, Veenstra, DL & Tunis, SR (2012). Stakeholder participation in comparative effectiveness research: defining a framework for effective engagement. *Journal of Comparative Effectiveness Research*, 1(2): 181–194.

Brownlee, S. (2002). Bad Science and Breast Cancer
<http://discovermagazine.com/2002/aug/featbad#.Uozz-tK-1Bk>

PCORI resources:

<http://www.pcori.org/funding-opportunities/what-we-mean-engagement>

<http://www.pcori.org/assets/2013/11/PCORI-Sample-Engagement-Plans.pdf>

<http://www.pcori.org/sites/default/files/PCORI-Compensation-Framework-for-Engaged-Research-Partners.pdf>

Feb 3: Designing Prospective Observational CER Studies (Dr. Winter)

Austin PC. An Introduction to Propensity Score Methods for Reducing the Effects of Confounding in Observational Studies. *Multivariate Behav Res* 2011;**46**(3):399-424.

Berger ML, Mamdani M, Atkins D, et al. Good research practices for comparative effectiveness research: defining, reporting and interpreting nonrandomized studies of treatment effects using secondary data sources: the ISPOR Good Research Practices for Retrospective Database Analysis Task Force Report--Part I. *Value Health* 2009;**12**(8):1044-52.

Concato J, Lawler EV, Lew RA, et al. Observational methods in comparative effectiveness research. *Am J Med* 2010;**123**(12 Suppl 1):e16-23.

Dreyer NA, Tunis SR, Berger M, et al. Why observational studies should be among the tools used in comparative effectiveness research. *Health Aff (Millwood)* 2010;**29**(10):1818-25.

Fleurence RL, Naci H, Jansen JP. The critical role of observational evidence in comparative effectiveness research. *Health Aff (Millwood)* 2010;**29**(10):1826-33.

Xian Y, O'Brien EC, Fonarow GC, et al. Patient-Centered Research into Outcomes Stroke Patients Prefer and Effectiveness Research: Implementing the patient-driven research paradigm to aid decision making in stroke care. *Am Heart J* 2015;**170**(1):36-45, 45 e1-11.

Feb 10: Overview of Large Databases for Retrospective CER Studies (Dr. Olsen)

Henk HJ, Li X, Becker LK, et al. Comparative effectiveness of colony-stimulating factors in febrile neutropenia prophylaxis: how results are affected by research design. *J Comp Eff Res* 2015;**4**(1):37-50.

Mi X, Hammill BG, Curtis LH, et al. Impact of immortal person-time and time scale in comparative effectiveness research for medical devices: a case for implantable cardioverter-defibrillators. *J Clin Epidemiol* 2013;**66**(8 Suppl):S138-44.

Naeim A, Henk HJ, Becker L, et al. Pegfilgrastim prophylaxis is associated with a lower risk of hospitalization of cancer patients than filgrastim prophylaxis: a retrospective United States claims analysis of granulocyte colony-stimulating factors (G-CSF). *BMC Cancer* 2013;**13**:11.

Weintraub WS, Grau-Sepulveda MV, Weiss JM, et al. Comparative effectiveness of revascularization strategies. *N Engl J Med* 2012;**366**(16):1467-76.

Feb 17: Stakeholder Engagement Methods (Dr. James)

Burke JG, Jones J, Yonas M, et al. PCOR, CER, and CBPR: alphabet soup or complementary fields of health research? *Clin Transl Sci* 2013;**6**(6):493-6.

Domecq JP, Prutsky G, Elraiyah T, et al. Patient engagement in research: a systematic review. BMC Health Serv Res 2014;14:89.

Forsythe LP, Ellis LE, Edmundson L, et al. Patient and Stakeholder Engagement in the PCORI Pilot Projects: Description and Lessons Learned. J Gen Intern Med 2016;31(1):13-21.

Robbins M, Tuftte J, Hsu C. Learning to "Swim" with the Experts: Experiences of Two Patient Co-Investigators for a Project Funded by the Patient-Centered Outcomes Research Institute. Perm J 2016;20(2):85-8.

Feb 24: Patient-Reported Outcomes Measures (Dr. McQueen)

Basch E. Patient-Reported Outcomes - Harnessing Patients' Voices to Improve Clinical Care. N Engl J Med 2017;376(2):105-08.

Bingham CO, 3rd, Bartlett SJ, Merkel PA, et al. Using patient-reported outcomes and PROMIS in research and clinical applications: experiences from the PCORI pilot projects. Qual Life Res 2016;25(8):2109-16.

Francis DO, Patel DA, Sharda R, et al. Patient-Reported Outcome Measures Related to Laryngopharyngeal Reflux: A Systematic Review of Instrument Development and Validation. Otolaryngol Head Neck Surg 2016;155(6):923-35.

Patient-Reported Outcomes Measurement Information System (PROMIS).
<http://www.healthmeasures.net/explore-measurement-systems/promis>.

Mar 3: Effectiveness and Pragmatic Trials (Dr. Winter)

Chalkidou K, Tunis S, Whicher D, et al. The role for pragmatic randomized controlled trials (pRCTs) in comparative effectiveness research. Clin Trials 2012;9(4):436-46.

Ezenwa MO, Suarez ML, Carrasco JD, et al. Implementing the PAINRelievelt Randomized Controlled Trial in Hospice Care: Mechanisms for Success and Meeting PCORI Methodology Standards. West J Nurs Res 2016.

Frobert O, Lagerqvist B, Olivecrona GK, et al. Thrombus aspiration during ST-segment elevation myocardial infarction. N Engl J Med 2013;369(17):1587-97.

Lauer MS. Commentary: How the debate about comparative effectiveness research should impact the future of clinical trials. Stat Med 2012;31(25):3051-3.

Loudon K, Treweek S, Sullivan F, et al. The PRECIS-2 tool: designing trials that are fit for purpose. *BMJ* 2015;**350**:h2147.

Mar 10: Examples and Applications of Pragmatic Trials in Asthma (Dr. Sumino)

Mar 17: CER Systematic Reviews and Meta-Analysis (Dr. Chang)

Brouwers MC, Thabane, L, Moher, D & Straus, SE (2012). Comparative Effectiveness Research Paradigm: Implications for Systematic Reviews and Clinical Practice Guidelines. *Journal of Clinical Oncology*, 30(34):4202-7.

AHRQ Methods guide for Effectiveness and Comparative Effectiveness Reviews:

<http://effectivehealthcare.ahrq.gov/search-for-guides-reviews-and-reports/?pageaction=displayproduct&mp=1&productID=318>

AHRQ also has a slide library for training for systematic reviews with their focus on comparative effectiveness: <http://www.effectivehealthcare.ahrq.gov/index.cfm/tools-and-resources/slide-library/#slidetrainingmodules>

Mar 24: Examples and Applications in Thoracic Surgery (Dr. Puri)

Mar 31: No class due to PCOR Symposium

Apr 7: Patient Centered Outcomes in Anesthesia (Dr. Avidan)

Apr 14: PCOR in Treatment-Resistant Depression in Older Adults (Dr. Lenze)

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April 26: PCOR symposium (8am-4pm)

April 28: Student Presentations

May 5: Student Presentations