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Comparative and Cost-Effectiveness Research: Competencies, Opportunities and Training for Nurse Scientists

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Background

Currently, the United States is spending almost 18 percent of the gross domestic product (GDP) on healthcare; and economists and actuaries from the Centers for Medicare and Medicaid projected healthcare spending to rise on average 5.6 percent per year between 2016 and 2025 to 19.9 percent of the GDP by 2025 (Keehan et al., 2017). This is far more than any other developed country and the United States population has poorer health outcomes and higher health risk factors (OECD). Therefore, achieving high value has become an overarching goal and a necessity with value defined as the health outcomes achieved per dollar spent (Porter, 2010). Furthermore, irrespective of any changes in national health policy, the projected cost pressures will likely be associated with patients, clinicians, payers and health policy makers continuing to seek innovative strategies to increase value.

One reason for the problem in optimizing healthcare value is the lack of comparative clinical data on the effectiveness and costs of treatments and care delivery models. The United States research community, including nurse scientists, has an unprecedented opportunity to inform decisions and improve the nation's health system, the health of the population, as well as increase value through comparative and cost-effectiveness research (Lauer & Collins, 2010; Pincus, 2011; United States. Dept. of Health and Human Services., 2009).

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The purposes of this article are to: 1) describe the overall goals of comparative and cost-effectiveness research and the unique contribution nurse-scientists may make; 2) identify needed competencies for comparative and cost-effectiveness research; 3) identify federal funding for comparative and cost-effectiveness research; and, 4) describe current training opportunities. This information should be useful to educators interested in including comparative and cost-effectiveness research methods in their PhD programs as well as the next generation of nurse scientists (i.e., predoctoral students, postdoctoral fellows and mid-career nurse scientists) that would like to be trained in these methods.

Goals of Comparative and Cost-effectiveness Research

Comparative effectiveness research is an interdisciplinary field of inquiry that develops knowledge on the effectiveness of various interventions to inform decisions about healthcare delivery and value (Jacobson, 2007). Specifically, comparative effectiveness research is a translational science that has been defined as, “the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in real world settings”(United States. Dept. of Health and Human Services., 2009); and, its purpose is to improve health outcomes by developing and disseminating evidence-based information about the effectiveness of interventions (Iglehart, 2009; United States. Dept. of Health and Human Services., 2009; Volpp & Das, 2009). This is in contrast to efficacy research where the question is typically whether the treatment can work under a controlled environment (Greenfield & Kaplan, 2012).

Because comparative effectiveness research aims to inform actual clinical situations, it is much more patient-centered. Indeed, there is overlap between the terms patient-centered outcomes research and comparative effectiveness research. Both of these research paradigms focus on the everyday needs of and outcomes of concern to patients in making healthcare decisions.

The Patient Centered Outcomes Research Institute (PCORI) is an independent nonprofit organization that was authorized by Congress in 2010 (<http://www.pcori.org/>). PCORI's mandate is to improve the quality and relevance of evidence available to help patients, caregivers, clinicians, employers, insurers, and policy makers to make informed health decisions. For example, PCORI has funded researchers at the University of Rochester to analyze how telehealth can impact participants' well-being, access to care and system efficiency; this has led to publication that outlines the issues that health systems should consider when making decisions about adoption of telehealth and a discussion of the current disincentives due lack of reimbursement (Dorsey & Topol, 2016). Furthermore, PCORI supports work that will improve the methods used to conduct comparative effectiveness studies and emphasizes the importance of including patients and other stakeholders' values through engagement in the entire research process (i.e., from developing the questions to disseminating the results).

Cost-effectiveness research is part of a comprehensive comparative effectiveness evaluation (Roberts, 2016). Specifically, cost-effectiveness analysis is a set of economic evaluation

tools designed to compare relative costs and effectiveness of two or more comparable healthcare interventions (Frick & Stone, 2009; Garber, 2011; Jacobson, 2007). While cost-effectiveness research evaluates both cost and effectiveness, it has sometimes been equated with rationing and therefore is associated with some degree of political controversy (Rich, 2012). Indeed, this is why PCORI is mandated by the Congress not to fund cost-effectiveness research (Pincus, 2011). However, leading scientists recognize the rigor and applicability of well-conducted cost-effectiveness analyses; for example, in a recent NIH Director's Blog, Dr. Francis Collins discussed the importance of results from a NIH funded cost-effectiveness analysis in informing clinical decision making and health policy (Collins, 2016). Furthermore, the Council for the Advancement of Nursing Science Idea Festival Advisory Committee identified health economics as an emerging area of nursing science to be incorporated in nursing PhD programs because of its importance to achieving high value care (Henly, McCarthy, Wyman, Alt-White, et al., 2015; Henly, McCarthy, Wyman, Heitkemper, et al., 2015; Henly, McCarthy, Wyman, Stone, et al., 2015). Last, the National Institute of Nursing Research (NINR) leadership recognizes that innovative study designs and analytic techniques are needed to identify, "cost-effective, sustainable wellness interventions across communities and populations"(NINR, 2016).

Pincus (2009) discusses six roles and tasks of clinical and translational researchers, which are applicable to nurse scientists conducting comparative and/or cost-effectiveness research (Pincus, 2009). First, the core professional function of any clinical/translational researcher is to conduct research that enhances the health of the individual, community, nation and/or world. Second, this research takes an intellectual orientation with creative and disciplined thinking. Third, as with any research there is a technical skill that includes systematic and objective investigation. Fourth, management skills are needed to organize and efficiently conduct the research. Fifth, values and integrity are necessary for the scholarly process to adhere to the highest standards of ethical conduct. Sixth, understanding interdisciplinary perspectives is necessary with collaboration across disciplines. These roles and tasks are well-matched to the mission of the NINR, which is to promote and improve the health of individuals, families and communities (NINR, 2016). Indeed, with the clinical relevance of nursing science, nurses are well suited to lead clinical comparative and cost-effectiveness studies and/or partner with researchers from other disciplines. However, for nurse scientists to do this effectively, not only is discipline-specific training essential, interdisciplinary training in comparative and cost-effectiveness research is necessary (Hastings-Tolsma, Matthews, Nelson, & Schmiede, 2013).

Competencies

To determine the requisite competencies needed for nurse scientists to conduct comparative and cost-effectiveness research we first reviewed comparative effectiveness curricular foci and competencies put forward by two Clinical Translation Science Awards and two Institute of Medicine (now known as the National Academy of Science) expert work groups (Kroenke et al., 2010; Subcommittee, 2009; United States. Dept. of Health and Human Services., 2009; Workgroup, 2009). We chose to review the recommendations of these four groups because the membership are scientific leaders from across the nation. At least three of the four expert groups identified clinical epidemiology (including practical clinical trials

and systematic reviews), biomedical informatics, health services research, biostatistics for comparative effectiveness research, and health economics as necessary curriculum foci for comparative effectiveness research. We further reviewed published core competencies for health economics, health services research and comparative effectiveness research (Forrest, Martin, Holve, & Millman, 2009; Platt, Kwasky, & Spetz, 2016; Segal et al., 2012; Stone, Smaldone, & Lucero, 2011). Based on the review of published competencies and the recommended core curricula, we identified 14 core competencies for comparative and cost-effectiveness research for nursing scientists, which are outlined in Table 1. These competencies have both analytic and theoretical foci from clinical epidemiology, biomedical informatics, health services research, biostatistics, health economics, communication and dissemination, and nursing.

Level of Interest in Comparative and Cost-effectiveness Research: Synthesis of Current Funding

To identify the current level of federal funding spent on comparative and cost-effectiveness research, we conducted a National Institute of Health (NIH) RePORTER query as well reviewed the PCORI website. NIH RePORTER (<https://report.nih.gov/index.aspx>) is a public repository that allows users to search for NIH funded projects as well as and other federally funded projects. In November 2016, we submitted a query that included a text search using the key terms “comparative and cost-effectiveness”. We searched for awards funded by NIH, the Agency for Healthcare Research and Quality (AHRQ) and the Veterans Administration (VA). We excluded other funding institutes such as the Food and Drug Administration and the Centers for Disease Control and Prevention, because they are not primary funders for most nursing scientists nor comparative and cost-effectiveness research. We selected “active projects”. Data exported included: the agency or NIH institute that administers the award; the funding activity type, which we categorized into training (i.e., K, F and T awards) or research (i.e., R, P, U and other); and, total fiscal year cost per project. We calculated the median annual costs for training and research awards as well as the total annual costs.

Results of the NIH RePORTER query are displayed in Table 2. At the time of the query, NIH had funded over 1,800 projects meeting our criteria for a total annual cost of \$935,169,541. The majority (88%) were research awards with the median annual cost just over \$450,000. A subset of these awards (n = 34) were from NINR, representing an almost \$18 million investment in this research. Only three of NINR’s T32 grants met our inclusion criteria (i.e., Advanced Training in Nursing Outcomes Research, T32NR007104, Pennsylvania University; Training in Behavioral Nursing, Indiana University, T32NR007066; and, Comparative and Cost-Effectiveness Research Training for Nurse Scientists (CER²), T32NR014205, Columbia University). Almost half (38 of 79, 48%) of AHRQ awards were training grants. The VA did not list training grants and 14 grants were identified. Neither the VA nor AHRQ reports award amounts in the NIH RePORTER repository, therefore median annual costs could not be calculated.

PCORI posts its annual report on its website (<http://www.pcori.org/sites/default/files/PCORI-Annual-Report-2015.pdf>). For FY 2015, PCORI spent \$214,304,095 on research and engagement awards (i.e., engagement in research, as defined by PCORI, refers to meaningful involvement of stakeholders including patients, caregivers, clinicians and others) and budgeted \$331,526,300 for this in 2016. Thirty-five awards were made in the Spring and Winter of 2015, with the awarded amount totaling \$192,259,139 (median award amount \$2,461,861). Of note, these are total award amounts not annual award amounts as with the NIH awards, so the numbers are not directly comparable. The credentials of the principal investigators was not available through the PCORI website, but a web-based search of each of the 35 principal investigators was conducted and none of these investigators listed nursing credentials on their primary institution websites. However, some of the topics funded would certainly be of interest to some nursing scientists (e.g., “Enhancing Patient Ability to Understand and Utilize Complex Information Concerning Medication Self-Management” and “Using a Teachable Moment Communication Process to Improve Outcomes of Quitline Referrals”). Furthermore, others have been able to identify nurse scientists leading PCORI-funded research (Barksdale, Newhouse, & Miller, 2014). Indeed, PCORI has shown interest in nursing science and a representative presented on the 2016 Council of Nursing Science “Funding Panel”(CANS, 2016).

Nurse Training in Comparative and Cost-effectiveness Research

There are multiple ways for both faculty and graduate students to be trained in comparative and cost-effectiveness research methods. Faculty may consider applying for a K08 Mentored Clinical Scientist Research Career Development Award (see: <https://researchtraining.nih.gov/programs/career-development>). Additionally, many universities offer certificates in comparative effectiveness research.

Graduate students and postdoctoral fellows may choose to apply for positions through the three NINR funded T32s or apply for individual funding through any of NIH or AHRQ’s many mechanisms. To be eligible for Columbia University School of Nursing program (T32NR014205) predoctoral students must be matriculated in school’s PhD program. Postdoctoral fellows must be a recent PhD graduate (within 3 years of defense of dissertation) from any accredited university who is also a registered nurse. As with all federally funded T32s, the trainees must be U.S. citizens, non-citizen U.S. nationals, or those lawfully admitted for permanent residence; all trainees are provided a stipend set by NIH, tuition benefits and funds for other training related expenses such as health insurance and/or travel to a relevant conference.

The Columbia training program has been developed to provide trainees with skillsets to meet the competencies listed in Table 1. The training includes four key activities: 1) didactic graduate courses, 2) weekly seminars, 3) a supervised research experience, and 4) career development and grant writing workshops. Predoctoral students are expected to select their dissertation topic addressing comparative and cost effectiveness research in high-risk, underserved populations; all trainees are expected to produce at least one publishable comparative or cost-effectiveness research related paper prior to program completion.

In addition, all trainees are required to have an interdisciplinary mentoring team that includes one interdisciplinary faculty preceptor and one nurse scientist preceptor. Key considerations for matching a faculty mentoring team with a trainee include the complementarity of the trainee's background and interest as well as support available through the mentors. Each mentoring team will *at least* have one preceptor with a successful history of mentoring and one preceptor with a funded program of research of interest to the trainee. The trainee will be embedded within this funded program of research and participate in ongoing interdisciplinary comparative or cost-effectiveness research activities. It is expected that each trainee meet with at least one of the faculty on the mentoring team on a weekly basis and have a joint mentoring meeting with both faculty members at least once per semester, or more often as needed. Using an interdisciplinary mentoring team exposes the trainee to interdisciplinary science. Using a mentoring team also allows for an optimal mix of faculty with successful history and expertise of mentoring pre- and/or post-doctoral students and relevant methodological expertise. Furthermore, it allows for a mix of senior and junior faculty, which is appropriate faculty development.

The Columbia University training program is fairly new and small with only 4 pre- and 2 postdoctoral fellows to date. Table 3 presents the fellows level (pre or postdoctoral), the years of fellowship, the title of their project, the fit with NINR's strategic focus, the expertise of the nursing mentor and the interdisciplinary mentor, other collaborations/projects, productivity measured by the number of peer reviewed published manuscripts, submitted manuscripts and presentations, and positions post fellowship, as applicable. Five of the six fellows identify their project as synergistic with NINR's "Wellness: Promoting Health and Preventing Disease" strategic aim (NINR, 2016). The interdisciplinary mentors have a variety of expertise including informatics, health economics, behavioral health and outcomes research. The fellows have been very productive with 0 to 9 manuscripts published, 0 to 5 submitted manuscripts and 3 to 10 presentations. Those that have completed their fellowship have gone on to research-intensive positions and have obtained further federal funding.

Conclusion

Comparative and cost-effectiveness research has the opportunity to transform healthcare delivery and improve the outcomes of patients. Nurses, as clinicians and scientists, are in a unique position to contribute to this important research. Resources are available to nurses who are seeking this training both through NIH and AHRQ. We encourage interested nurse graduate students, postdoctoral fellows and faculty members to seek the needed interdisciplinary research training needed to participate in this important endeavor. We also encourage nurse educators to use the competencies and processes identified to help shape their doctoral programs.

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Highlights

- Nurses have an opportunity to transform healthcare with comparative and cost-effectiveness research
- Fourteen core competencies were identified that have both analytic and theoretical foci from nursing and other fields.
- There are multiple sources of federal funding for research and training.
- Interdisciplinary training is needed.

Table 1**Comparative Clinical Effectiveness Research Competencies for Nurse Scientists**

		Curricular Focus	
Competency		Analytic	Theory
1.	Demonstrate breadth of comparative clinical research theoretical and conceptual knowledge by applying alternative models from a range of relevant disciplines including clinical epidemiology, biomedical informatics, health services research, biostatistics and health economics.		C, I, H, B, E
2.	Apply in-depth nursing disciplinary knowledge and skills relevant to comparative clinical effectiveness research.	C, I, H, B, E, N	C, I, H, B, E, N
3.	Apply knowledge of the structures, performance, quality, policy, and environmental context of health and health care to formulate value nursing solutions for health policy problems.	C, I, H, B, E	N
4.	Pose innovative and important comparative clinical effectiveness research questions informed by systematic reviews of the literature, stakeholder needs, and relevant theoretical and conceptual models to improve population health.	C, I, H, B, E	C, I, H, B, E, N
5.	Select appropriate interventional, observational, or qualitative study designs to address specific comparative clinical effectiveness research questions.	C, I, H, B, E	N
6.	Know how to collect primary health outcome and health care utilization data obtained by survey, qualitative or mixed methods.	C, I, H, B, E	
7.	Know how to assemble and access secondary data from existing public and private sources.	C, I, H, B, E	
8.	Use conceptual models and operational measures to specify study constructs for comparative clinical effectiveness research questions and develop variables that reliably and validly measure these constructs.		C, I, H, B, E, N
9.	Implement comparative clinical effectiveness research protocols with standardized procedures that ensure reproducibility of the science.	C, I, H, B, E, N	
10.	Ensure the ethical and responsible conduct of research in the design, implementation, and dissemination of comparative clinical effectiveness research.	C, I, H, B, E	N, D
11.	Work collaboratively in multidisciplinary teams.	C, I, H, B, E, N	
12.	Use appropriate analytical methods in comparative clinical effectiveness research to clarify associations between variables and to delineate causal inferences.	C, I, H, B, E	
13.	Effectively communicate the findings and implications of comparative clinical effectiveness research through multiple modalities to technical and lay audiences.	D	
14.	Understand the importance of collaborating with stakeholders, such as policymakers, organizations, and communities to plan, conduct and translate comparative clinical effectiveness research into policy and practice.	D	

C = clinical epidemiology, I = informatics, H = health services research, B = biostatistics in comparative effectiveness research and E = health economics, N = nursing, D = communication and dissemination

Table 2
Active Funded Projects of Comparative-Effectiveness or Cost-Effectiveness Research

Funder	Total awards	Total annual institute cost	Training awards	Annual median cost per award	Research awards	Annual median cost per award
NIH	1832	935,169,541	218	170,753	1614	457,605
NINR	34	17,859,775	3	314,712	31	529,667
AHRQ	79	NA	38	NA	41	NA
VA	14	NA	0	NA	14	NA

NIH = All National Institutes of Health, NINR = National Institute of Nursing Research, AHRQ = Agency for Healthcare Research and Quality, VA = Veterans Administration, NA = not available

Table 3

Examples of Comparative Clinical Effectiveness Training

Trainee	Predoctoral					Postdoctoral	
	I	II	III	IV	V	VI	
Year(s) of Fellowship	3	1	1	2	2	1	
CER ² Dissertation/Project Title	Evaluation of a school nurse-led intervention for children with severe obesity in New York City schools	Assessing the Potential of a Diabetes Self-Management Technology Intervention for Underserved Adults	Comparative Effectiveness of WPATH Standards of Care in Promoting Optimal Health Outcomes among Transgender People	Nurse Practitioner-Physician Co-management of Primary Care Patient Panels: Impact, Perspective, and Measurement toward a New Delivery Care Model	Economic evidence for mobile health (mHealth)	Cost-Effectiveness of Infection Prevention and Control Policies of Nursing Homes	
Fit with NINR Strategic Plan	W	S	W	W	W, S	W	
Expertise of Interdisciplinary Mentor	H, E	I, D	C, H	B, H	I, C	H	
Published Manuscripts	5	2	0	2	9	1	
Submitted Manuscripts	2	2	0	5	4	1	
Presentations	10	6	3	5	10	7	
Role Post Fellowship	NRSA Postdoctoral Fellow, University of Pennsylvania	Not Applicable	Not Applicable	Not Applicable	Tenure-track Assistant Professor, University of Washington School of Nursing	Health Policy Research Scientist, RAND, Corporation	

CER² = Comparative and Cost-effectiveness Training for Nurse Scientists

NINR Strategic Plan Codes: W = Wellness; Promoting Health and Preventing Illness; S = Self-management: Improving Quality of Life for Individuals with Chronic Conditions

Expertise Codes: C = clinical epidemiology, I = informatics, H = health services research, B = biostatistics in comparative effectiveness research and E = health economics, D = communication and dissemination