

**AMERICAN COLLEGE OF RHEUMATOLOGY
POSITION STATEMENT**

SUBJECT: Comparative Effectiveness Research:

PRESENTED BY: Committee on Government Affairs

**FOR DISTRIBUTION TO: Members of the American College of Rheumatology
Agency for Healthcare Research and Quality
National Institute of Health
Medical Societies
Arthritis Foundation**

BACKGROUND:

1 Comparative Effectiveness Research, a government sponsored program to evaluate the safety
2 and effectiveness of a given medical treatment or service relative to other treatments for the same
3 condition, is now being implemented by the U.S. government.
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5 Largely an artifact of the well meaning and fundamental change in clinical research mandated by
6 the Food and Drug Agency as it developed a much more systematic process for drug and medical
7 treatment approval beginning in the 1960's. Placebo controlled trials became the norm for most
8 clinical trials except in those instances when ethical considerations required placebo equivalents
9 in the form of existing treatments. This more rigorous paradigm, followed by government and
10 commercially sponsored researchers alike, has resulted in a legacy of many drugs and treatments
11 that "are better than nothing" but are largely unmeasured relative to each other. As a result, as
12 the Centers for Medicare and Medicaid Services have repeatedly observed, substantial regional
13 differences exist within the U.S. with regard to how a given disease or condition is treated,
14 nearly always without a cogent rationale, since outcomes tend to be very similar at the
15 population level. The obvious fiscal implications aside, the necessary conditions for physician
16 guidance and patient education will remain unsatisfied until these large gaps in our clinical
17 databases have been remedied.
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19 **POINTS OF CONCERN:**
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21 There are many concerns about the implementation of CER initiative if it is to avoid wasting
22 valuable time and resources on a massive scale. Among them are the limitations inherent to
23 CER itself, budget considerations, and the structure and purpose of those entities charged with
24 CER oversight.
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26 With regard to the first, CER, as it is currently envisioned must take one of two forms (or both):
27 head-to-head trials and/or literature-based analyses (or, where appropriate, meta analyses of
28 existing trials). The former, while generally accepted as more methodologically sound, will
29 doubtless require substantial amounts of time and money to address even a fraction of the
30 possible comparisons between drugs, drugs and non drug treatments and between non drug
31 treatments, e.g. surgical techniques. And even having carried out such trials, there is, of course,

32 no guarantee that the results will be valid. The scientific clinical literature is replete with large
33 well-funded trials that have failed to achieve their outcome objectives, not because of problems
34 with the underlying drug or treatment, but because of unanticipated flaws in the trial design or
35 the methods used to conduct the study. In other words, some very good unanswered clinical
36 questions may remain unanswered despite generous amount of money and CER resources.
37 Similarly, though generally quicker, literature-based comparisons suffer from numerous potential
38 methodological pitfalls. To mitigate issues with the quality and probability of success of the
39 research enterprise, CER researchers and those who choose them must, therefore, possess great
40 research skills and experience, and be free of conflict of interest. In sum, the foregoing suggest
41 CER must realistically be expected to be applied to a very limited number of comparisons and
42 without expectations that the results will drive decision making in the near term.

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44 The second large practical problem with CER as currently planned is financial. All summaries
45 and reports of the CER initiative note the comparatively small amount of funding previously
46 dedicated to it. The current stimulus package provides \$1.1 billion in funding. While seemingly
47 a large increase, when compared to the magnitude of needed research, the idea that any
48 organization expected to provide meaningful additions to accepted clinical treatment pathways
49 must be tempered by what can be accomplished with these sums. Again, clinical trials,
50 especially the head-to-head sort, are expensive, time-consuming and may require replication to
51 validate their results and/or to extend those results to various sub-populations.

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53 Finally, expectations of CER must be viewed with uncertainty until all of the details of CER
54 execution have been worked out. At present, a Federal Coordinating Council is charged with
55 providing oversight to coordinate all research efforts, to avoid duplication of research and to
56 provide recommendations to Congress for further resources and infrastructure. To allay patient
57 and physician-driven concerns that CER results will not be used to make coverage or
58 reimbursement decisions, the FCC-CER has been specifically prohibited from doing so. How
59 effective that prohibition will be and whether or not payers will independently use CER results
60 for the same purpose remains to be seen. In simple terms, if the noble cause of defining the best
61 treatment for a given condition is to enjoy widespread political support, it must not be subsumed
62 by those who would use it to ration care or drive clinical decision making toward in abstentia
63 health care.

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65 **POSITION:**

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67 In summary, a determined effort by our government to fund CER serves the lofty purpose of
68 enabling patients to receive the best known treatment for whatever ails them. The ACR must,
69 therefore, strongly endorse this effort. Opportunities to study key comparisons within our own
70 realm of expertise that would greatly enhance the healthcare of very large segments of our
71 population abound. Expertise to conduct such studies is also to be found within our
72 organization. With proper funding and protection from inappropriate use of the resulting data,
73 the quality of rheumatologic healthcare could be vastly enhanced. However, all concerned must
74 be cautioned that sound research will most likely take more time and resource than was
75 envisioned by those who framed the creating legislation and not be frustrated by overly
76 optimistic expectations.

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79 Approved by Government Affairs Committee: September 2009
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81 Approved by Board of Directors: October 2009