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What Does "Evidence-based Research" Mean?
Limitations in Our Current Approach

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Disclaimers

- I truly respect and admire epidemiologists and biostatisticians.
- I Do NOT believe RCTs should be abandoned in their entirety.
- I am a strong proponent of quality improvement in health care.

Why Do We Need Evidence-based Research?

- There is a well-documented crisis in the quality and equity of health care in America.
- There are too many factors that influence reaching a desired health outcome.
- Our society watches too much TV.

Why Do We Need Evidence-based Research?

- If the outcome obtained is not always related to the quality of care delivered, process controls (guidelines) are needed.
- Guidelines require evidence.
- Evidence is often - although not always - generated through research.

So Why Am I Here?

- Fairly strict and inflexible hierarchies for evidence have been established.
- These hierarchies generally ignore crucial components in medical decision-making and technology assessment, such as patient preferences and costs.
- Application of the evidence (as currently defined) may not result in improvement of health care quality.

Guideline Example

Pressure Ulcer Prevention and Treatment Following Spinal Cord Injury

Available through the National Guideline Clearinghouse
www.guideline.gov

Hierarchy of Scientific Evidence

I. Large randomized trials with clear-cut results (and low risk of error).

II. Small randomized trials with uncertain results (and moderate to high risk of error).

III. Nonrandomized trials with concurrent or contemporaneous controls.

IV. Nonrandomized trials with historical. controls

V. Case series with no controls.

www.guideline.gov/summary/summary.aspx?ss=15&doc_id=2589&nbr=1815&string=#s22

Strength of Evidence Associated with Each Recommendation

A. The recommendation is supported by scientific evidence from properly designed and implemented controlled trials providing statistical results that consistently support the guidelines statement.

B. The recommendation is supported by scientific evidence from properly designed and implemented clinical series that support the guidelines statement.

C. The recommendation is supported by expert opinion.

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Common Theme of Evidence Hierarchies

- The Randomized Controlled Trial (RCT) is the “Gold Standard” of evidence.

But…

- RCTs are great at proving efficacy.
  - Efficacy = “Capacity or power to produce a desired effect.”
- Observational studies may be better at proving effectiveness.
  - Effectiveness = “The quality of being able to bring about an effect.”

(www.wordreference.com)

Limitations of RCTs

- **R**estrictive inclusion / exclusion criteria limits generalizability.
- **C**onflict between results observed in "controlled" experiment versus use in "real" life.
- **T**oo expensive.
- **S**ingle intervention, single-dose (although increasing use of factorial designs).

More Limitations of RCTs

- Ethical considerations.
- Bias in what interventions are studied and what studies are published.
- Translation of large sample-derived risks and outcomes to individual patients.
- Patient preferences.

Still More Limitations of RCTs

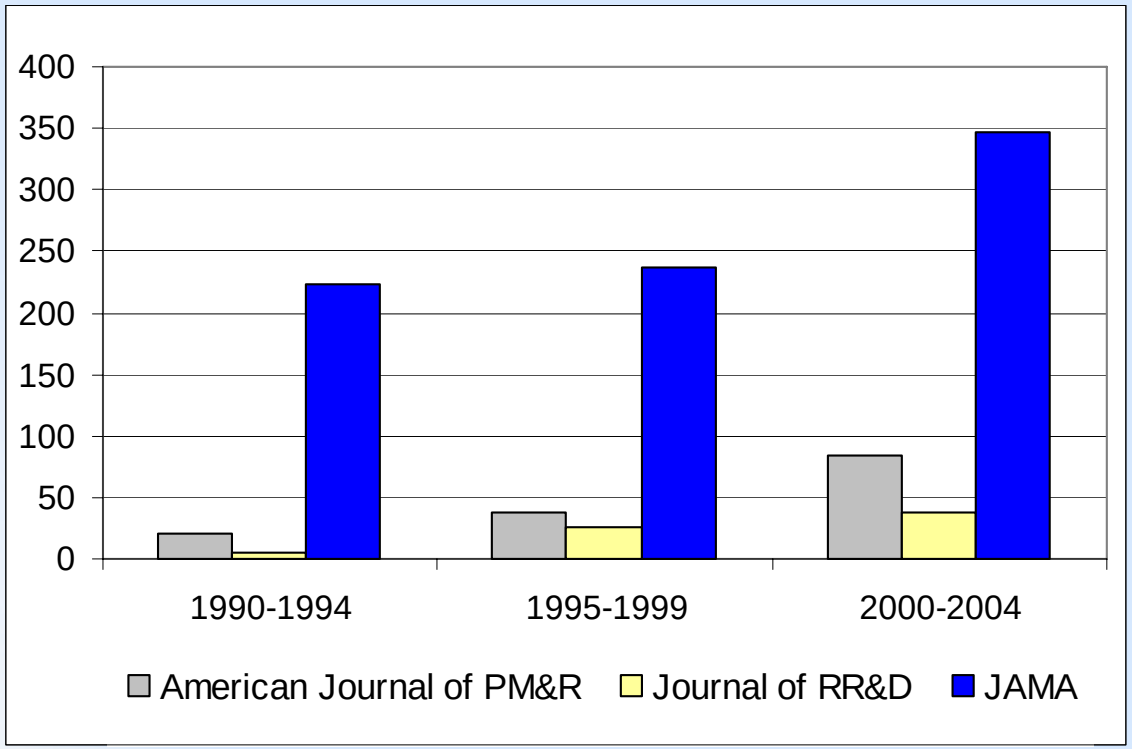
- Short-term focus may miss medium and long-term side effects.
- Short expiration date when treatment is rapidly evolving.

The Current Reality of RCTs

"A randomized clinical trial (RCT) is currently the strongest method for evaluating interventions in clinical practice. RCTs also provide the politically most powerful form of evidence."

Wade DT (2005) "Randomized Clinical Trials in Clinical Rehabilitation", Clinical Rehabilitation, 19(3): 233-6.

Number of RCTs Published



Back to Our Guideline Example

- 32 recommendations are made.
- The recommendations cover:
  - Prevention
  - Nutrition
  - Assessment Following Onset
  - Treatment
  - Complications
  - Support Surfaces and Positioning
- All of the recommendations resulted from “Strong” expert panel consensus after systematic review of the evidence.

As a Reminder…
Hierarchy of Scientific Evidence

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More from our Guideline Example

- Of the 32 recommendations:
  - Only 8 included Level I evidence.
  - 7 included only Level V evidence.
  - 2 included no “evidence”, except expert opinion.
  - 22 included evidence from more than one level.
  - The most common level of evidence cited is level V (26 times).
- And yet, all of the recommendations resulted in “Strong” expert panel consensus.

What Happened to the Evidence Hierarchy?

- Large randomized trials are expensive and RCTs, in general, can be difficult to implement in the field of physical medicine and rehabilitation.

Solutions? Alternatives?

- RCTs with factorial designs.
- N=1 trials.
- Well-designed observational studies.
- Better post-market surveillance.

In Conclusion…

- Our current approach to evidence-based research is not creating strong pillars to lift and support quality improvement in health care (in general) or wound care (specifically).

A New Approach to Evidence-based Research is Required to Improve the Quality of Health Care

- Greater focus on the “Preponderance of the Evidence” from various, well-designed study models (triangulation) and less focus on hierarchies.

- Greater focus on patient-centered health care (both processes and outcomes).