Comparative Effectiveness Research

Opportunities in older persons with multiple conditions

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Comparative Effectiveness Research

“To improve the quality, effectiveness and efficiency of health care delivered through Medicare, Medicaid and the S-CHIP programs.” (per MMA)

Premise

Since the majority of Medicare beneficiaries have at least two co-existing conditions, CER must address multimorbidity
Background

The average 80 year old person has 3-4 chronic diseases PLUS 3 health related conditions, such as insomnia, pain and loss of appetite.

Most of the patients with any common index condition HAVE multiple coexisting conditions (eg DM, CHF, COPD, OA)

Among persons aged 70 and older, 60% take 5-9 medications and 20% take over 10 medications

Persons aged 65 and older who have 2 or more conditions consume about 80% of Medicare costs
The Challenge

Older persons with multiple diseases and conditions receive the most medications, and use the most health resources BUT...

We have almost no evidence base for the effectiveness (and harms) of their care
Why is there so little evidence?

Older adults with multiple conditions are generally excluded from clinical trials.

Even when research studies do include older persons with multiple conditions,
outcome assessments prioritize a focused set of diseases
The monitored spectrum of other treatment effects may be limited
Representation: Age distribution of common conditions and age of trial participants

<table>
<thead>
<tr>
<th>disease</th>
<th>% disease in population 65+</th>
<th>% 65+ in trials</th>
<th>Data source</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cancer</td>
<td>61%</td>
<td>25%</td>
<td>Lewis JCO 2003</td>
</tr>
<tr>
<td>Surgery for stress UI</td>
<td>50%+</td>
<td>&lt;10%</td>
<td>More AN Ob Gyn 2004</td>
</tr>
<tr>
<td>AMI</td>
<td>40% (75+)</td>
<td>10%</td>
<td>Lee PY JAMA 2001</td>
</tr>
<tr>
<td>Parkinsons</td>
<td>50% (75+)</td>
<td>6%</td>
<td>Mitchell S Arch Neurol 1997</td>
</tr>
<tr>
<td>Dementia</td>
<td>80%+ (75+)</td>
<td>&lt;50%</td>
<td>Gill SS Can J Clin Pharm 2004</td>
</tr>
</tbody>
</table>
What is the best treatment for patients with multimorbidity?

Dilemma

Treatments have not been systematically assessed in the patient with other conditions.

Treatments that benefit an index condition may be harmful for other conditions (e.g., CHF and renal insufficiency, androgen blockade and osteoporosis).
Solution

• Multimorbidity as a focus of comparative effectiveness research
An Agenda for Comparative Effectiveness Research in older adults with multimorbidity

General principles

Approach to defining multimorbidity
  Disease pairs
  Multiple diseases

Methodological issues

Priority areas
General Principles

What to compare? Eg “usual care”, disease guideline care, treatment intensity

Include benefits and harms (net benefit vs. harm)

Identify key subgroups (by total disease burden)
- rationale: Affects rate and magnitude of benefit and harm
- analysis: Can stratify to examine benefit and harm

Define, assess and compare universal outcomes across treatments
What is a universal outcome?

Final common pathway affected by ALL disease-specific outcomes
Valued by patients and families
Can be assessed across all diseases and conditions
Can use to compare treatments
Universal Outcomes

Symptoms: dyspnea, fatigue, anorexia, pain
Impairments: physical and cognitive performance, wasting/weight loss
Function: disability, restricted activity

Health Care Use: hospitalization, services, caregiving

Active Life Expectancy

<table>
<thead>
<tr>
<th>Age</th>
<th>Active</th>
<th>Impaired</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Age 85</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>women</td>
<td>2.3</td>
<td>4.1</td>
</tr>
<tr>
<td>men</td>
<td>2.6</td>
<td>2.8</td>
</tr>
<tr>
<td><strong>Age 65</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>women</td>
<td>2.5</td>
<td>13.6</td>
</tr>
<tr>
<td>men</td>
<td>11.9</td>
<td>2.5</td>
</tr>
</tbody>
</table>

Remaining Years of Life
How to define multiple morbidity?

Common Disease Pairs

- **Pairs with risk of therapeutic competition** (treatment of one disease may worsen another, e.g., hypertension and osteoporosis)

- **Goal of CER**: Determine optimal treatment target by balanced benefit to both elements of disease pair plus universal outcomes
HTN + osteop

↓↓BP

meds

↓BP

CV (MI, stroke, etc.); FX (hip, other fracture); Universal (fatigue, weakness, physical performance, ADLs, death)
The case of multiple coexisting diseases

Compare set of disease guidelines or usual care with…

Innovative models of care (e.g. chronic care model, self management, medical home)

Priority-driven treatment algorithms

Single intervention that benefits multiple diseases (e.g. exercise for BP, CAD, CHF, sarcopenia)
Example: Guided care (Boult)

- RN performs standard comprehensive assessment of diseases, function, etc.
- Collaboration among 1º MD, RN, patient, care givers →
  - Care guide for provider
  - Action plan for patient, family
- RN coordinates care across providers and transitions, monitors chronic conditions
Vs. disease-guideline driven care

- Each specialist prescribes according to evidence-based disease guideline
- Patient expected to adhere
- No coordination across providers, transitions
- Focus on disease-specific outcomes
Comparative effectiveness: Guided care vs. disease-guideline care

• Compare:
  – universal outcomes (symptoms, function, survival)
  – Health care utilization and costs
  – Patient and care giver satisfaction
  – Adverse treatment effects
Priority-driven treatment algorithms

• Priority-driven care:
  Older adults with multi-morbidity differ in universal outcome of greatest priority

  Able to map patients’ disease-specific priorities (e.g. stroke, MI, COPD) unto universal outcomes (e.g. symptoms, function, survival)

  Care focused on maximixing outcome of greatest priority to the individual
Priority-driven treatment algorithms

• **Step 1**: Ascertain outcome priorities

• **Step 2**: Determine which condition(s) most affecting outcome

• **Step 3**: Of these, which most amenable to intervention

• **Step 4**: Implement treatment strategy based on Step 3
Comparative effectiveness: Priority algorithm vs. disease-guideline care

• Compare:
  % participants who met their outcome priority
Health care utilization and costs
Patient and care giver satisfaction
Adverse treatment effects
Universal outcomes
Methodologic issues in CER for older adults with multimorbidity

Observational or RCT
Requires innovative methodologies due to
- Heterogeneity
- Multiple outcomes
- Universal plus disease-specific outcomes
- Varying 1º outcomes if driven by patient priorities
Methodologic issues

• Samples:
  Large, representative, multiple conditions
  Can use ongoing large, longitudinal national samples eg MCBS
  Might use electronic health records but key universal variables such as function often not included (?Some Medicare HMOS, VA)
Methodologic issues

Data:
- Baseline descriptive and prognostic (for risk stratified subgroups)
- Disease-specific and universal outcomes (longitudinal)
- Treatment characteristics
Priorities in CER for older adults with multimorbidity

- Key subgroups – total morbidity burden
- Key outcome(s) – disease-specific plus universal (symptoms, function, survival)
- Key comparisons
  - Treatment intensity
  - Treatment sequences eg behavioral followed by or combined with drugs
  - Coordination models vs. disease-guideline
SUMMARY
Comparative effectiveness for older adults with multimorbidity

Complex multimorbid health states have been avoided in research due to methodological challenges.

The bulk of care is provided to older persons with complex multimorbid health states.

Novel approaches to study design, variables and interventions can increase the feasibility and yield of research in this population.