Challenges and implications of patient reported clinical outcomes for randomised controlled trials

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Overview

- Clinical outcomes important component of RCTs
  - complement patient reported outcomes
  - collected by clinical exam, lab results or case notes
  - self-reporting by participants increasingly used

- Four case studies where patient reported clinical outcomes have been collected

- Verified using different clinical sources and compared
Case Studies

- UK NIHR Health Technology Assessment programme
- UK Medical Research Council
Case study 1

- Evaluate three variants of total knee replacement
  - average 10 years follow-up
  - questionnaires issued on an annual basis
  - report knee-related hospital re-admissions

- Verified using hospital notes & central records
  - comparisons revealed some inconsistencies
### Re-admission reporting

<table>
<thead>
<tr>
<th>Questionnaire</th>
<th>Routine data</th>
<th>Y</th>
<th>N</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Y</td>
<td>169 (71%)</td>
<td>38 (16%)</td>
</tr>
<tr>
<td></td>
<td>N</td>
<td>31 (13%)</td>
<td>? (?%)</td>
</tr>
</tbody>
</table>

**Total hospital re-admissions - 238**
### Re-operation reporting

<table>
<thead>
<tr>
<th>Questionnaire</th>
<th>Routine data</th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Y</td>
<td>Y</td>
<td>62</td>
<td>(74%)</td>
</tr>
<tr>
<td></td>
<td>N</td>
<td>7</td>
<td>(8%)</td>
</tr>
<tr>
<td>N</td>
<td>Y</td>
<td>15</td>
<td>(18%)</td>
</tr>
<tr>
<td></td>
<td>N</td>
<td>?</td>
<td>(?%)</td>
</tr>
</tbody>
</table>

**Total number of re-operations - 84**
Conclusions

- **Patient reported outcomes only:**
  13% of hospital readmissions not reported
  18% of re-operations not reported

- **Central records used only:**
  16% of hospital readmissions not reported
  8% of re-operations not reported

- **Use of both datasets helped us better evaluate the key clinical questions**
Case study 2

- Three different types of urethral catheters
  - symptomatic urinary tract infections (UTIs)
  - postal questionnaires completed
  - report incidence of UTI and antibiotic use

- Antibiotic use verified with the GP
  - GP reported antibiotic use for non-responders
Antibiotic use

- 830 participants (13%) reported a UTI
- 435 participants (52%) were prescribed an antibiotic for a UTI
- 209 GPs contacted for non-responders
- 45 participants confirmed as having received an antibiotic for a UTI
Conclusions

- If GP data had not been collected: UTIs would have been over-reported. 9% (45/480 confirmed UTIs) not collected.

- Patient reported data allowed focus of resources on those who may have had a UTI.

- Verification of patient reported clinical outcomes allowed the more accurate reporting of the primary outcome.
Case study 3

- Gastro-oesophageal reflux disease
  - laparoscopic surgery vs medical management
  - questionnaires issued on an annual basis
  - report hospital admissions for reflux symptoms

- All potential ‘repeat operation’ cases were verified
  - lead surgeon contacted to review medical notes
Repeat operation

- Lead surgeon was contacted about 19 patients

- Three patients (16%) confirmed as not having repeat surgery

- Type of surgery classified for the remaining 16 patients
Conclusions

- Patient reported outcomes only:
  3 patients would have been wrongly classified
- Overestimated the failure rate of initial operation
Case study 4

- Prevention of secondary fractures
  - Vitamin D3 and calcium, alone and in combination
  - postal questionnaires completed
  - report new low-trauma fractures

- Reports also collected from ‘best contacts’, GPs or hospital staff/study nurses
### Reporting source

<table>
<thead>
<tr>
<th>Participant/Best contact</th>
<th>Y</th>
<th>N</th>
</tr>
</thead>
<tbody>
<tr>
<td>Health professional</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Y</td>
<td>37 (5%)</td>
<td>162 (23%)</td>
</tr>
<tr>
<td>N</td>
<td>413 (59%)</td>
<td>86 (12%)</td>
</tr>
</tbody>
</table>

Total number of fractures - 698
Conclusions

- Patient reported outcomes only:
  64% (450/698) fractures reported

- Reports from non-clinical sources effective strategy

- Supplementing data derived from a variety of clinical professionals resulted in a more complete dataset
Overall conclusions

- Obtaining clinical data from patients important
  - potential inaccuracies associated with this strategy
  - consider verification with clinical sources
  - implications in terms of staff time and costs

- No verification of the absence of a clinical outcome
  - impossible to quantify under reporting
Thank you

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