Development of a multi-attribute tool for setting priorities for trials in the musculoskeletal field:
A project of the Australia and New Zealand Musculoskeletal (ANZMUSC) Clinical Trial Network

Will Taylor, Rachelle Buchbinder, on behalf of the ANZMUSC Clinical Trials Network
Background

• ANZMUSC Clinical Trials Network needs a reliable and transparent method for determining which research questions are the most important
Typical approach to research prioritisation

Directly identify the research topics

Stakeholder engagement

Generate lists of research questions or topics and review research gaps

Choices usually implicit

Identify the ‘top ten’ research topics

- No understanding of why priorities were ranked in the order that they were
- Participant bias
- Need to repeat the process when new topics arise
Another approach

- Directly identify the research topics
- Generate lists of research questions or topics and review research gaps
- Identify the criteria on which to base a judgement of research importance
- Multi-attribute criteria approach
- Identify the ‘top ten’ research topics
- Choices made explicit
- Stakeholder engagement
- Identify the underlying basis for research question value
Aim

To develop a multi-attribute priority setting tool for the explicit and transparent ranking of musculoskeletal research questions.
Methods

Scoping review
- To identify the range of potential criteria and methodology suggested by others

Delphi survey
- To identify what ANZMUSC members think are potential criteria

Consensus workshop
- To agree on a manageable set of criteria

Weighting workshop
- To assign meaningful numeric scores to each criteria

Research question ranking exercise
- To help illustrate the variation of research question ranking

Generation of initial research questions by membership survey and documents review

List of research questions that are ranked by their importance
1 Scoping review of priority-setting of research topics for musculoskeletal conditions

See poster


49 included priority-setting studies
– only 2 specified any criteria, both for physiotherapy
2 Delphi survey to identify determinants of research question importance (online, RedCap)

**Round 1:** ANZMUSC members (N=178) invited to nominate factors that make a research question really important.
- Items of similar meaning merged, duplicates removed, wording improved [1 researcher, checked by 3 other researchers, iterative]

**Round 2:** ANZMUSC members (N=185) invited to indicate agreement (1=strongly disagree to 9=strongly agree) with importance of each item in final list

**Rounds 3, 4:** Respondents (N=68) re-rated ‘uncertain’ items (median rating of 4 to 6) & items with significant disagreement (RAND/UCLA disagreement index >1)
Results: Delphi survey

- Up to 71 respondents (66, 71, 54) depending upon round (~40%)
- Included 5 consumers, 1 funder, 12-23 clinicians, 31-44 researchers
- 347 possible determinants of research question importance
- Reduced to 47 items
- 32 items with median ratings of at least 7 (possible range 1 through 9)
- Grouped under five themes
<table>
<thead>
<tr>
<th>Nature of the condition (36)</th>
<th>Nature of the intervention (50)</th>
<th>Potential for impact (66)</th>
<th>Broad appeal (56)</th>
<th>Project is able to deliver (21)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Condition has a high patient and social burden (8)</td>
<td>Extent to which the intervention could prevent disease or disease progression (8)</td>
<td>Addresses large evidence-practice gap (8)</td>
<td>Agreed importance for funders, consumers and providers (7)</td>
<td>The extent to which the question can be robustly answered (testable) (7)</td>
</tr>
<tr>
<td>Condition has few effective treatments (7)</td>
<td>Intervention is easily and widely implementable (7)</td>
<td>Results have potential for fundamental shifts in understanding (8)</td>
<td>Extent to which the question is important to consumers (7)</td>
<td>Study is highly likely to be completed (7)</td>
</tr>
<tr>
<td>Highly prevalent condition (7)</td>
<td>Tests interventions that are easily and widely accessible to consumers (7)</td>
<td>Results likely to lead to real world changes in clinical care (8)</td>
<td>Results are likely to influence government policy (7)</td>
<td>Study will lead to a definitive answer to the question (7)</td>
</tr>
<tr>
<td>Area of research with little prior work or where prior work is not definitive (7)</td>
<td>Tests intervention in clinical use that have questionable or unknown benefit (7)</td>
<td>Results likely to lead to cost-savings for consumers and/or the healthcare system (7)</td>
<td>Results likely to advance knowledge in other fields (7)</td>
<td></td>
</tr>
<tr>
<td>Condition is costly (7)</td>
<td>Addresses the safety of an intervention (7)</td>
<td>Tests interventions with likelihood of significant benefit (7)</td>
<td>Extent to which the question is important to clinicians, consumers and funders (7)</td>
<td></td>
</tr>
<tr>
<td>Test of interventions as they are delivered in real clinical practice (7)</td>
<td>Addresses timing of delivery and best combinations of interventions (7)</td>
<td>Results have potential for the cure of a health condition (7)</td>
<td>Extent to which the question is important to consumers and clinicians (7)</td>
<td></td>
</tr>
<tr>
<td>Study is able to identify the most responsive subgroups (7)</td>
<td></td>
<td></td>
<td>Question is a priority for policy-makers and funders (7)</td>
<td></td>
</tr>
<tr>
<td>Results likely to improve treatment access, especially equity of access (7)</td>
<td></td>
<td></td>
<td>Extent to which the question is important to clinicians (7)</td>
<td></td>
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<tr>
<td>Advances methods for improving implementation into practice and adherence (7)</td>
<td></td>
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</table>
3 Consensus workshop: criteria for what makes a research question important

- ANZMUSC members submit research questions, varying in importance
- 30/227 selected providing range from not very important to important
- Prior to workshop, invitees ranked questions in order of importance
- Facilitated discussion on what makes a research question important using results of the ranking exercise
- Small group activities to develop key attributes, definitions and categories that describe underlying basis of question importance
- Facilitated discussion and voting for alternative descriptions of the dimensions and categories
Consensus workshop participants

• 4 consumers or consumer advocates
• 3 research funders or health insurance provider
• 15 clinicians and/or researchers: 2 GPs, 4 rheumatologists, 1 orthopaedic surgeon, 2 sport and exercise medicine physicians, 3 physiotherapists, 1 chiropractor, 1 biostatistician, 1 epidemiologist
Ranking of 30 questions by respondents vs importance by proposer
Reasons for importance ranking

More highly ranked questions
• high patient burden of disease and prevalence
• identification of patients most likely to benefit from an intervention
• widely used interventions that have a poor evidence base

More lowly ranked questions
• animal model studies
• testing interventions known to be ineffective or known to be effective
• testing interventions with little prospect of scalability or uptake
• diagnostic research not linked to patient outcomes or benefit

Feasibility and study design generally considered should be evaluated separately
Draft attributes of research question importance

1. Extent to which the question is important to patients and other health decision-makers
2. Addresses an area of high patient burden
3. Addresses an area of high social burden
4. Potential reduction in patient and/or social burden due to (clinical or implementation) intervention
5. Potential scalability and uptake of intervention
6. Extent to which the question addresses health equity
Addresses an area of high patient burden

<table>
<thead>
<tr>
<th>Category</th>
<th>Descriptor/definition of the category</th>
</tr>
</thead>
<tbody>
<tr>
<td>2A Low</td>
<td>Mild symptoms and little or no associated disability</td>
</tr>
<tr>
<td>2B Medium</td>
<td>Moderate symptoms and some disability</td>
</tr>
<tr>
<td>2C High</td>
<td>Significantly disabling, associated with mortality risk or no effective treatments available</td>
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</table>
## Potential scalability and uptake of intervention

<table>
<thead>
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<th>Category</th>
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<tr>
<td>5A Low potential for scalability and uptake</td>
<td>Prohibitive costs to patient or healthcare system, major systems restructure; and substantial behaviour/belief change by clinicians or patients</td>
</tr>
<tr>
<td>5B High potential for uptake but low scalability</td>
<td>Minimal behaviour/belief change by clinicians or patients required but prohibitive costs to healthcare system, major systems restructure</td>
</tr>
<tr>
<td>5C High potential for scalability but low potential for uptake</td>
<td>Immediately feasible with minimal changes required to healthcare system but requires a substantial change in patients/clinicians beliefs or behaviour or has high direct patient costs</td>
</tr>
<tr>
<td>5D High potential for both scalability and uptake</td>
<td>Immediately feasible and minimal behaviour/belief change by clinicians or patients required</td>
</tr>
</tbody>
</table>
Scoping review ▸ Delphi survey ▸ Consensus workshop ▸ Weighting workshop/s

**Research question evaluation**
- To determine reliability and ceiling effects of attribute framework and identify a threshold score for endorsement

**Generation of initial research questions by membership survey and documents review**
- ANZMUSC survey, systematic review of major guidelines across topics, trials in progress and planning, etc.

**List of research questions that are ranked by their importance**
4 Assign numerical scores to each attribute

• 14 research questions from ranking exercise across spectrum of importance

• Add information to enable a judgement on each attribute in the framework (23 categories across 6 attributes)

• 30 raters, web survey using Qualtrics to measure reliability of categorisation, aiming for ICC ≥ 0.75 (95% CI 0.60 to 0.90)

• Assign scores to each criteria and attribute of framework by discrete choice experiment, 1000minds
Weighting of criteria, discrete choice experiment
Further work required

• Can the attribute/category framework be reliably applied to actual research questions?
• Should each attribute/category be valued equally?
• Will there be an unacceptable ceiling effect?
• Where should the threshold be set to identify ‘important enough’ questions?
• Can the final multi-attribute tool be used across different research topics and questions?
Acknowledgements

• Funding: Musculoskeletal Australia and NHMRC
• Denise O’Connor facilitated the consensus workshop
• Workshop participants and ANZMUSC members
## Extent to which the question is important to patients and other health decision-makers

<table>
<thead>
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<th>Category</th>
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</tr>
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<tbody>
<tr>
<td>1A Not shown to be important to either patients or decision-makers</td>
<td>No clinician or relevant healthcare consumer consultation</td>
</tr>
<tr>
<td>1B Shown to be important to health decision-makers but not patients</td>
<td>Relevant healthcare consumers not consulted or do not rate research question highly</td>
</tr>
<tr>
<td>1C Shown to be important to patients but not decision-makers</td>
<td>Relevant healthcare consumers rate research question highly but other health decision makers do not</td>
</tr>
<tr>
<td>1D Shown to be important to both patients and decision-makers</td>
<td>Relevant healthcare consumers and other health decision makers (not researchers only) rate the research question highly</td>
</tr>
</tbody>
</table>
Addresses an area of high social burden

<table>
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</thead>
<tbody>
<tr>
<td>3A Condition is rare</td>
<td>(&lt;0.1% prevalence)</td>
</tr>
<tr>
<td>3B Condition is somewhat common</td>
<td>(0.1 – 1% prevalence)</td>
</tr>
<tr>
<td>3C Condition is common</td>
<td>(1 – 10% prevalence)</td>
</tr>
<tr>
<td>3D Condition is highly prevalent</td>
<td>(&gt;10% prevalence)</td>
</tr>
</tbody>
</table>
Potential reduction in patient and/or social burden due to (clinical or implementation) intervention

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<tr>
<td>4A Symptomatic treatment only and small potential effect size</td>
<td>Intervention has potential to only improve patient symptoms to a modest degree (anticipated effect size &lt;1)</td>
</tr>
<tr>
<td>4B Symptomatic treatment and large potential effect size</td>
<td>Intervention has potential to only improve patient symptoms to a substantial degree (anticipated effect size &gt;1)</td>
</tr>
<tr>
<td>4C Potential for intervention to treat both symptoms and underlying disease pathology</td>
<td>There is a plausible case that some pathophysiological consequences of disease (eg anatomical damage) could be prevented</td>
</tr>
<tr>
<td>4D Potential for cure or fundamental alteration of disease course</td>
<td>There is a plausible case that the disease could be rendered entirely non-active with minimal risk of recurrence, with or without ongoing treatment</td>
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</tbody>
</table>
### Extent to which the question addresses health equity

<table>
<thead>
<tr>
<th>Category</th>
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</tr>
</thead>
<tbody>
<tr>
<td>6A No information</td>
<td>No attempt to address health equity</td>
</tr>
<tr>
<td>6B Not relevant</td>
<td>Discussed using Progress Plus items (O'Neill et al., 2014) but intervention not relevant or appropriate</td>
</tr>
<tr>
<td>6C Somewhat (may have some application to reduce health disparity)</td>
<td>Intervention shown to have some potential application to improving health equity issues</td>
</tr>
<tr>
<td>6D Reducing health disparity is the focus</td>
<td>The intervention is explicitly designed to improve health equity issues</td>
</tr>
</tbody>
</table>
1. Scoping review of priority-setting of research topics for musculoskeletal conditions

**American Physiotherapy Association**: Clinical questions, answerable within 5 years

**Rankin et al 2012**:  
1. Addresses significant need or gap in evidence for physiotherapy practice and/or service delivery  
2. Potential impact of research for quality of care and experience for patients, their carers, service users and members of the public  
3. Potential impact of research for physiotherapy practice  
4. Potential impact of the research for managers, service providers and commissioner/purchasers and relevance to government policy and priorities