How big does the effect of an intervention have to be? Application of two novel methods to determine the smallest worthwhile effect of a fall prevention programme: a study protocol

Marcia Rodrigues Franco,1 Manuela L Ferreira,1 Kirsten Howard,2 Catherine Sherrington,1 John Rose,3 Terry P Haines,4 Paulo Ferreira5

ARTICLE SUMMARY

Article focus
- To determine the smallest worthwhile effect (SWE) of exercise programmes to prevent falls and investigate the relative importance of various factors that influence older people's decision to participate or not in these programmes.
- To determine the extent of trade-offs between harms and benefits that older people are willing to accept in making decisions about participation in exercise programmes to prevent falls.
- To investigate whether the benefit-harm trade-off method and the discrete choice experiment yield similar estimates of the SWE of an exercise programme to prevent falls.

Key messages
- Despite the clear evidence that the rate of falls in older people can be reduced with exercise interventions, there has not yet been any formal evaluation of whether potential recipients of such interventions consider effects of the magnitude observed in randomised trials to be worthwhile to justify the costs and inconveniences they experience in participating.
- Trade-offs between the potential benefits and harms of exercise programmes to prevent falls should be weighed by older people deciding whether to participate or not in these exercise programmes.
- The findings of this study should enable the construction of fall prevention programmes with high participation rates, high levels of adherence and high levels of perceived benefit.

INTRODUCTION

Randomised controlled trials have become the method of choice for determining the effects of health interventions. More than 500 000
SWE of exercise to prevent falls from their own perspective.

The aims of the study are to answer the following questions:
1. What do older people who have previously fallen consider to be the SWE of an exercise-based fall prevention programme?
2. To what extent is the SWE of an exercise-based fall prevention programme influenced by expectations of the costs, risks or inconveniences of intervention?
3. What characteristics of exercise-based fall prevention programmes do older people who have previously fallen value most?
4. Do the benefit-harm trade-off method and discrete choice experiment yield similar estimates of the SWE of an exercise-based fall prevention programme?

METHODS AND ANALYSIS
Overview of approach and methods
Different face-to-face interviews will be conducted for each experiment. The first interview concerns the benefit-harm trade-off method and will be used to address aim 1. The second interview will contribute to the discrete choice experiment and will address aims 1–3. A subsample of 60 participants will participate in both interviews to address aim 4. A systematic review of qualitative studies will inform the development of both designs. The review seeks information on the perceptions and experiences of older people on barriers and facilitators of exercise programmes to prevent falls. Data from this qualitative review will be used to determine the potential attributes for both the benefit-harm trade-off and the discrete choice experiment, such as benefits, costs and inconveniences associated with an exercise-based fall prevention programme.

In the benefit-harm trade-off method, these attributes will have fixed levels and participants will decide the smallest expected benefit of intervention for which he or she would consider choosing to have the intervention. In contrast, in the discrete choice experiment, participants are asked to choose between alternatives defined by a set of attributes with varying levels. In this case, we can identify which attributes are driving participants’ preferences, the trade-offs participants make between attributes and how changes in attributes can lead to changes in the patients’ willingness to participate in the intervention.

Participants
Participants will be recruited via newspaper, radio and online media advertisements as well as through community organisations that target older audiences. To be eligible to participate in both the benefit-harm trade-off study and the discrete choice experiment, study participants must meet the criteria below:
▸ Community-dwelling people aged 60 years or over,
▸ Able to comprehend and read English fluently.
▸ Have experienced one or more falls in the past,
▸ Present no obvious cognitive impairment,
▸ Present no serious neurological, cardiovascular or
  musculoskeletal condition that might hinder their
  participation in exercise program programmes.

Participants will not be excluded based on their par-
  ticipation in exercise programmes (ie, participants who
  are participating and who are not participating in exer-
  cise programmes will be eligible).

**Benefit-harm trade-off method**

For this approach, the interviewer will describe, for each
  participant, the potential benefits as well as the costs
  and risks and inconveniences associated with an
  exercise-based programme to prevent falls. The partici-
  pants will then be told to assume that the treatment
  effect will be of a certain size. Participants will then be
  asked if, given this expected effect of treatment, they
  would choose to have the intervention. Subsequently,
  estimates of the SWE will be elicited by asking the par-
  ticipant to consider the situation in which, hypothetic-
  ally, the expected effect of intervention is larger or
  smaller. The size of the hypothetical benefit will be
  varied up and down in progressively smaller increments
  until it is possible to identify the threshold benefit of
  intervention for which the participant would consider
  choosing to have the intervention. This is the SWE for
  that participant.

**Discrete choice experiment**

The discrete choice experiment will be conducted in a
  way that is consistent with current recommendations.7
  This part of the study will use a survey which includes
  the same attributes associated with an exercise-based
  programme to prevent falls. Participants will be pre-
  sented with multiple choice sets of two hypothetical pro-
  grammes, where the levels of each attribute vary
  systematically between alternatives and scenarios, and
  will choose the optimal alternative in each choice set. As
  it is not feasible to present all participants with all pos-
  sible combinations of attribute levels, each participant
  will be presented with a subset of all possible choice sets.
  An efficient design will be used.8 With this approach,
  the aim is to present participant choice sets that minim-
  ise the elements of the asymptotic variance–covariance
  matrix of the statistical methods used to analyse the
  data, that is, the aim is to maximise the precision of esti-
  mates of the value of attributes.

From participants’ choices, a mathematical function
  that describes numerically the value that respondents
  attach to different choice options will be estimated. This
  is one way to quantify patients’ preferences for healthcare
  programmes. The results will be used to estimate the rela-
  tive value attached to attributes by examining trade-offs
  that people are willing to make between attributes. As a
  consequence, it is possible to determine the probability
  that a person will consider the effects of an exercise-
  based programme to prevent falls worthwhile, given the
  particular costs, risks and inconveniences.

**Table 1** is an example of a discrete choice study.

The example includes nine attributes (costs, transpor-
  tation alternatives, travel time, type of exercise, fre-
  quency, time per day, improvement in the ability to
  undertake daily tasks at home, improvement in the
  ability to leave the house to undertake tasks and to
  socialise and falls risk reduction), with their speci-
  fic levels. Participants would be asked to choose pro-
  gramme 1 or 2.

<table>
<thead>
<tr>
<th>Scenario 1</th>
<th>Exercise option A</th>
<th>Exercise option B</th>
</tr>
</thead>
<tbody>
<tr>
<td>Out of pocket cost ($ per session)</td>
<td>$100</td>
<td>$5</td>
</tr>
<tr>
<td>Is transport provided</td>
<td>No transport is provided; you need to provide your own</td>
<td>No need for transport</td>
</tr>
<tr>
<td>Travel time</td>
<td>About 45 min</td>
<td>5 min or less</td>
</tr>
<tr>
<td>Type of exercise</td>
<td>Combination of different types of exercise (balance and strength training), 1x/week 30 min 10%</td>
<td>Yoga</td>
</tr>
<tr>
<td>How often do you exercise per week?</td>
<td>1x/week 30 min 10%</td>
<td>1x/week 10 min or less 30%</td>
</tr>
<tr>
<td>Time per day</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Improvement in the ease with which you can undertake daily tasks at home (daily tasks at home include bathing, dressing, preparing meals and cleaning the house)</td>
<td>30%</td>
<td>60%</td>
</tr>
<tr>
<td>Improvement in the ability to leave the house to undertake tasks or socialise (Tasks include shopping, banking, walking outdoors, using public transport)</td>
<td>20 of 100</td>
<td>10 of 100</td>
</tr>
<tr>
<td>Falls risk reduction</td>
<td></td>
<td></td>
</tr>
<tr>
<td>On average, 30 of 100 older people fall at least once each year. Exercise can reduce the number of people who fall to...</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Which would you choose? □ Prefer option A □ Prefer option B.
The initial design will be tested with a pilot study including community-dwelling older people living in Australia to assess comprehension and understanding of attributes and their levels. From the findings of this pilot study, we will be able to estimate prior parameters, which will inform the efficient design of the final study. Strategies to facilitate appropriate interpretations of the DCE questionnaire will be used, such as diagrams and plain language. If the final design results in a large number of scenarios, the design will be blocked into two blocks to ensure that respondents are not overly burdened by the number of questions.

**Sample size**

For the benefit-harm trade-off experiment, our simulations indicate that, at least with normally distributed data, a sample size of 60 participants will provide expected CI widths of \( \leq 0.4 \) SDs.

For the discrete choice experiment, the current theory of sampling determines that sample sizes are based upon the characteristics of the design of the study, such as the number of attributes included, the attribute level range, the number of hypothetical scenarios presented and the number of alternatives in each choice set. Consequently, the sample size cannot be determined until the attributes to be included in the final design are identified. An efficient design, which minimises D-error, will be estimated using NGENE software. D-error is a measure of statistical efficiency of the design (lower D-error indicates greater design efficiency). With an efficient choice design, we expect that a sample size of approximately 200 respondents will be sufficient to answer our questions of interest.

**Data analysis**

Benefit-harm trade-off method

The distributions of estimates of the SWE elicited in the benefit-harm trade-off will be plotted as frequency histograms. Non-parametric bootstrap methods will be used to generate 95% CIs for effects that are considered to be worthwhile by 20, 50 and 80% of participants.

Discrete choice experiment

A summary of descriptive statistics will be calculated for respondent samples. Data will be analysed with a mixed multinomial logit model (MMNL). With this approach, it is implicitly assumed that preferences do not vary between responses made by an individual respondent, but variation in preferences between respondents is modelled explicitly. Mixed models allow for dependence of observations provided by the same respondent. The use of MMNLs relaxes the statistical assumptions made with more commonly used fixed effect multinomial logit models and is likely to better explain choice behaviour than fixed effect models. Interactions between attributes and between attributes and population characteristics (eg, age, gender, education and prior recent experience with falls) will be explored by including the appropriate interaction terms in the model. The analysis will provide an estimate of the probability that participants consider an intervention to be worthwhile (or, more directly, of the log odds that participants would choose to have the intervention) based on any particular combination of attributes, and also will allow us to examine the trade-offs between attributes that participants are willing to accept. In accordance with the recommendations of Lancsar and Louviere, responses deemed by researchers as ‘irrational’ will not be excluded from analysis, and all available responses will be included in the model.

**DISCUSSION**

This project will determine, from the older person’s perspective, the SWE of an exercise programme designed to prevent falls. Estimates of the SWE can be used to determine whether the effects of new and existing fall prevention programmes are large enough to justify their implementation.

The outcomes will be important for falls researchers and policy makers because they will provide the first rigorous analysis of the features of falls prevention programmes that might increase the participants’ participation and adherence to these programmes. Therefore, the results could be used to optimise interventions, as they will identify attributes of falls prevention programme that maximise value to the recipients.

This project also has a broader significance as it involves the development of novel methods that are applicable to the design and interpretation of clinical trials across all areas of healthcare. Benefit-harm trade-off studies are simple enough to be routinely conducted prior to clinical trials. This would provide robust, justifiable, empirical estimates of the SWE for use in sample size calculations and the interpretation of trial findings. It is possible that, if these simple procedures were widely adopted, clinical trials could be very different in size; some clinical conditions might be managed very differently, and health resources might be radically redistributed.

**Author affiliations**

1Musculoskeletal Division, The George Institute for Global Health, Sydney, Australia
2School of Public Health, University of Sydney, Sydney, Australia
3Business School, Institute of Transport and Logistics Studies, University of Sydney, Sydney, Australia
4Faculty of Medicine, Nursing & Health Science, Monash University, Melbourne, Australia
5Faculty of Health Science, University of Sydney, Sydney, Australia

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**Competing interests** None.

**Ethics approval** This project has received Ethics Approval from the University of Sydney Human Ethics Committee (Protocol number: 14404). Individual
written informed consent will be obtained before the conduct of the research in accordance with the ethics of medical research.

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