McMaster University

Family Medicine

Acknowledgements:
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Moving your research from idea to action: A hands-on exercise to map out a pilot study

Watch a recording of this session

September 22, 2020

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Michael G. DeGroote SCHOOL OF MEDICINE

Pilot and Feasibility Studies in Primary Care

- Discuss the purpose of pilot and feasibility studies: what they aren't and what they are
- Think about aspects of design
- Understand some appropriate outcomes for pilot and feasibility studies
- Highlight supporting tools and resources

Introductions

- Please introduce yourself in the “Chat” feature
  - Name, role, city/location where you work

A typical Monday?

9:00 You check your labs and find the MSU culture is actually negative on a woman who came in with dysuria and frequency.

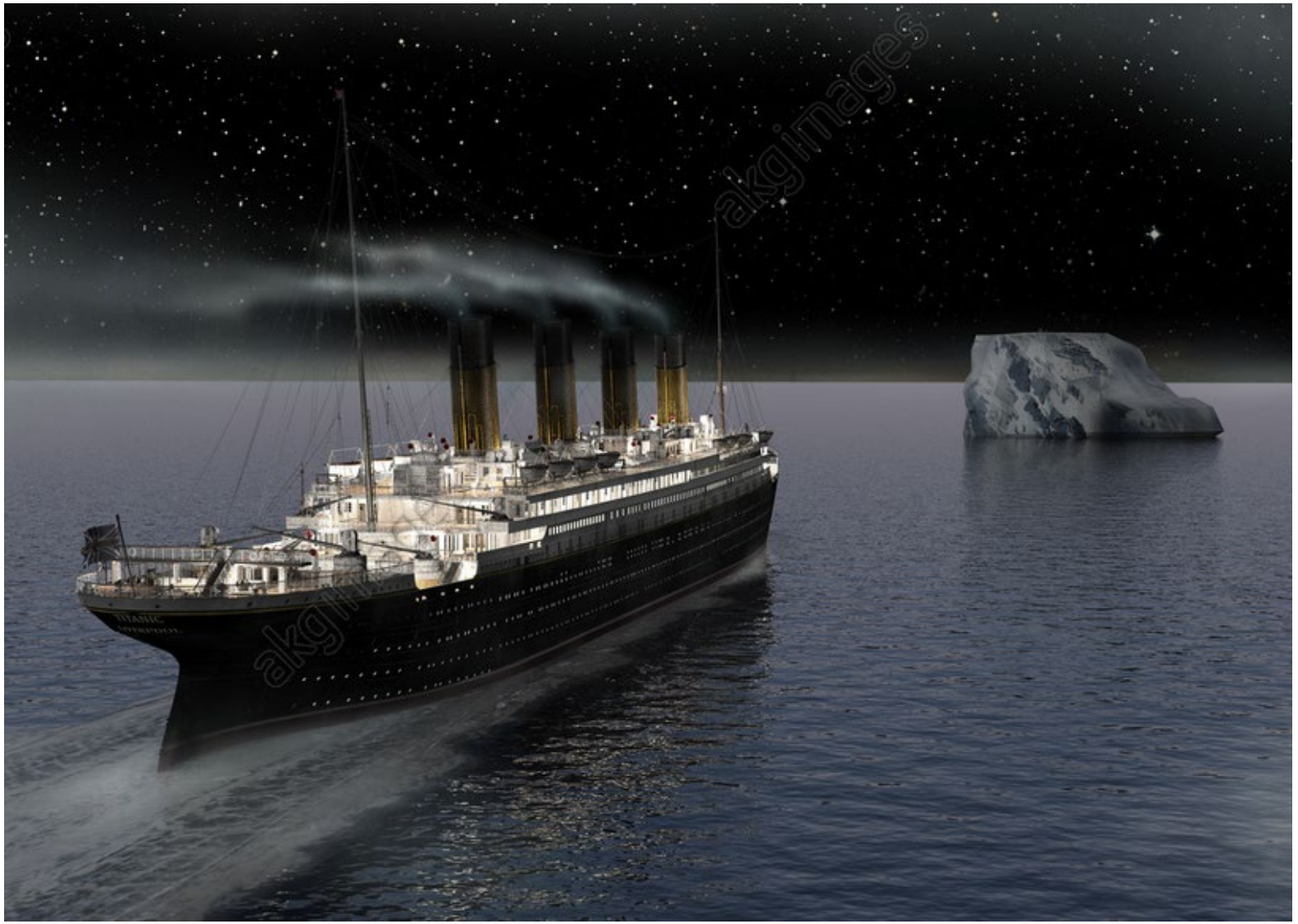
9:30 65 year old woman with chest infection and signs of pneumonia needing admission to hospital

9:45 34 year old woman in for repeat prescription of her long term SSRI

10:00 Mother at the end of her tether trying to deal with tantrums in her 2 year old, has heard iron deficiency can cause behaviour problems

10:15 A 75 year old woman who is confused, bought in by her daughter with a bag with 9 medicines in it

…… you read in your emails that Family Doctors are to be restricted from using D-dimer and ultrasounds for DVT as they would over use them if allowed open access



You do not want this to become the ship of doom

Pilot and feasibility studies: What they aren’t

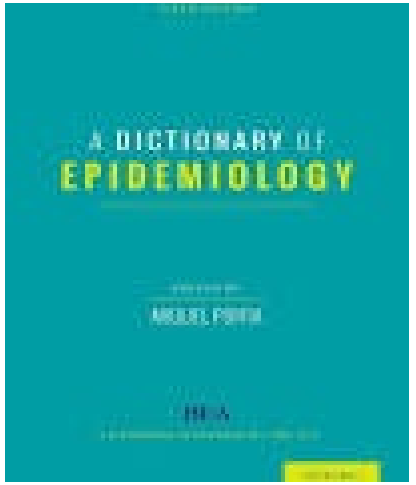
- **A small study because of limited funding**

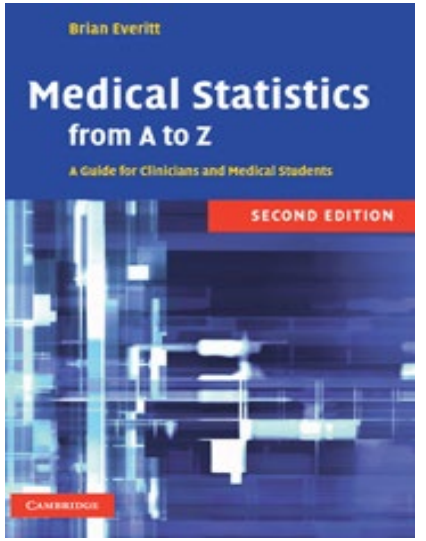
  I have funding for only 10 patients and maybe if I’m really lucky, I’ll see an effect!

What they are:

Small-scale investigation designed to

test the feasibility of methods and of procedures for later use on a large scale or **search for possible effects and associations** that may be worth following up in a larger study



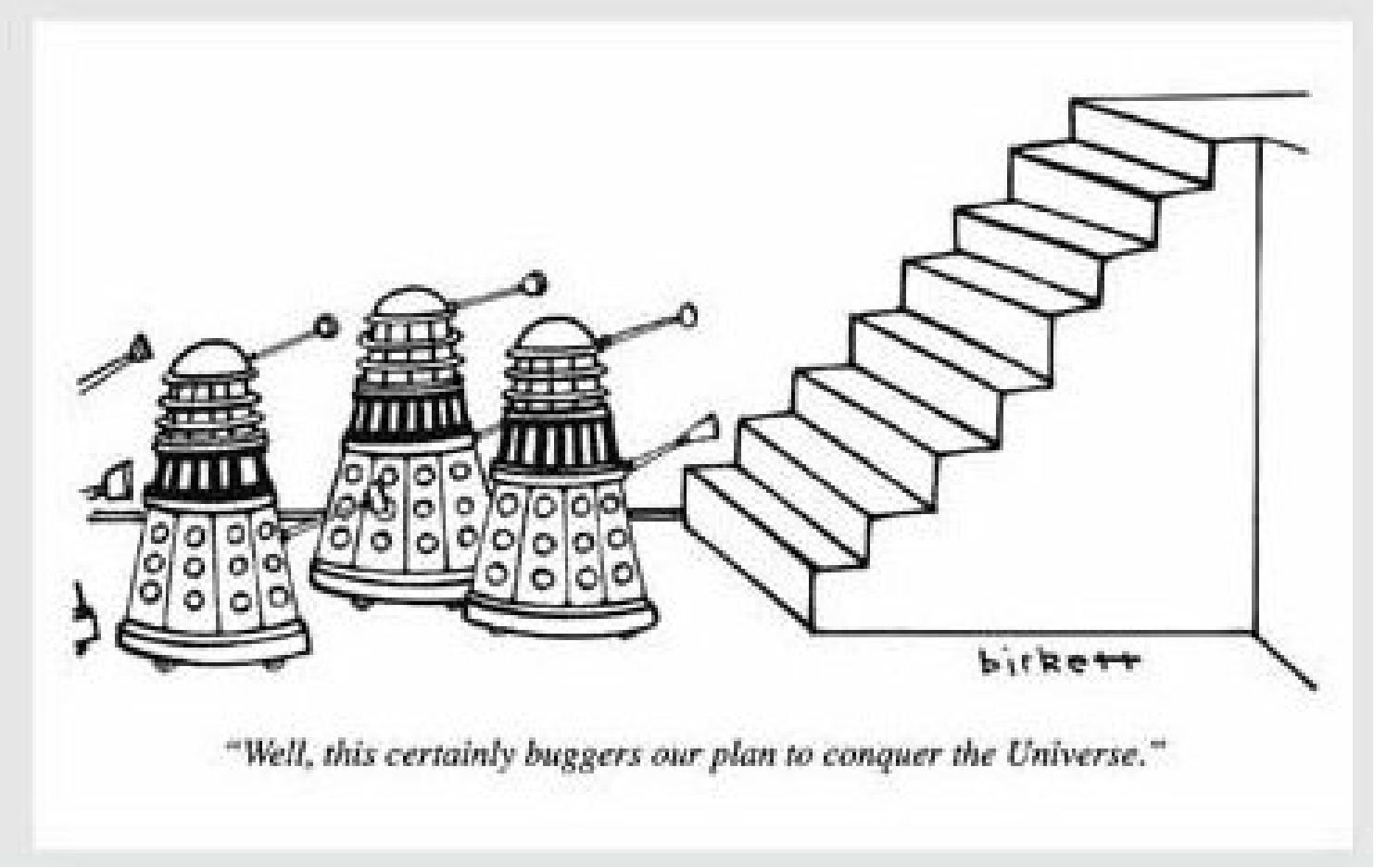


Answers to process, clinical, methodological questions:

- Can I recruit people for this study?
- Will they stay in the study (retention)?
- Are the processes or intervention feasible in a primary care clinical setting?
- What human and other resource will be needed?
- Are the ways I want to measure outcomes feasible?

  Will they measure what I think they will?
  - "floor" or "ceiling" effects
  - ease of administration / time taken
  - any signal of effect in the direction I hope (can I use it to plan sample size)

The essential element for success in primary care research: being prepared to sacrifice good ideas



Challenges with Pilot Studies: pitfalls to avoid

- Not well designed
  - No clear feasibility objectives
  - No clear rationale for piloting
  - No clear analytic plan
  - No clear criteria for success of feasibility
- Not publishing your pilot study (Journal of Pilot and Feasibility Studies)
- Using pilot studies to estimate treatment effects
  - Estimates may be unrealistic/biased
  - **Conducting underpowered trials to assess outcomes is unethical**

Key Resource

Lehana Thabane
Professor McMaster University
Editor-in-chief, Pilot and Feasibility Studies journal

Thabane *et al. BMC Medical Research Methodology* 2010, **10**:1
http://www.biomedcentral.com/1471-2288/10/1

BMC Medical Research Methodology

COMMENTARY Open Access

A tutorial on pilot studies: the what, why and how

Lehana Thabane[1,2*], Jinhui Ma[1,2], Rong Chu[1,2], Ji Cheng[1,2], Afisi Ismaila[1,3], Lorena P Rios[1,2], Reid Robson[3], Marroon Thabane[1,4], Lora Giangregorio[5], Charles H Goldsmith[1,2]

Abstract

Pilot studies for phase III trials - which are comparative randomized trials designed to provide preliminary evidence on the clinical efficacy of a drug or intervention - are routinely performed in many clinical areas. Also commonly know as "feasibility" or "vanguard" studies, they are designed to assess the safety of treatment or interventions; to assess recruitment potential; to assess the feasibility of international collaboration or coordination for multicentre trials; to increase clinical experience with the study medication or intervention for the phase III trials. They are the best way to assess feasibility of a large, expensive full-scale study, and in fact are an almost essential pre-requisite. Conducting a pilot prior to the main study can enhance the likelihood of success of the main study and potentially help to avoid doomed main studies. The objective of this paper is to provide a detailed examination of the key aspects of pilot studies for phase III trials including: 1) the general reasons for conducting a pilot study; 2) the relationships between pilot studies, proof-of-concept studies, and adaptive designs; 3) the challenges of and misconceptions about pilot studies; 4) the criteria for evaluating the success of a pilot study; 5) frequently asked questions about pilot studies; 7) some ethical aspects related to pilot studies; and 8) some suggestions on how to report the results of pilot investigations using the CONSORT format.

Feasibility of Procedures & Methods

JAMDA 15 (2014) 943–945

ELSEVIER

JAMDA

journal homepage: www.jamda.com

Brief Report

Implementing a Knowledge Translation Intervention in Long-Term Care: Feasibility Results From the Vitamin D and Osteoporosis Study (ViDOS)

Courtney C. Kennedy PhD [a], Lehana Thabane PhD [b], George Ioannidis PhD [a], Jonathan D. Adachi MD, FRCPC [a], Alexandra Papaioannou MD, MSc, FRCP (C) FACP [a,*] on behalf of the ViDOS Investigators

[a] Department of Medicine, McMaster University, Hamilton, ON, Canada
[b] Department of Epidemiology and Biostatistics, McMaster University, Hamilton, ON, Canada

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Feasibility Outcomes

- Recruitment rate
- Retention rate
- Participation rate
- Completion of action plans, feedback reports
- Completeness of data

Criteria for Success

It is always important to state the criteria for success or feasibility

Overall Outcome can be

Stop - main study not feasible;

Continue, but modify protocol – feasible with modifications;

Continue without modifications – feasible

The criteria should be based on key primary feasibility aims

Outcomes and Criteria for Success

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[a] Department of Medicine, McMaster University, Hamilton, ON, Canada
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Table 1
Feasibility Results for the ViDOS Trial

Measure	Target, %	Observed, %	Description
Recruitment	40	22	■ Acceptance rate was low; took several months ■ Accomplished target sample size (n = 40)
Retention	80	63	■ 7 INT homes withdrew active participation.
Participation	80	25–100	■ Overall: n = 164 participants from 12 active INT homes; 56% attended at least 2 meetings* **Key roles:** ■ ≥ 2 meetings: Director/assistant director of care†; pharmacist = 100%; medical director† = 92% ■ 3 meetings: Director/assistant director† = 83%; pharmacist = 92%; medical director† = 25%
Action plans	80	100	■ Completed by all homes
Feedback reports	80	100	■ Reviewed at all INT sessions (months 0, 6, 12)
Data completion	80	86–92	■ All spreadsheets: 86% control/92% INT homes

Sample Size for Pilot Studies

- **Sample Size calcs generally not required for pilot / feasibility studies**

  A pilot study may be used to generate information to be used for sample size calculations

- **Sample needs to be representative of the target population**

  It should also be based on the same inclusion/exclusion criteria

Stat Med 1990:9(1-2);65-71; discussion 71-2.

Consent

Consent form should be explicit about the feasibility nature

"The overall purpose of this pilot study is to assess the feasibility of conducting a large study to ...[state primary objective of the main study] . A feasibility or pilot study is a study that… [state a general definition of a feasibility study]. The specific feasibility objectives of this study are …[state the specific feasibility objectives of the pilot study]. We will determine that it is feasible to carry on the main study if …[state the criteria for success of feasibility] ."

Publication

- **Can I publish the results of a pilot study?**

  Yes!
- **Why is it important to publish the results of pilot studies?**

  To provide information about feasibility to the research community
- **Most pilot studies do not show statistically significant results:**

  “no evidence of effect” is not “evidence of no effect”

  The focus in reporting the results of a pilot should be on feasibility,

  NOT statistical significance

Randomisation?

- **Can I randomise patients in a pilot study?**

  Yes - to assess how a randomisation might work in main study or whether it might be acceptable to patients

  (In general, it is always best to maintain the same design as the main study)

- **Can I use these data in the main study?**

  Possibly…. if you don't unblind for analysis

Key Messages

- **Provide a good opportunity to assess feasibility of large studies**

✓It can enhance the success probability of the main study or help with a go/no-go decision

- **Pilot studies should be well designed**

✓Clear **feasibility** objectives prior to starting

✓Clear rationale for piloting – needs to be linked to main study

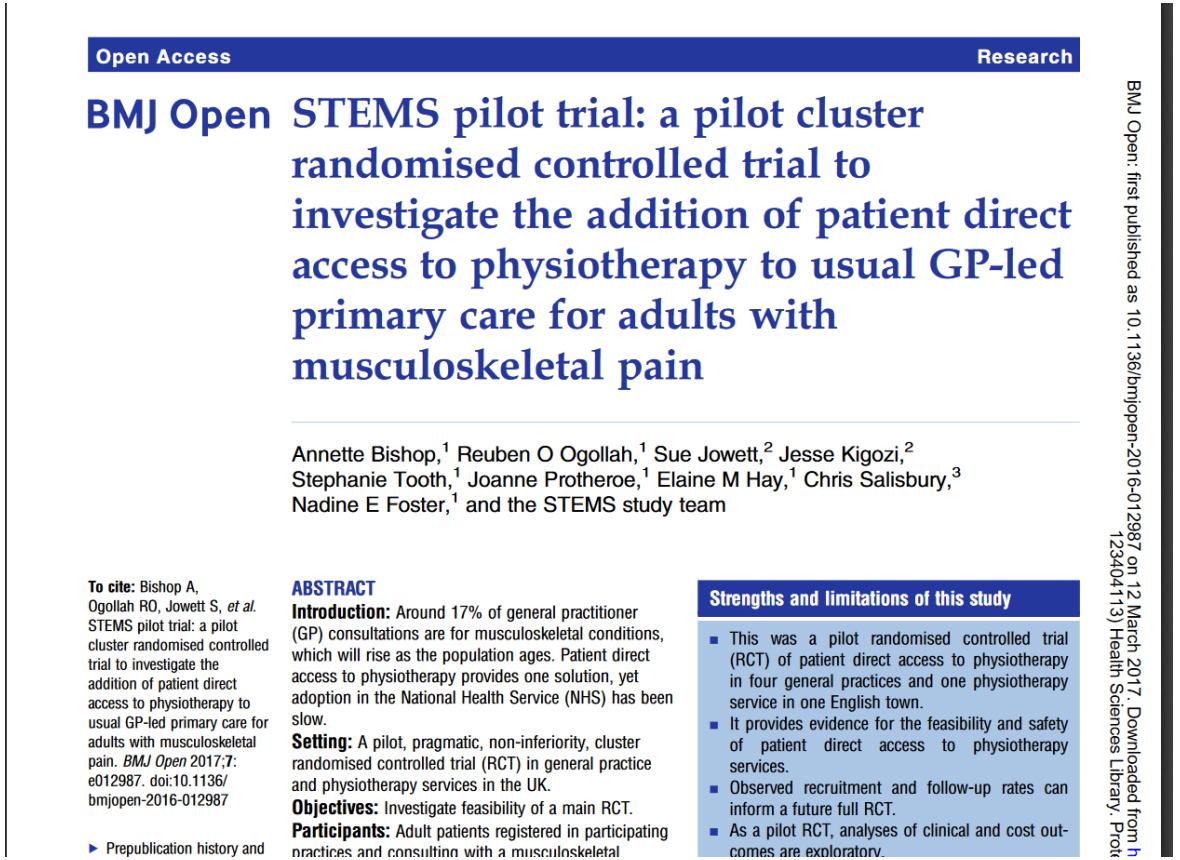
✓Clear analytic plans

✓Clear criteria for determining success of feasibility

✓Publish

Our practice example

Bishop A, Ogollah RO, Jowett S and the STEMS study team*, et al.* STEMS pilot trial: a pilot cluster randomised controlled trial to investigate the addition of patient direct access to physiotherapy to usual GP-led primary care for adults with musculoskeletal pain *BMJ Open* 2017;**7:**e012987. doi: 10.1136/bmjopen-2016-012987



Open Access Research

BMJ Open

STEMS pilot trial: a pilot cluster randomised controlled trial to investigate the addition of patient direct access to physiotherapy to usual GP-led primary care for adults with musculoskeletal pain

Annette Bishop,[1] Reuben O Ogollah,[1] Sue Jowett,[2] Jesse Kigozi,[2] Stephanie Tooth,[1] Joanne Protheroe,[1] Elaine M Hay,[1] Chris Salisbury,[3] Nadine E Foster,[1] and the STEMS study team

To cite: Bishop A, Ogollah RO, Jowett S, *et al*. STEMS pilot trial: a pilot cluster randomised controlled trial to investigate the addition of patient direct access to physiotherapy to usual GP-led primary care for adults with musculoskeletal pain. *BMJ Open* 2017;**7**: e012987. doi:10.1136/bmjopen-2016-012987

► Prepublication history and

ABSTRACT

Introduction: Around 17% of general practitioner (GP) consultations are for musculoskeletal conditions, which will rise as the population ages. Patient direct access to physiotherapy provides one solution, yet adoption in the National Health Service (NHS) has been slow.

Setting: A pilot, pragmatic, non-inferiority, cluster randomised controlled trial (RCT) in general practice and physiotherapy services in the UK.

Objectives: Investigate feasibility of a main RCT.

Participants: Adult patients registered in participating practices and consulting with a musculoskeletal

Strengths and limitations of this study

- This was a pilot randomised controlled trial (RCT) of patient direct access to physiotherapy in four general practices and one physiotherapy service in one English town.
- It provides evidence for the feasibility and safety of patient direct access to physiotherapy services.
- Observed recruitment and follow-up rates can inform a future full RCT.
- As a pilot RCT, analyses of clinical and cost outcomes are exploratory.

BMJ Open: first published as 10.1136/bmjopen-2016-012987 on 12 March 2017. Downloaded from h 12340413) Health Sciences Library. Prot

Q1. Why do the authors say they are doing a pilot study?

A specific recommendation in the recent Primary Care Workforce Commission is to investigate whether direct access to physiotherapy for some conditions makes cost-effective use of NHS resources.[11]

The aim of the STEMS pilot trial was to assess the feasibility of a future large trial to compare the clinical and cost-effectiveness of the addition of patient direct access to physiotherapy versus continuing with usual GP-led care alone for adults with common musculoskeletal problems. The specific objectives of this pilot trial comprised process objectives to assess the feasibility of a future large cluster randomised trial, and research objectives that focused on exploratory analysis of clinical outcomes and costs.

Q2. What were the investigators hoping to learn by the process objectives?

Process objectives

1. To assess the engagement of GP practices and physiotherapy services to participate and stay in the trial through follow-up.
2. To assess the feasibility of establishing a patient's direct access to physiotherapy pathway.
3. To develop and test approaches to marketing the direct access pathway to ensure a sufficient proportion of patients use the pathway.
4. To estimate recruitment rates in the control and intervention practices.
5. To explore any evidence of selection bias in the control and intervention practices.
6. To estimate follow-up rates at each follow-up time point.

Q3. What were the investigators hoping to learn by the research objectives?

Research objectives

1. To explore changes in the proposed main trial primary outcome measure (physical health measured using the 36-item Short Form Health Survey (SF-36)v2 Physical Component Summary (PCS)).
2. To explore changes in other outcome measures (overall perceived change, SF-36v2 Mental Component Summary, pain self-efficacy, quality of life, understanding of the condition, experience of care, and convenience and accessibility of services).
3. To provide an exploratory estimate of costs, both healthcare and societal costs.
4. To estimate the parameters needed for a realistic sample size calculation for a future large cluster RCT.
5. To explore the use of willingness to pay methodology to capture the strength of patient preferences for self-referral to NHS physiotherapy. This analysis will be reported in a separate future paper.

Q4. RESULTS

- Were the research methods feasible?

Process results

Engagement of GP practices and the physiotherapy service

Of the five GP practices approached, four participated with three full-time equivalent GPs at each of the two intervention practices and five and two in the control practices. In the physiotherapy service, of the physiotherapists who undertook the STEMS training programme, four worked in the clinic local to the participating general practices and delivered care to patients using the direct access pathway (NHS Agenda for Change band 7 (n=1), band 6 (n=2) and band 5 (n=1)). Agenda for Change is the NHS Terms and Conditions of Service and determines salary scales for NHS staff.

Participant recruitment and retention

Of 2696 participants invited to take part in the data collection, 978 (36%) participated (425 (44%) in the intervention practices and 553 (57%) in the control practices). Recruitment was completed within 6 months (June to December 2013). Response rates were as follows: 74.3% at 2 months (intervention 70.4%, control 77.4%), 78.4% at 6 months (intervention 76.7%, control 79.8%) and 70.7% at 12 months (intervention 69.4%, control 71.6%). MDC was included at 6 and 12 months only resulting in higher response rates at these time points.

Q5. RESULTS

- Was the intervention feasible?

Feasibility of the patient direct access pathway

Of the participants in the STEMS pilot trial (n=978), a total of 256 (26.2%) accessed physiotherapy care during the study period, 152 (35.8%) in the intervention arm and 104 (18.8%) in the control arm. Of the 152 who accessed physiotherapy care in the intervention arm, 142 (93.4%) were self-referrals (comprising 98 (69%) 'recommended' and 44 (31%) 'true' self-referrals).

Comparisons to service-level data (which may not be entirely comparable, due to the introduction of new administration software in the physiotherapy service), from the same period 1 year prior to start of direct access suggests one practice in the intervention arm had broadly similar rates of patients accessing physiotherapy care, whereas the other had a substantial increase once the direct access pathway had been introduced. The referral rates to physiotherapy prior to the trial showed considerable variation

Q6. RESULTS

- What was the impact? (it's a non-inferiority trial-no difference is good)

Patients' physical health measured using the SF-36 PCS is the anticipated primary clinical outcome for a future main trial. Higher scores represent better physical health. In this exploratory analysis very similar scores over time in participants in both arms of the trial were seen. The adjusted mean difference between groups and 95% CI showed estimates of effect at 2 months −0.14 (95% CI −1.02 to 0.74), at 6 months −0.28 (−1.35 to 0.79) and at 12 months 0.12 (−1.27 to 1.51). The lower limit of the two-sided 95% CI for intervention versus control mean difference was >−2, the smallest expected standard treatment effect for the outcome (SF-36 PCS). Exploratory adjusted estimates of treatment effects for other outcomes are provided in the online supplementary table S2.

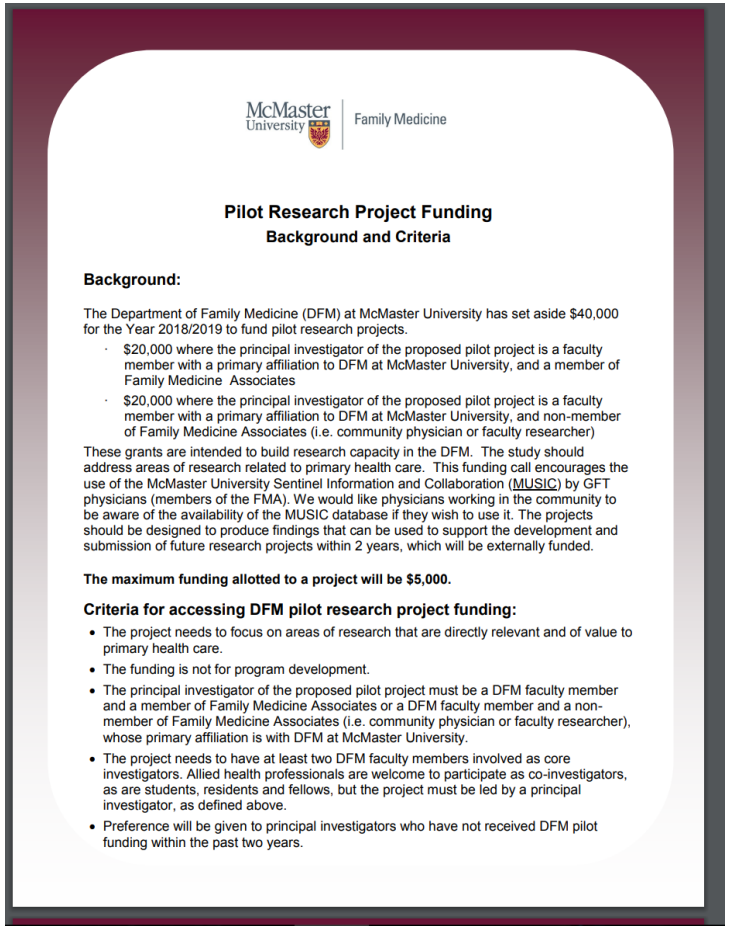
Resources for Pilot Studies in Primary Care

Can I apply for funding for a pilot study?

Yes (We really hope you do!)

DFM (and others) have a fund for pilot grants

- Annual peer reviewed application round
- Up to $5000 per proposal
- Strictly one year timeframe
- Should be in the context of the main study, or next step – a bridge to somewhere
- Not for program funding



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Pilot Research Project Funding
Background and Criteria

Background:

The Department of Family Medicine (DFM) at McMaster University has set aside $40,000 for the Year 2018/2019 to fund pilot research projects.

- $20,000 where the principal investigator of the proposed pilot project is a faculty member with a primary affiliation to DFM at McMaster University, and a member of Family Medicine Associates
- $20,000 where the principal investigator of the proposed pilot project is a faculty member with a primary affiliation to DFM at McMaster University, and non-member of Family Medicine Associates (i.e. community physician or faculty researcher)

These grants are intended to build research capacity in the DFM. The study should address areas of research related to primary health care. This funding call encourages the use of the McMaster University Sentinel Information and Collaboration (MUSIC) by GFT physicians (members of the FMA). We would like physicians working in the community to be aware of the availability of the MUSIC database if they wish to use it. The projects should be designed to produce findings that can be used to support the development and submission of future research projects within 2 years, which will be externally funded.

The maximum funding allotted to a project will be $5,000.

Criteria for accessing DFM pilot research project funding:

- The project needs to focus on areas of research that are directly relevant and of value to primary health care.
- The funding is not for program development.
- The principal investigator of the proposed pilot project must be a DFM faculty member and a member of Family Medicine Associates or a DFM faculty member and a non-member of Family Medicine Associates (i.e. community physician or faculty researcher), whose primary affiliation is with DFM at McMaster University.
- The project needs to have at least two DFM faculty members involved as core investigators. Allied health professionals are welcome to participate as co-investigators, as are students, residents and fellows, but the project must be led by a principal investigator, as defined above.
- Preference will be given to principal investigators who have not received DFM pilot funding within the past two years.

Family Medicine and Community Health

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DFM Resources Ask!

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General inquiries, staffing, research project development