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Conducting and analysing pilot and feasibility trials

Christine Bond

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My talk today

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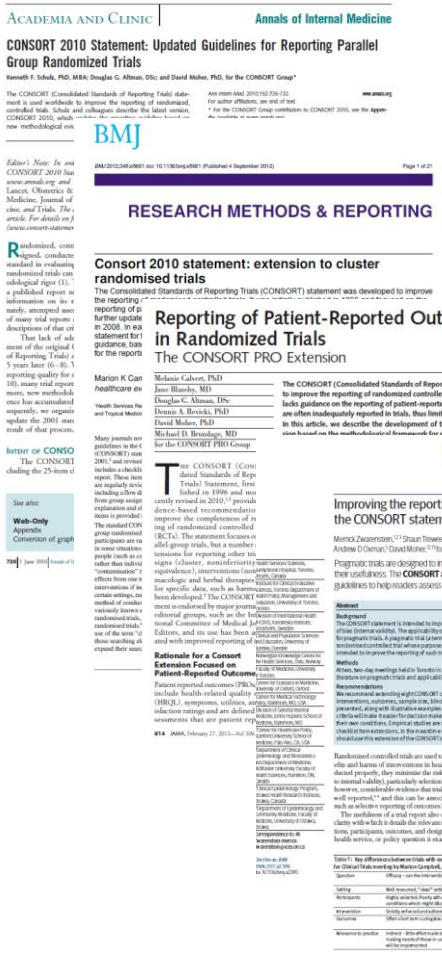
CONSORT extensions


ACADEMIA AND CLINIC | Annals of Internal Medicine

CONSORT 2010 Statement: Updated Guidelines for Reporting Parallel Group Randomized Trials

BMJ

RESEARCH METHODS & REPORTING

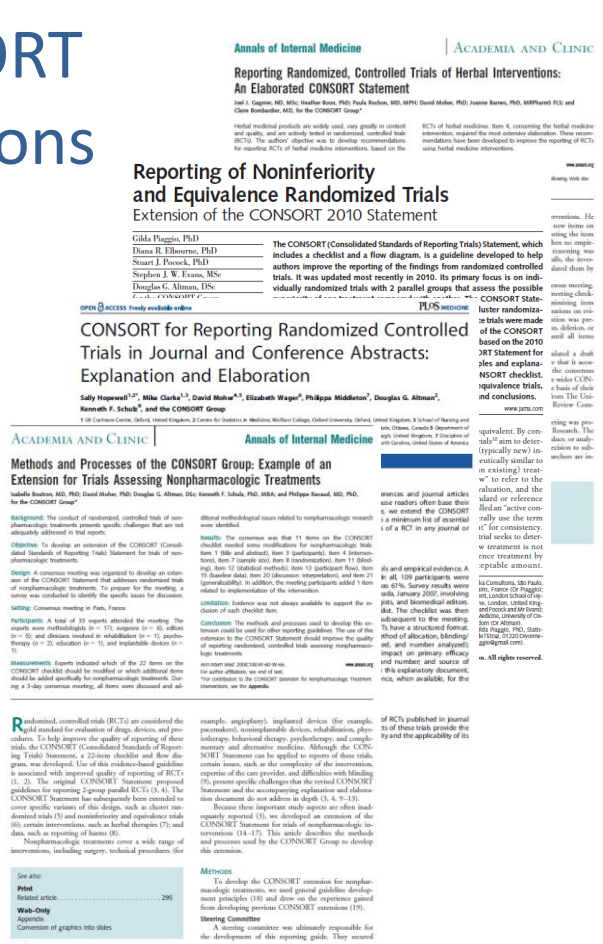
Consort 2010 statement: extension to cluster randomised trials

Reporting of Patient-Reported Outcomes in Randomized Trials

The CONSORT PRO Extension

RESEARCH METHODS & REPORTING

Improving the reporting of pragmatic trials: an extension of the CONSORT statement


Annals of Internal Medicine | ACADEMIA AND CLINIC

Reporting Randomized, Controlled Trials of Herbal Interventions: An Elaborated CONSORT Statement

Reporting of Noninferiority and Equivalence Randomized Trials

Extension of the CONSORT 2010 Statement

PLOS MEDICINE

CONSORT for Reporting Randomized Controlled Trials in Journal and Conference Abstracts: Explanation and Elaboration

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Methods and Processes of the CONSORT Group: Example of an Extension for Trials Assessing Nonpharmacologic Treatments

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Checklist development

Review of literature

CONSORT adaptation

Delphi exercise

Stakeholder consensus meeting

Iterative review and refinement

New and adapted items

CONSORT extension for randomized pilot and feasibility trials

Checklist applies to:

- Randomized trials conducted in preparation for a future definitive trial of effectiveness or efficacy
  - Primary aim: feasibility of the future definitive trial
- No restrictions on terminology used to describe the preparatory trial
- No restrictions on the design of either trial
- Doesn't apply to internal pilot studies.

RESEARCH METHODS AND REPORTING

OPEN ACCESS

CONSORT 2010 statement: extension to randomised pilot and feasibility trials

Sandra M Eldridge,[1] Claire L Chan,[1] Michael J Campbell,[2] Christine M Bond,[3] Sally Hopewell,[4] Lehana Thabane,[5] Gillian A Lancaster[6] on behalf of the PAFS consensus group

[1]Centre for Primary Care and Public Health, Queen Mary University of London, London, UK
[2]School of Health and Related Research, University of Sheffield, Sheffield, UK
[3]Centre of Academic Primary Care, University of Aberdeen, Aberdeen, Scotland, UK

The Consolidated Standards of Reporting Trials (CONSORT) statement is a guideline designed to improve the transparency and quality of the reporting of randomised controlled trials (RCTs). In this article we present

Consequently, although much of the information to be reported in these trials is similar to those in randomised controlled trials (RCTs) assessing effectiveness and efficacy, there are some key differences in the type of

Objectives

Section/topic and item No	Standard checklist item	Extension for pilot trials
Title and abstract		
1a	Identification as a randomised trial in the title	Identification as a pilot or feasibility randomised trial in the title
1b	Specific objectives or research questions for pilot trial	ethods, nce see)
Introduction		
Background and objectives:		
2a	Scientific background and explanation of rationale	Scientific background and explanation of rationale for future definitive trial, and reasons for randomised pilot trial
2b	Specific objectives or hypotheses	Specific objectives or research questions for pilot trial

Main uncertainties in future trial?

Design	Population	Setting & Recruitment
Intervention	Similarity of interventions	Randomisation implementation
Randomisation type	Allocation concealment	Blinding
Outcomes	Statistical methods	Stopping rules

An example from care homes

- "In this feasibility trial, the research aim was to explore trial design, staff and resident acceptability of the interventions and outcome measures and to provide data to estimate the parameters required to design a definitive RCT….The primary objectives of the trial were as follows:
- 1. To assess how many care homes accepted the invitation to participate
- 2. To determine whether the eligibility criteria for care home residents were too open or too restrictive by estimating feasible eligibility and recruitment rate.
- 3. To assess retention of care homes and residents by estimating 3 and 6-month follow-up rates.
- 4. To investigate the acceptability of nutritional support interventions to malnourished care home residents in terms of compliance and to care home staff in terms of adherence to the intervention schedule.
- 5. To assess the acceptability and feasibility of the outcome measures

Stow R, Ives N, Smith C, Rick C, Rushton A. A cluster randomised feasibility trial evaluating nutritional interventions in the treatment of malnutrition in care home adult residents. Trials. 2015;16(1):433.

Sample selection

Methods		
Trial design:		
3a	Description of trial design (such as parallel, factorial) including allocation ratio	Description of pilot trial design (such as parallel, factorial) including allocation ratio
3b	Important changes to methods after trial	Important changes to methods after pilot trial criteria), with
Participants:		
4a		
4b	Settings and locations where the data were collected	
4c		How participants were identified and consented
Interventions:		
5	The interventions for each group with sufficient details to allow replication, including how and when they were actually administered	

How participants were identified and consented

An example from mindfulness

Between May and October 2013, clinical staff at participating gastroenterology outpatient clinics scanned and identified potential participants that met the study inclusion criteria. Then, either study invitation packs were sent to patients with researchers contact details or patients seen consecutively in clinics were approached with the study information. All study information was co-designed with patients from the patient-involvement group. Interested participants then registered their interest with the researcher by telephone or email. This was followed up with a screening visit with the researcher and then informed written consent was obtained

Schoultz M, Atherton I, Watson A. Mindfulness-based cognitive therapy for inflammatory bowel disease patients: findings from an exploratory pilot randomised controlled trial. Trials. 2015;16(1):1-13

Outcomes

Outcomes:		
6a	Completely defined pre-specified primary and secondary outcome measures, including how and when they were assessed	Completely defined pre-specified assessments or measurements to address each pilot trial objective specified in 2b, including how and when they were assessed
8b	Type of randomisation; details of any restriction (such as blocking and block size)	Type of randomisation(s); details of any restriction (such as blocking and block size)
Allocation concealment mechanism:		
9	Mechanism used to implement the random allocation sequence (such as sequentially numbered containers), describing any steps taken to conceal the sequence until interventions were assigned	
Implementation:		
10	Who generated the random allocation sequence, who enrolled participants, and who assigned participants to interventions	

Completely defined pre-specified assessments or measurements to address each pilot trial objective specified in 2b, including how and when they were assessed

Diabetes and virtual support

Acceptability and *demand* were assessed in terms of the usage and repeated usage of the intervention by the patients in the trial indicated by logged user statistics. The interventions' *practicability* was considered as the ability to log in and occurrence of constraints in delivery and was assessed in terms of the percentage of users in adolescents and professionals, its bounce percentage (percentage of login-errors) and other login-problems. …….*Integration* was assessed in terms of the extent to which our web-based intervention promotes care that was consistent with recognized standards of diabetes care for adolescents including those published by the International Diabetes Federation (IDF) in collaboration with the International Society for Pediatric and Adolescent Diabetes (ISPAD) and the American Diabetes Association (ADA; 3, 33)

Boogerd EA, Noordam C, Kremer JA, Prins JB, Verhaak CM. Teaming up: feasibility of an online treatment environment for adolescents with type 1 diabetes. Pediatric diabetes. 2014;15(5):394-402

Analysis

Blinding:		
11a	If done, who was blinded after assignment to interventions (for example, participants, care providers, those assessing outcomes) and how	
11b		
Analytical method		
12a	Statistical methods used to compare group for primary and secondary outcomes	Methods used to address each pilot trial objective whether qualitative or quantitative
12b	Methods for additional analyses, such as subgroup analyses and adjusted analyses	Not applicable

Methods used to address each pilot trial objective whether qualitative or quantitative

Role of anaesthetic volume and concentration

- The feasibility outcomes were reported descriptively and narratively. For the clinical endpoints, only descriptive statistics, mean (standard deviation) for continuous outcomes and raw count (%) for categorical outcomes, were reported

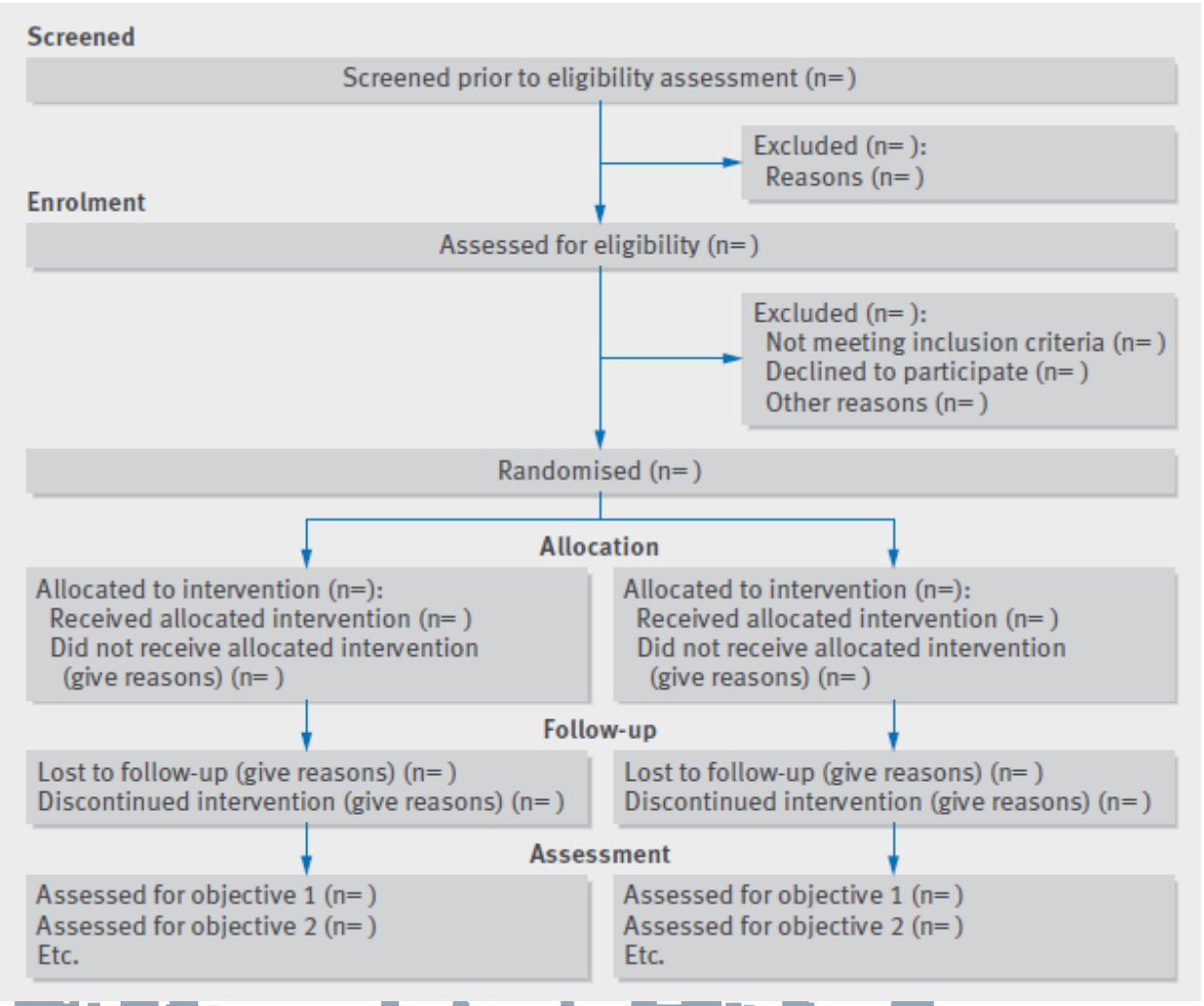
- *Forero M, Heikkila A, Paul JE, Cheng J, Thabane L. Lumbar transversus abdominis plane block: the role of local anesthetic volume and concentration—a pilot, prospective, randomized, controlled trial. Pilot and Feasibility Studies. 2015;1(1):10.*

Details for CONSORT diagram

Results		
Participant flow (a diagram is strongly recommended):		
13a	For each group, the numbers of participants who were randomly assigned, received intended treatment, and were analysed for the primary outcome	For each group, the numbers of participants who were approached and/or assessed for eligibility, randomly assigned, received intended treatment, and were assessed for each objective
13b	For each group, losses and exclusions after randomisation, together with reasons	
Recruitment:		
14a		
14b		
Baseline data:		
15	A table showing baseline demographic and clinical characteristics for each group	
Numbers analysed:		
16	For each group, number of participants (denominator) included in each analysis and whether the analysis was by original assigned groups	For each objective, number of participants (denominator) included in each analysis. If relevant, these analyses should be by randomised group

For each group the numbers of participants who were approached and/or assessed for eligibility, randomly assigned, received intended treatment and were assessed for each objective

13a: Participant flow diagram



Remember: methods and analyses must address each objective (primary and secondary)

Feasibility eg. recruitment, adherence

Patient-centred eg. data collection

Analysis revisited

Outcomes and estimation:		
17a	For each primary and secondary outcome, results for each group, and the estimated effect size and its precision (such as 95% confidence interval)	For each objective, results including expressions of uncertainty (such as 95% confidence interval) for any estimates. If relevant, these results should be by randomised group
17b	For binary outcomes, presentation of both absolute and relative effect sizes is recommended	Not applicable
Ancillary analyses:		
18		
Harms:		
19	All important harms or unintended effects in each group (for specific guidance see CONSORT for harms)	
19a		If relevant, other important unintended consequences

For each objective, results including expressions of uncertainty (such as 95% confidence interval for any estimates). If relevant these results should be by randomised group.

- Often requires mixed methods
- Qualitative and quantitative to tease out strengths and weaknesses for all participants
- Helps identify things that need to change

Lifestyle

Rates of initiation of lifestyle change also favoured the individualized assessment arm but less clearly. At 3months, 75% of the individualized assessment arm and 68% of the usual assessment arm had initiated changes in their lifestyle (unadjusted odds ratio, 1.38 [95%CI, 0.55 to 3.52]). At 6 months, the percentages were 85% and 75%, suggesting increased initiation of change over time in both arms, with the gap widening slightly (unadjusted odds ratio, 1.86 [95% CI, 0.64 to 5.77])…. Wide CIs again point to the degree of uncertainty around this conclusion

Hill K, Walwyn R, Camidge D, Murray J, Meads D, Reynolds G, et al. A Randomized Feasibility Trial of a New Lifestyle Referral Assessment Versus Usual Assessment in an Acute Cardiology Setting. The Journal of cardiovascular nursing. 2015.

What do you hope to conclude?

Discussion		
Limitations:		
20	Trial limitations, addressing sources of potential bias, imprecision, and, if relevant, multiplicity of analyses	Pilot trial limitations, addressing sources of potential bias and remaining uncertainty about feasibility
Generalisability:		
21	Generalisability (external validity, applicability) of the trial findings	Generalisability (applicability) of pilot trial methods and findings to future definitive trial and other pilot studies
Interpretation:		
22	Interpretation consistent with results, balancing benefits and harms, and considering other relevant evidence	Interpretation consistent with pilot trial objectives and findings, balancing potential benefits and harms, and considering other relevant evidence
22a		Implications for progression from pilot to future definitive trial including any proposed amendments

Remember: a pilot trial is about….

Making a decision about whether to proceed with the next stage

- Which may be a main trial
- Or main trial with modifications
- Or may be another feasibility study

What comes next?

Example: DECISION+ pilot trial (Leblanc et al 2011)

Aim of main study: Optimal use of antibiotics for treating acute respiratory infections in primary care

Intervention: Education in shared decision-making among family physicians and patients

Objective of pilot trial: To assess feasibility and acceptability of study design, procedures, and intervention

Pre-specified criteria for judging whether to proceed to main trial

- Family medicine groups participating >=50%
- Recruited family physicians participating in all three workshops >=70%
- Mean level of satisfaction from family physicians regarding the workshops >=65%
- Missing data in each completed questionnaire <10%

Example result : Only 24% of family medicine groups agreed to participate

"Not reaching the pre-established criteria does not necessarily indicate unfeasibility of the trial but rather underlines changes to be made to the protocol"

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Success

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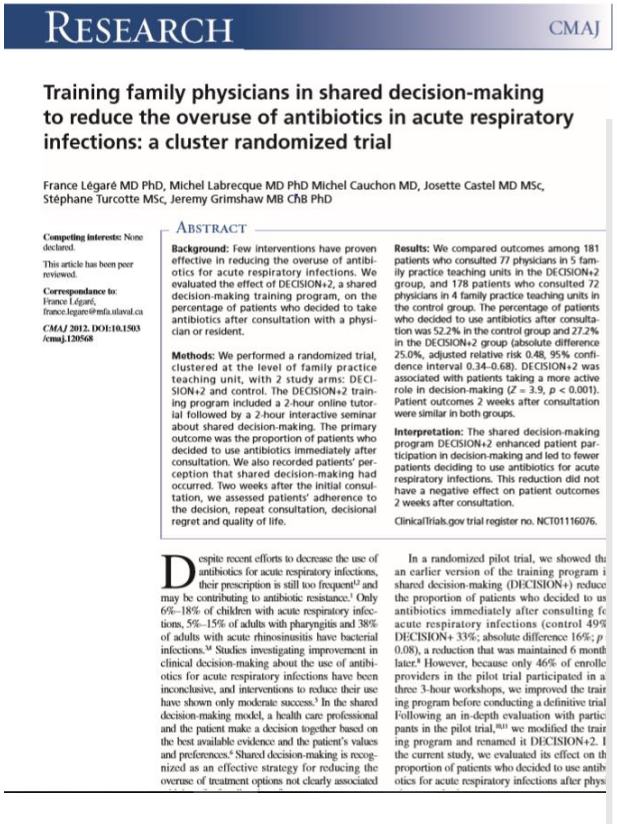

RESEARCH CMAJ

Training family physicians in shared decision-making to reduce the overuse of antibiotics in acute respiratory infections: a cluster randomized trial

France Légaré MD PhD, Michel Labrecque MD PhD Michel Cauchon MD, Josette Castel MD MSc, Stéphane Turcotte MSc, Jeremy Grimshaw MB ChB PhD

Competing interests: None declared.

This article has been peer reviewed.

Correspondance to:
France Légaré,
france.legare@mfa.ulaval.ca

CMAJ 2012. DOI:10.1503/cmaj.120568

Abstract

Background: Few interventions have proven effective in reducing the overuse of antibiotics for acute respiratory infections. We evaluated the effect of DECISION+2, a shared decision-making training program, on the percentage of patients who decided to take antibiotics after consultation with a physician or resident.

Methods: We performed a randomized trial, clustered at the level of family practice teaching unit, with 2 study arms: DECISION+2 and control. The DECISION+2 training program included a 2-hour online tutorial followed by a 2-hour interactive seminar about shared decision-making. The primary outcome was the proportion of patients who decided to use antibiotics immediately after consultation. We also recorded patients' perception that shared decision-making had occurred. Two weeks after the initial consultation, we assessed patients' adherence to the decision, repeat consultation, decisional regret and quality of life.

Results: We compared outcomes among 181 patients who consulted 77 physicians in 5 family practice teaching units in the DECISION+2 group, and 178 patients who consulted 72 physicians in 4 family practice teaching units in the control group. The percentage of patients who decided to use antibiotics after consultation was 52.2% in the control group and 27.2% in the DECISION+2 group (absolute difference 25.0%, adjusted relative risk 0.48, 95% confidence interval 0.34–0.68). DECISION+2 was associated with patients taking a more active role in decision-making ($Z = 3.9$, $p < 0.001$). Patient outcomes 2 weeks after consultation were similar in both groups.

Interpretation: The shared decision-making program DECISION+2 enhanced patient participation in decision-making and led to fewer patients deciding to use antibiotics for acute respiratory infections. This reduction did not have a negative effect on patient outcomes 2 weeks after consultation.

ClinicalTrials.gov trial register no. NCT01116076.

Despite recent efforts to decrease the use of antibiotics for acute respiratory infections, their prescription is still too frequent[1,2] and may be contributing to antibiotic resistance.[3] Only 6%–18% of children with acute respiratory infections, 5%–15% of adults with pharyngitis and 38% of adults with acute rhinosinusitis have bacterial infections.[3,4] Studies investigating improvement in clinical decision-making about the use of antibiotics for acute respiratory infections have been inconclusive, and interventions to reduce their use have shown only moderate success.[5] In the shared decision-making model, a health care professional and the patient make a decision together based on the best available evidence and the patient's values and preferences.[6] Shared decision-making is recognized as an effective strategy for reducing the overuse of treatment options not clearly associated

In a randomized pilot trial, we showed th an earlier version of the training program i shared decision-making (DECISION+) reduce the proportion of patients who decided to us antibiotics immediately after consulting fc acute respiratory infections (control 49% DECISION+ 33%; absolute difference 16%; p 0.08), a reduction that was maintained 6 month later.[8] However, because only 46% of enrolle providers in the pilot trial participated in a three 3-hour workshops, we improved the trair ing program before conducting a definitive trial Following an in-depth evaluation with partic pants in the pilot trial,[9,11] we modified the trair ing program and renamed it DECISION+2. I the current study, we evaluated its effect on th proportion of patients who decided to use antib otics for acute respiratory infections after phys

Légaré et al. BMC Family Practice 2011, 12:3
http://www.biomedcentral.com/1471-2296/12/3

BMC Family Practice

STUDY PROTOCOL Open Access

Training family physicians and residents in family medicine in shared decision making to improve clinical decisions regarding the use of antibiotics for acute respiratory infections: protocol for a clustered randomized controlled trial

France Légaré[1,2*], Michel Labrecque[1,2], Gaston Godin[3], Annie LeBlanc[4], Claudine Laurier[5], Jeremy Grimshaw[6], Josette Castel[2], Isabelle Tremblay[2], Pierre Frémont[2], Michel Cauchon[2], Kathleen Lemieux[2], Caroline Rhéaume[2]

Abstract

Background: To explore ways to reduce the overuse of antibiotics for acute respiratory infections (ARIs), we conducted a pilot clustered randomized controlled trial (RCT) to evaluate DECISION+, a training program in shared decision making (SDM) for family physicians (FPs). This pilot project demonstrated the feasibility of conducting a large clustered RCT and showed that DECISION+ reduced the proportion of patients who decided to use antibiotics immediately after consulting their physician. Consequently, the objective of this study is to evaluate, in patients consulting for ARIs, if exposure of physicians to a modified version of DECISION+, DECISION+2, would reduce the proportion of patients who decide to use antibiotics immediately after consulting their physician.

Methods/design: The study is a multi-center, two-arm, parallel clustered RCT. The 12 family practice teaching units (FPTUs) in the network of the Department of Family Medicine and Emergency Medicine of Université Laval will be randomized to a DECISION+2 intervention group (experimental group) or to a no-intervention control group. These FPTUs will recruit patients consulting family physicians and residents in family medicine enrolled in the study. There will be two data collection periods: pre-intervention (baseline) including 175 patients with ARIs in each study arm, and post-intervention including 175 patients with ARIs in each study arm (total n = 700). The primary outcome will be the proportion of patients reporting a decision to use antibiotics immediately after consulting their physician. Secondary outcome measures include: 1) physicians and patients' decisional conflict; 2) the agreement between the parties' decisional conflict scores; and 3) perception of patients and physicians that SDM occurred. Also in patients, at 2 weeks follow-up, adherence to the decision, consultation for the same reason, decisional regret, and quality of life will be assessed. Finally, in both patients and physicians, intention to engage in SDM in future clinical encounters will be assessed. Intention-to-treat analyses will be applied and account for the nested design of the trial will be taken into consideration.

Discussion: DECISION+2 has the potential to reduce antibiotics use for ARIs by priming physicians and patients to share decisional process and empowering patients to make informed, value-based decisions.

Trial Registration: ClinicalTrials.gov: NCT01116076

* Correspondence: france.legare@mfa.ulaval.ca
[1]Research Center of Centre Hospitalier Universitaire de Québec, Hospital St-François D'Assise, Knowledge Transfer and Health Technology Assessment Research Group, 10 Espinay, Québec, QC, G1L 3L5, Canada
Full list of author information is available at the end of the article

Légaré et al. Implementation Science 2013, 8:144
http://www.implementationscience.com/content/8/1/144

IMPLEMENTATION SCIENCE

RESEARCH Open Access

Impact of DECISION + 2 on patient and physician assessment of shared decision making implementation in the context of antibiotics use for acute respiratory infections

France Légaré[1,2*], Mireille Guerrier[1], Catherine Nadeau[1], Caroline Rhéaume[2,3], Stéphane Turcotte[1] and Michel Labrecque[1,2]

Abstract

Background: DECISION + 2, a training program for physicians, is designed to implement shared decision making (SDM) in the context of antibiotics use for acute respiratory tract infections (ARTIs). We evaluated the impact of DECISION + 2 on SDM implementation as assessed by patients and physicians, and on physicians' intention to engage in SDM.

Methods: From 2010 to 2011, a multi-center, two-arm, parallel randomized clustered trial appraised the effects of DECISION + 2 on the decision to use antibiotics for patients consulting for ARTIs. We randomized 12 family practice teaching units (FPTUs) to either DECISION + 2 or usual care. After the consultation, both physicians and patients independently completed questionnaires based on the D-Option scale regarding SDM behaviors during the consultation. Patients also answered items assessing the role they assumed during the consultation (active/collaborative/passive). Before and after the intervention, physicians completed a questionnaire based on the Theory of Planned Behavior to measure their intention to engage in SDM. To account for the cluster design, we used generalized estimating equations and generalized linear mixed models to assess the impact of DECISION + 2 on the outcomes of interest.

Results: A total of 270 physicians (66% women) participated in the study. After DECISION + 2, patients' D-Option scores were 80.1 ± 1.1 out of 100 in the intervention group and 74.9 ± 1.1 in the control group ($p = 0.001$). Physicians' D-Option scores were 79.7 ± 1.8 in the intervention group and 76.3 ± 1.9 in the control group ($p = 0.2$). However, subgroup analyses showed that teacher physicians D-Option scores were 79.7 ± 1.5 and 73.0 ± 1.4 respectively ($p = 0.001$). More patients reported assuming an active or collaborative role in the intervention group (67.1%), than in the control group (49.2%) ($p = 0.04$). There was a significant relation between patients' and physicians' D-Option scores ($p < 0.01$) and also between patient-reported assumed roles and both D-Option scores (as assessed by patients, $p < 0.01$; and physicians, $p = 0.01$). DECISION + 2 had no impact on the intention of physicians to engage in SDM.

Conclusion: DECISION + 2 positively influenced SDM behaviors as assessed by patients and teacher physicians. Physicians' intention to engage in SDM was not affected by DECISION + 2.

Trial registration: ClinicalTrials.gov trials register no. NCT01116076.

Keywords: Shared decision making, Implementation, Theory of planned behavior, Training

* Correspondence: France.Legare@mfa.ulaval.ca
[1]Research Center of the Centre Hospitalier Universitaire de Québec, l'Hôpital St-François d'Assise, 10, Rue Espinay, Quebec City, QC, G1L 3L5, Canada
[2]Department of Family Medicine and Emergency Medicine, Université Laval, Quebec City, QC, Canada
Full list of author information is available at the end of the article

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Other information		
Registration:		
23	Registration number and name of trial registry	Registration number for pilot trial and name of trial registry
Protocol:		
24	Where the full trial protocol can be accessed, if available	Where the pilot trial protocol can be accessed, if available
Funding:		
25	Sources of funding and other support (such as supply of drugs), role of funders	
26		Ethical approval/research review committee approval confirmed with reference number

Key points

- The objectives must reflect uncertainty
- The design must be optimal to address objectives
- The analysis must link back to the objectives
- A main trial may not always be justified

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The team

Christine Bond

University of Aberdeen

Michael Campbell

University of Sheffield

Claire Chan

Queen Mary University of London

Sandra Eldridge

Queen Mary University of London

Sally Hopewell

University of Oxford

Gill Lancaster

Keele University

Lehana Thabane

McMaster University

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Thank you and questions?

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