COMED
PUSHING THE BOUNDARIES
OF COST AND OUTCOME ANALYSIS
OF MEDICAL TECHNOLOGIES

WP 3 Developing methods to assess patient reported outcome of mHealth

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Outline

1. Content and goals of the WP
2. One step back…definitions and rationale
3. SR preliminary results
4. Conclusions and next steps

Content and goals of the WP
Tasks

- TASK 1: To review and map outcomes and methodologies for the assessment of mHealth applications

- TASK 2: To test the scientific validity and relevance of the framework for the assessment of mHealth apps

- TASK 3: Policy recommendations

Background

mHealth: "Use of mobile devices – such as mobile phones, patient monitoring devices, PDAs, and wireless devices – for medical and public health practice" (GOe, 2015)

Mobile medical apps:

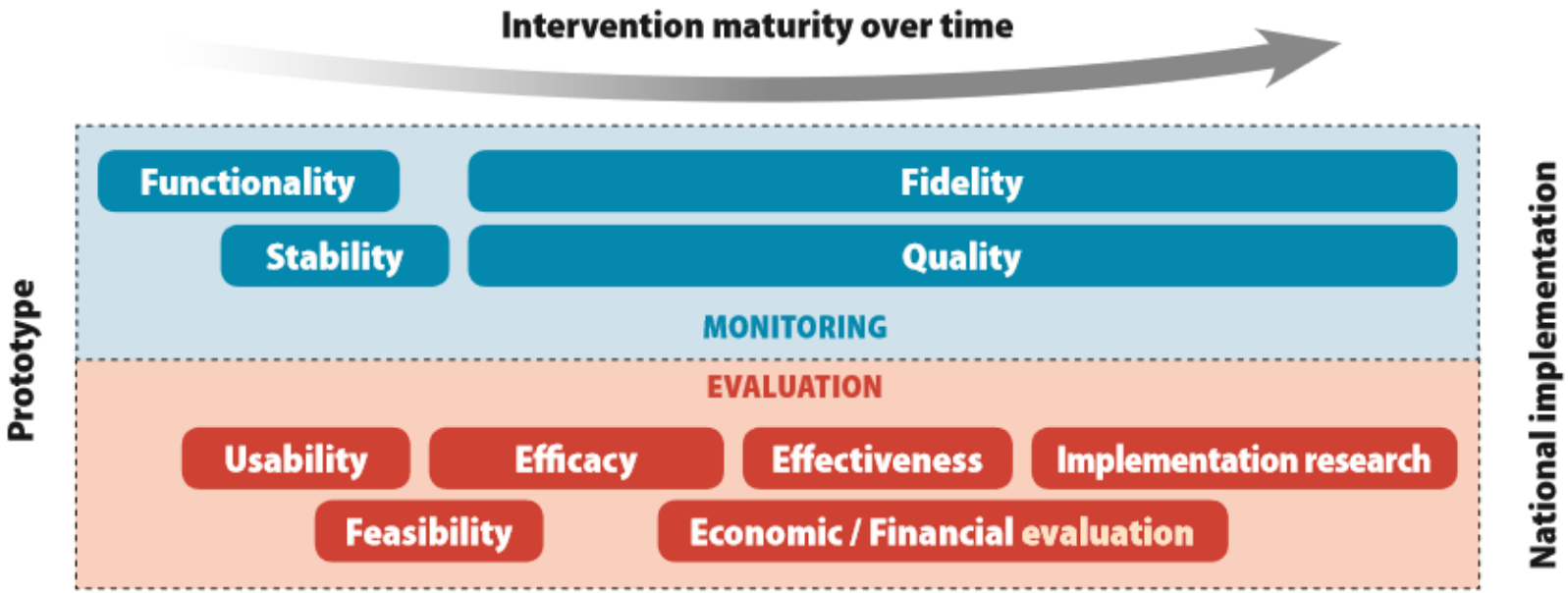
- "Mobile apps (including accessories or attachments) available on mobile platforms that have a therapeutic or diagnostic intended purpose" - no apps aiming at disease prevention, health promotion, pregnancy assessment (Ruth Moshi, 2018)
- "Medical devices that are mobile apps, meet the definition of a medical device and can transform a platform into a regulated medical device" (FDA)

Overall WP3 rationale

- Plenty of app development, but massive need for support in guiding adoption and diffusion
- Need to take into account the specificities of these technologies that need to be handled differently than we have handled any technology so far

Research objective

To develop and test/validate a framework for the assessment of mHealth apps, taking into account specific app features



Source: WHO, 2016

Previous contributions

Original review: Nasi, Cucciniello and Guerrazzi, The Performance of mHealth in Cancer Supportive Care: A Research Agenda, Journal of Medical Internet Research 2015;17(2):e9

Original Paper

The Performance of mHealth in Cancer Supportive Care: A Research Agenda

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Previous contributions

Letters

RESEARCH LETTER

Characteristics of Digital Health Studies Registered in ClinicalTrials.gov

Digital health is the application of software or hardware, often using mobile smartphone or sensor technologies to improve patient or population health and health care delivery.[1] In contrast to drugs and traditional medical devices, which have strict regulatory guidelines on safety and efficacy, the clinical evidence generation for digital health tools may be motivated by other factors, including adoption, utilization, and value, that may influence study design and quality. The landscape of clinical evidence underlying digital health interventions has not been well characterized.[2,3] We sought to evaluate the characteristics of digital health studies registered in ClinicalTrials.gov.

Invited Commentary

Whether results will drive substantial clinical adoption is unknown because small studies, even if randomized, are unlikely to be significantly powered to demonstrate meaningful treatment effects. Although the pipeline of digital health studies appears to be promising, these factors could limit their ability to yield a high level of evidence, demonstrate value, or motivate stakeholder adoption.

Systematic review of mobile health apps performance

COMED Review

Review aim: to identify all performance dimensions of mobile health apps that have been addressed by empirical studies in some of the main chronic NCDs

Specific research questions:

- What are characteristics of the included studies in terms of study design and features of mHealth apps?
- What are the outcome findings of studies?
- Which are the performance dimensions, clinical and non-clinical, on which mobile health apps have proved to have a significant impact on?

Systematic review of mobile health apps performance

COMED Review – Inclusion criteria

1. **Study design:** Empirical studies with a prospective design and a quantitative approach
2. **Participants:** Studies examining population groups with the 4 main types of chronic diseases, as identified by the World Health Organization (WHO): "four main types of non-communicable chronic diseases are cardiovascular diseases, cancers, chronic respiratory diseases (such as chronic obstructed pulmonary disease and asthma) and diabetes" (WHO, 2016)
3. **Interventions:** Of interest are all mobile interventions that focus on improving health using an app device - specialized software downloaded onto mobile devices that support their utilization such as mobile phones and tablets
4. **Outcomes:** All relevant performance dimensions and their relative outcomes were included
5. **Publication date:** Studies published from 2008 (iPhone App store and Android App store were both launched on 2008) to present (November 6, 2018)
6. **Timing, setting and language:** No restrictions based on the timing and type of setting of the retrieved studies. Only articles reported in English and Italian were included

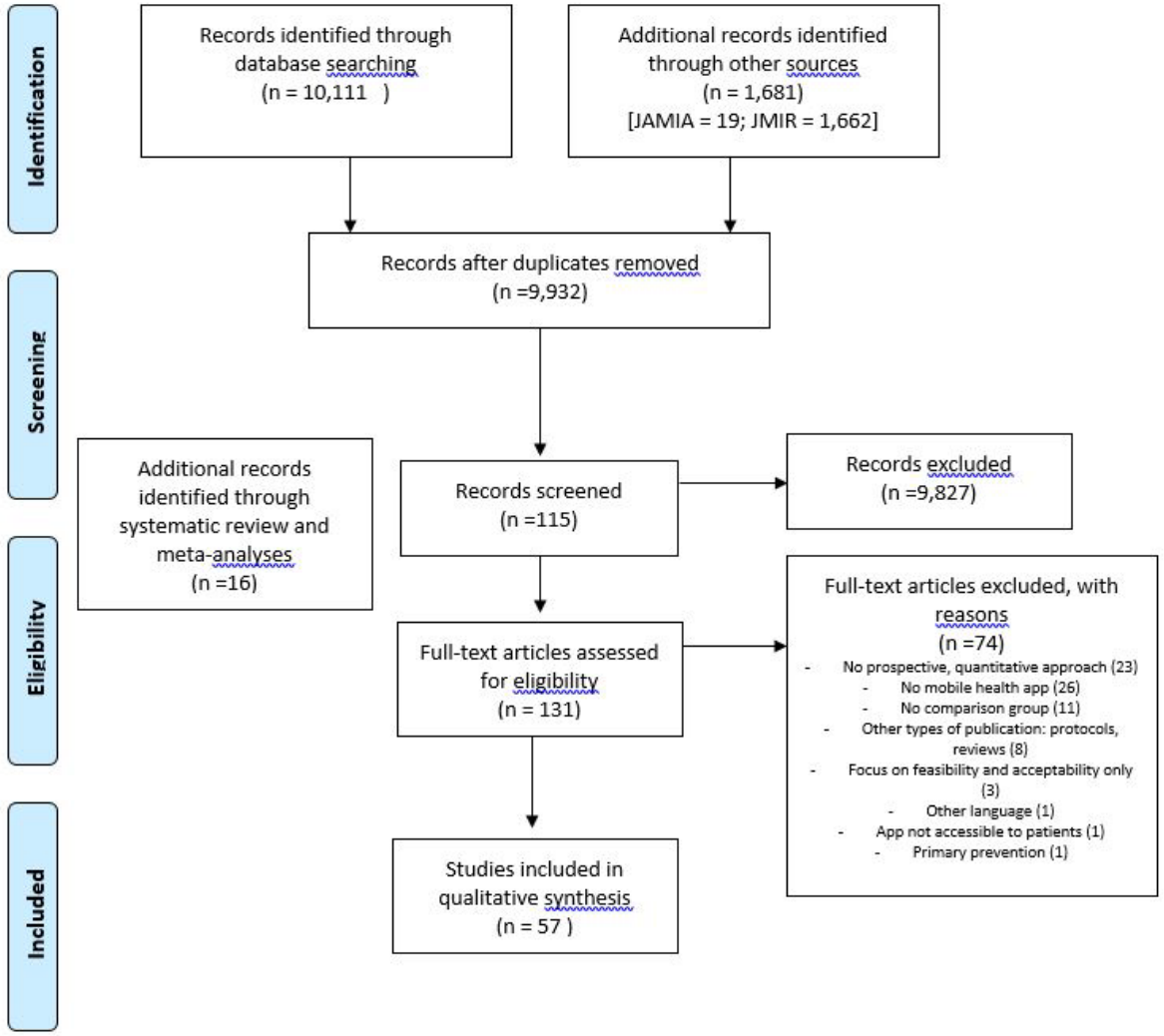
Systematic review of mobile health apps performance

COMED Review – Exclusion criteria

- Studies with **no control group**
- Studies assessing **mHealth app's feasibility and usability only,** with not even a preliminary evaluation of app efficacy
- Different study designs or publication type
- Apps not accessible to patients

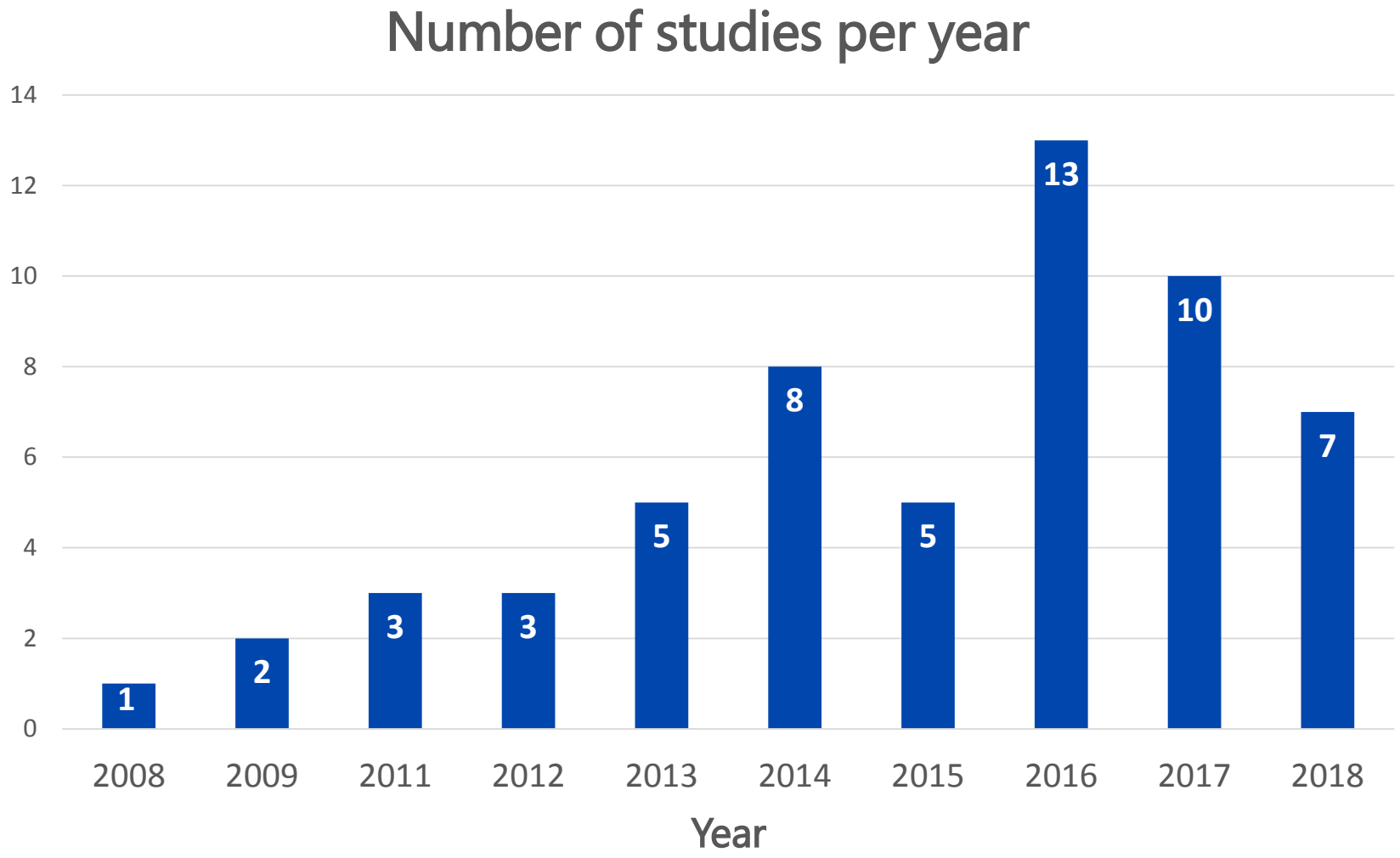
Systematic review of mobile health apps performance

PRISMA FLOWCHART

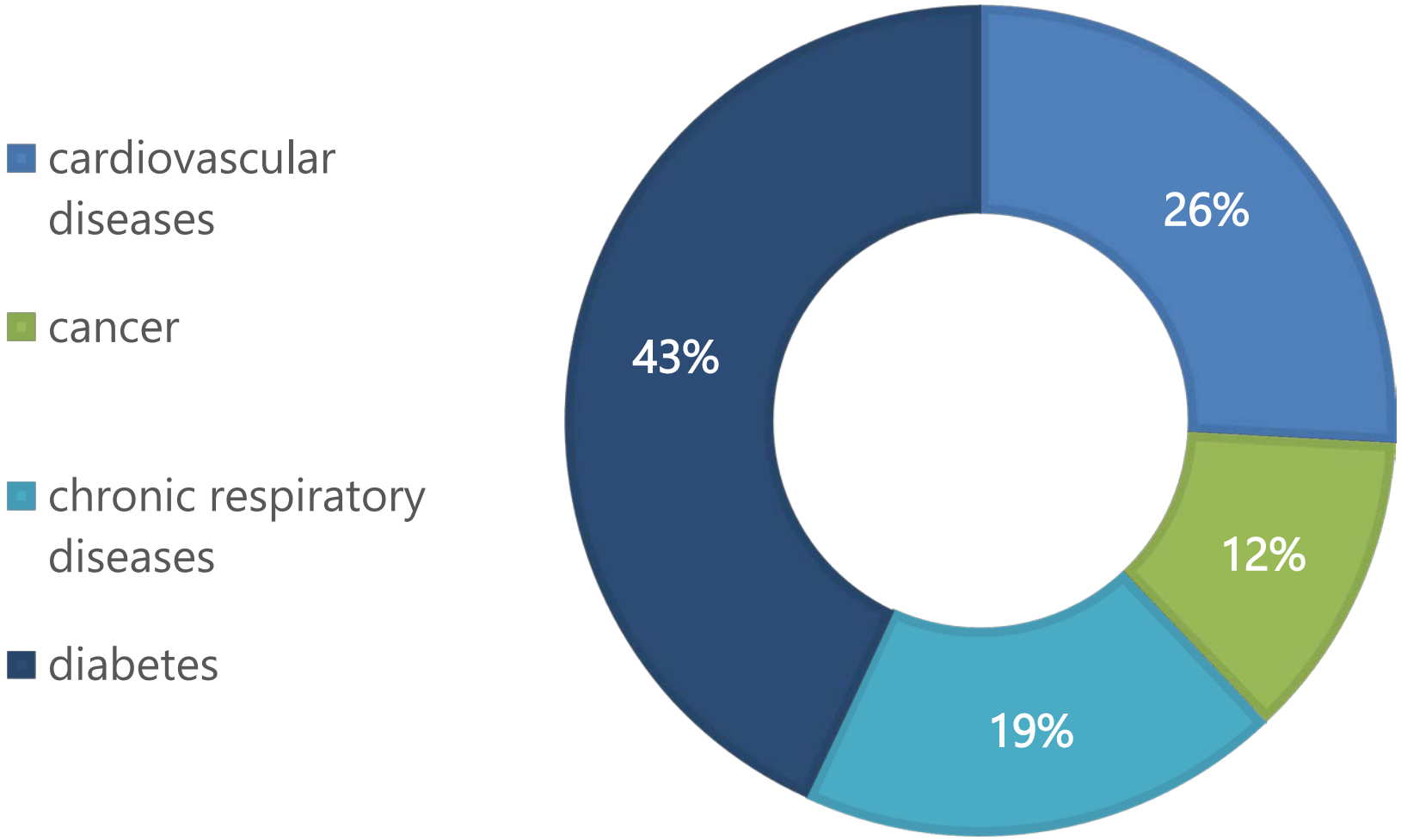


Descriptive and study design characteristics of included studies

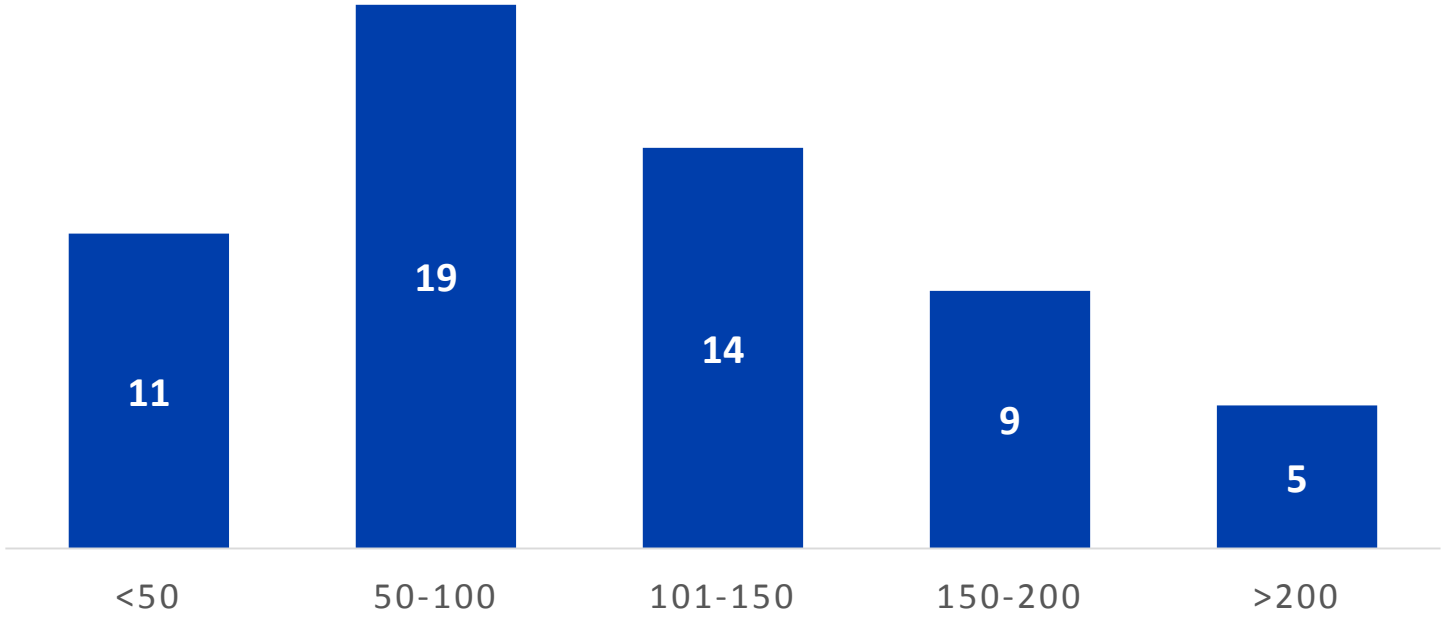
Trend of published studies



Number of studies by Chronic NCD



Sample size of the studies



Min=18
Average= 113
Max=376

	Cardiovascular diseases	Cancer	Chronic respiratory disease	Diabetes
N	13	7	12	26
Average sample size	92	127	121	120

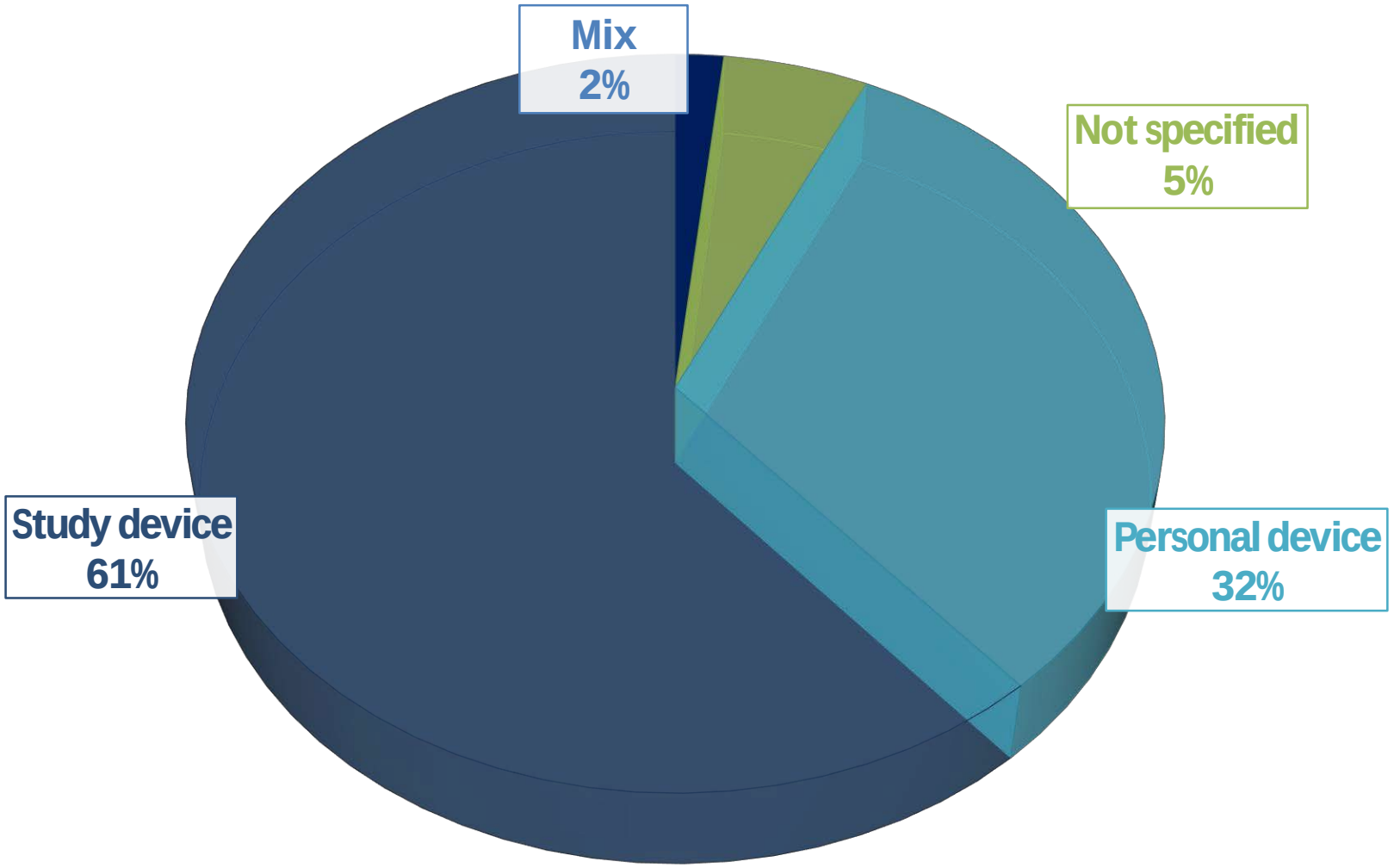
Further study characteristics

Length	Number of studies	%
Up to 1 month	4	7.0%
1-3 months	21	36.8%
4-6 months	10	35.1%
7-12 months	12	21.1%

- No innovative **study designs** (85.2% RCTs)

- Only few of the identified apps are currently available on common stores

Device used in the studies



Taxonomies for BCT and outcome findings

Outcomes taxonomy used to classify the studies

ELSEVIER

CrossMark

Journal of Clinical Epidemiology 96 (2018) 84–92

Journal of Clinical Epidemiology

Paula R. Williamson

ORIGINAL ARTICLE

A taxonomy has been developed for outcomes in medical research to help improve knowledge discovery

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Abstract

Objectives: There is increasing recognition that insufficient attention has been paid to the choice of outcomes measured in clinical trials. The lack of a standardized outcome classification system results in inconsistencies due to ambiguity and variation in how outcomes are described across different studies. Being able to classify by outcome would increase efficiency in searching sources such as clinical trial registries, patient registries, the Cochrane Database of Systematic Reviews, and the Core Outcome Measures in Effectiveness Trials (COMET) database of core outcome sets (COS), thus aiding knowledge discovery.

Study Design and Setting: A literature review was carried out to determine existing outcome classification systems, none of which were sufficiently comprehensive or granular for classification of all potential outcomes from clinical trials. A new taxonomy for outcome classification was developed, and as proof of principle, outcomes extracted from all published COS in the COMET database, selected Cochrane reviews, and clinical trial registry entries were classified using this new system

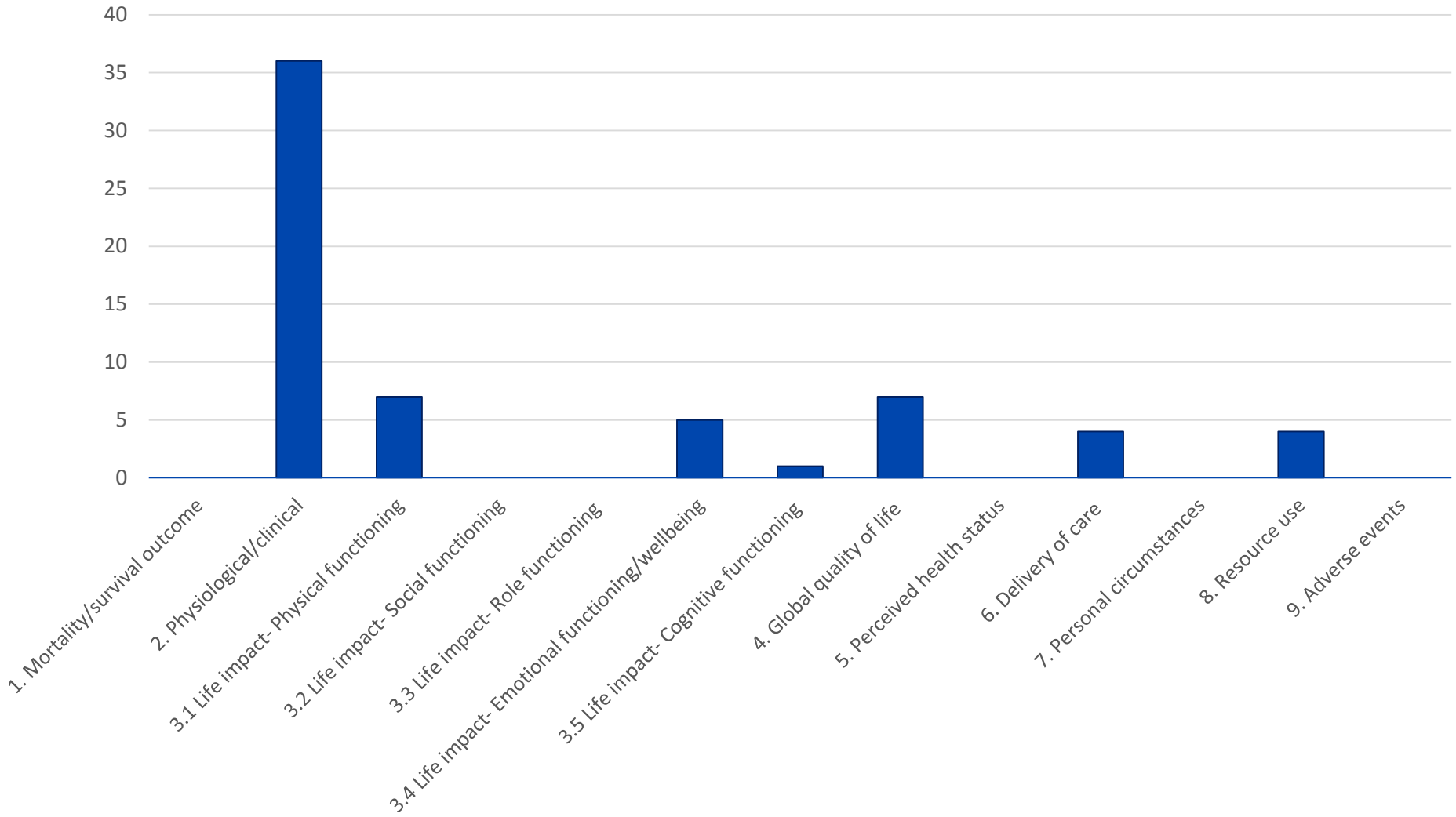
Outcomes taxonomy used to classify the studies

Table 1. Development of 38-category scale

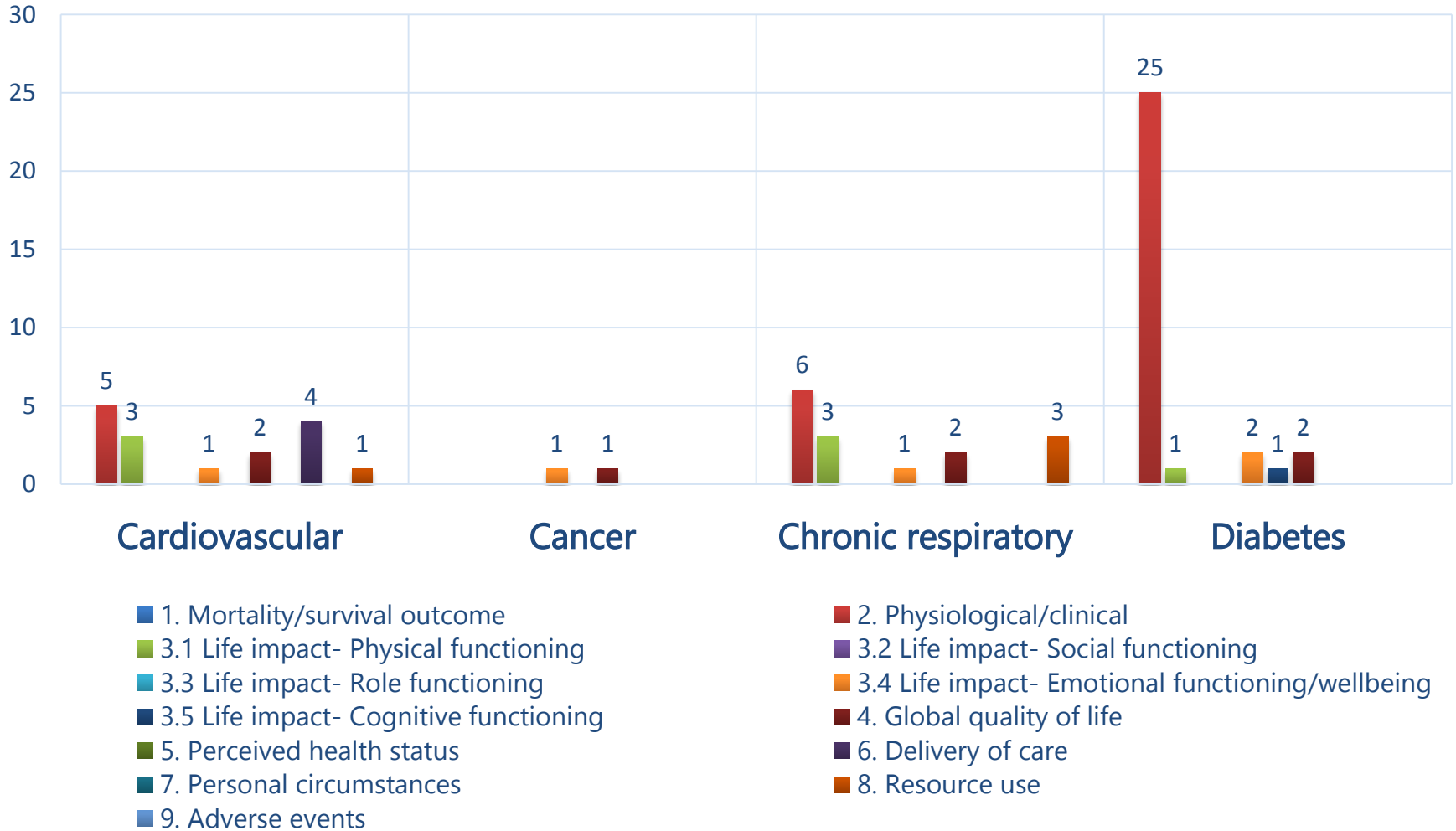
Core area	Smith	Williamson/Clarke (initial)	Williamson/Clarke (revised)
Death	1: Mortality/survival	1: Mortality/survival	1: Mortality/survival
Physiological or clinical	2: Physiological/clinical	2: Physiological/clinical	2–24: **Physiological/clinical**
	3: Infection	3: Infection	2: Blood and lymphatic system outcomes
	4: Pain	4: Pain	3: Cardiac outcomes
			4: Congenital, familial and genetic outcomes
			5: Endocrine outcomes
			6: Ear and labyrinth outcomes
			7: Eye outcomes
			8: Gastrointestinal outcomes
			9: General outcomes
			10: Hepatobiliary outcomes
			11: Immune system outcomes
			12: Infection and infestation outcomes
			13: Injury and poisoning outcomes
			14: Metabolism and nutrition outcomes
			15: Musculoskeletal and connective tissue outcomes
			16: Outcomes relating to neoplasms: benign, malignant and unspecified (including cysts and polyps)
			17: Nervous system outcomes
			18: Pregnancy, puerperium, and perinatal outcomes
			19: Renal and urinary outcomes
			20: Reproductive system and breast outcomes
			21: Psychiatric outcomes
			22: Respiratory, thoracic and mediastinal outcomes
			23: Skin and subcutaneous tissue outcomes
			24: Vascular outcomes
Life impact	5: Activities of daily living	5: Function - Physical - Social - Role	**Functioning** 25: Physical functioning 26: Social functioning 27: Role functioning
	6: Psychosocial	6: Psychosocial	28: Emotional functioning/well-being
		7: Mental health	29: Cognitive functioning
	7: QoL	8: HRQL	30: Global quality of life
			31: Perceived health status
	8: Compliance	9: Compliance (including withdrawal from treatment)	32: Delivery of care, including
	9: Withdrawal from treatment/study		- Satisfaction/patient preference - Acceptability and availability
	10: Satisfaction (patient, carer, health care provider)	10: Satisfaction	- Adherence/compliance - Withdrawal from treatment - Appropriateness of treatment - Process, implementation, and service outcomes
			33: Personal circumstances
Resource use	11: Medication	11: Resource use	**Resource use**
	12: Economic	- Economic	34: Economic
	13: Hospital	- Hospital	35: Hospital
	14: Operative	- Operative	36: Need for further intervention
		- Medication	37: Societal/carer burden
Adverse events	15: Adverse events/effects	12: Adverse events/effects	38: Adverse events/effects

Dodd, S., Clarke, M., Becker, L., Mavergames, C., Fish, R., & Williamson, P. R. (2018). A taxonomy has been developed for outcomes in medical research to help improve knowledge discovery. *Journal of clinical epidemiology*, *96*, 84-92.

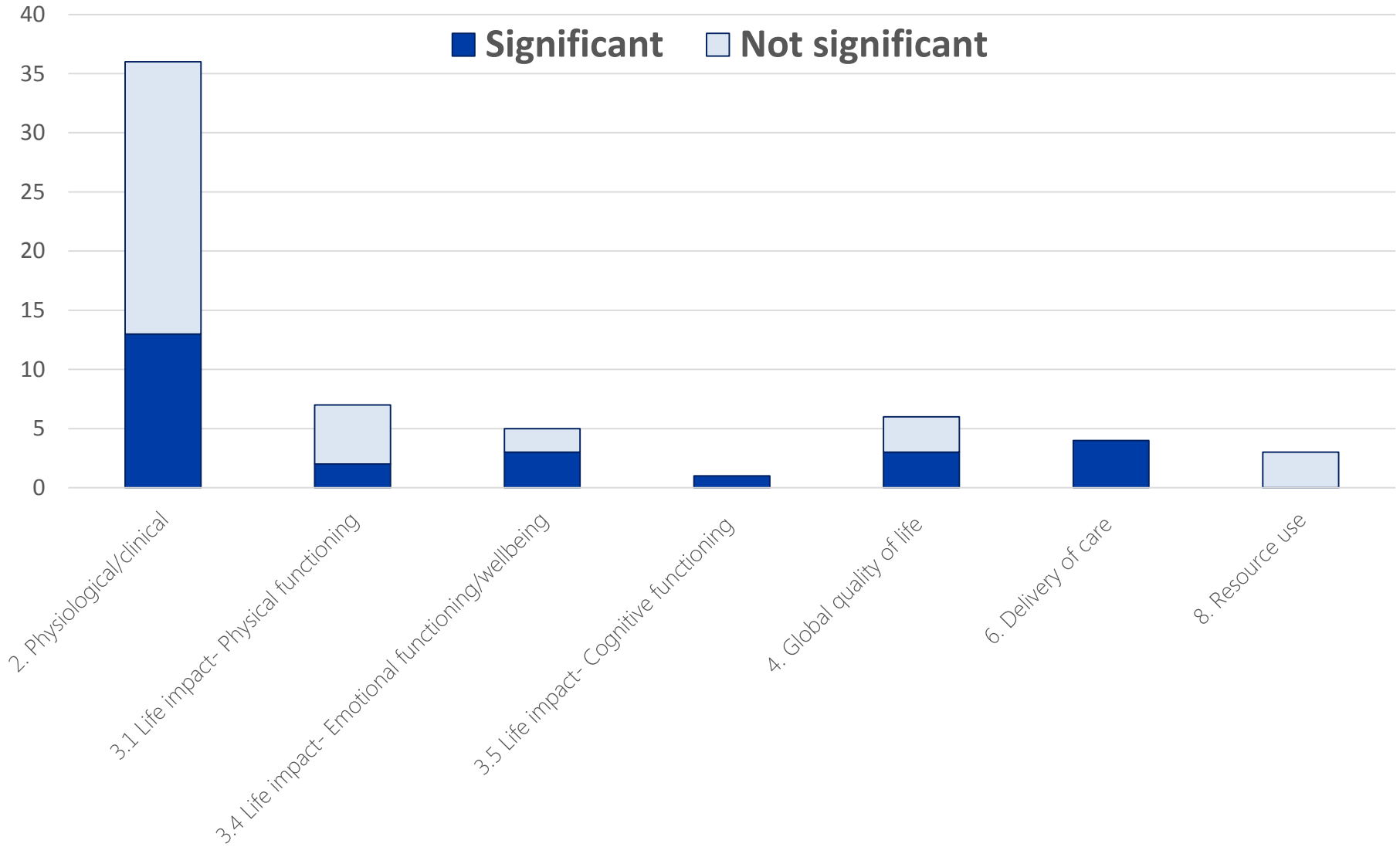
Primary outcomes- Frequency



Primary outcomes - Disease Type



Primary outcomes - Statistical significance



Content of Intervention

ORIGINAL ARTICLE

The Behavior Change Technique Taxonomy (v1) of 93 Hierarchically Clustered Techniques: Building an International Consensus for the Reporting of Behavior Change Interventions

Susan Michie, DPhil, CPsychol · Michelle Richardson, PhD · Marie Johnston, PhD, CPsychol · Charles Abraham, DPhil, CPsychol · Jill Francis, PhD, CPsychol · Wendy Hardeman, PhD · Martin P. Eccles, MD · James Cane, PhD · Caroline E. Wood, PhD

Published online: 20 March 2013

Abstract

Background CONSORT guidelines call for precise reporting of behavior change interventions: we need rigorous methods of characterizing active content of interventions with precision and specificity.

Objectives The objective of this study is to develop an extensive, consensually agreed hierarchically structured taxonomy of techniques [behavior change techniques (BCTs)] used in behavior change interventions.

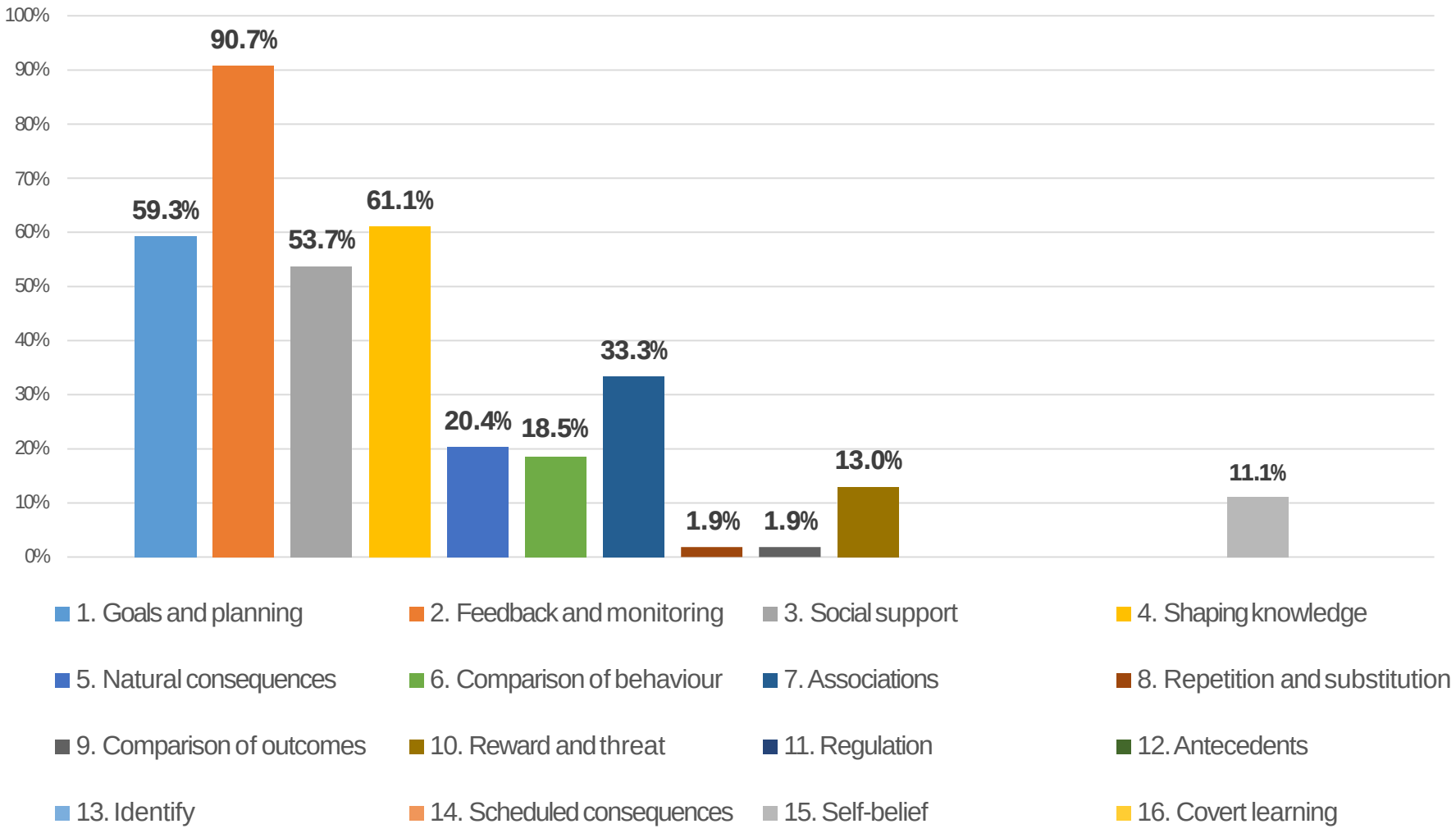
Methods In a Delphi-type exercise, 14 experts rated labels and definitions of 124 BCTs from six published classification systems. Another 18 experts grouped BCTs according to similarity of active ingredients in an open-sort task. Inter-rater agreement amongst six researchers coding 85 intervention descriptions by BCTs was assessed.

Results This resulted in 93 BCTs clustered into 16 groups. Of the 26 BCTs occurring at least five times, 23 had adjusted kappas of 0.60 or above.

Conclusions "BCT taxonomy v1," an extensive taxonomy of 93 consensually agreed, distinct BCTs, offers a step change as a method for specifying interventions, but we anticipate further development and evaluation based on international, interdisciplinary consensus.

Michie, S., Richardson, M., Johnston, M., Abraham, C., Francis, J., Hardeman, W., ... & Wood, C. E. (2013).
The behavior change technique taxonomy (v1) of 93 hierarchically clustered techniques: building an international consensus for the reporting of behavior change interventions. *Annals of behavioral medicine*, *46*(1), 81-95.

Content of Intervention – BCT categories



Risk of Bias (Revised Cochrane RoB tool)

	Randomization process	Assignment/adherence to intervention	Missing outcome	Measurement of outcome	Selection of the reported results
Quinn 2008					
Rossi 2010					
Charpentier 2011					
Quinn 2011					
Logan 2012					
Kirwan 2013					
Orsama 2013					
Rossi 2013					
Forjuoh 2014					
Torbjørnsen 2014					
Drion 2015					
Karhula 2015					
Wayne 2015					
Bee 2016					
Zhou 2016					
Baron 2017					
Goyal 2017					
Grady 2017					
Kleinman 2017					
Alanzi 2018					
Castensøe-Seidenfaden 2018					
Wang 2018					

Conclusions and next steps

- A growing body of literature: more than 10,000 records identified through database searching and 131 full-text articles assessed for eligibility
  - → However…few studies looking at "performance" of mobile health apps: only 57 studies testing the apps
  - → Many studies focus on the previous steps in the design and use of the app without measuring the effects
- Mixed results in terms of statistical significance:

  Could be due to?
  - → Study design features (**small sample sizes** – 113 patients on average; **short duration of interventions** - almost 80% of studies under 6 months of follow-up (insufficient time horizon?); **traditional study design** – no adoption of innovative designs)
  - → Type and content of the apps
  - → Devices used
  - → Attrition problems (constraints in demonstrating the long term usage of the apps by both patients and clinicians)

Conclusions and next steps

- Primary Outcomes are mainly “clinical”: 36 studies show physiological primary outcomes
  - → The real potential of apps with respect to NON clinical types of outcome still has to be proved

- As a results of these issues, generalizability of findings is weak and rarely addressed in the study discussion

- Coming up:
  - → Analysis of app features that may impact on outcomes
  - → Final drafting of the evaluation framework for mHealth apps
  - → Testing and validation of the framework