

BMJ Open is committed to open peer review. As part of this commitment we make the peer review history of every article we publish publicly available.

When an article is published we post the peer reviewers' comments and the authors' responses online. We also post the versions of the paper that were used during peer review. These are the versions that the peer review comments apply to.

The versions of the paper that follow are the versions that were submitted during the peer review process. They are not the versions of record or the final published versions. They should not be cited or distributed as the published version of this manuscript.

BMJ Open is an open access journal and the full, final, typeset and author-corrected version of record of the manuscript is available on our site with no access controls, subscription charges or pay-per-view fees (<u>http://bmjopen.bmj.com</u>).

If you have any questions on BMJ Open's open peer review process please email <u>info.bmjopen@bmj.com</u>

BMJ Open

Research quality and dissemination of paediatric randomized controlled trials with and without patient and family engagement: systematic review

Journal:	BMJ Open
Manuscript ID	bmjopen-2024-086934
Article Type:	Original research
Date Submitted by the Author:	26-Mar-2024
Complete List of Authors:	Borkhoff, Cornelia; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health Hattangadi, Nayantara ; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Nurse, Kimberly ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Kay, Tatjana; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Bhalla, Manav ; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Bhalla, Manav ; Hospital for Sick Children, Division of Hospital Library and Archives Buchanan, Francine; The Hospital for Sick Children, Child Health and Evaluative Sciences Taljaard, Monica; Ottawa Hospital Research Institute, Clinical Epidemiology Program Cohen, Eyal; Hospital for Sick Children Research Team (PORT) ; Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; Hospital for Sick Children Research Team (PORT) ; University of Toronto, Department of Paediatrics, Temerty Faculty of Medicine Macarthur, Colin; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences
Keywords:	PAEDIATRICS, Child, Social Media, Randomized Controlled Trial
	*

SCHOLARONE[™] Manuscripts



I, the Submitting Author has the right to grant and does grant on behalf of all authors of the Work (as defined in the below author licence), an exclusive licence and/or a non-exclusive licence for contributions from authors who are: i) UK Crown employees; ii) where BMJ has agreed a CC-BY licence shall apply, and/or iii) in accordance with the terms applicable for US Federal Government officers or employees acting as part of their official duties; on a worldwide, perpetual, irrevocable, royalty-free basis to BMJ Publishing Group Ltd ("BMJ") its licensees and where the relevant Journal is co-owned by BMJ to the co-owners of the Journal, to publish the Work in this journal and any other BMJ products and to exploit all rights, as set out in our <u>licence</u>.

The Submitting Author accepts and understands that any supply made under these terms is made by BMJ to the Submitting Author unless you are acting as an employee on behalf of your employer or a postgraduate student of an affiliated institution which is paying any applicable article publishing charge ("APC") for Open Access articles. Where the Submitting Author wishes to make the Work available on an Open Access basis (and intends to pay the relevant APC), the terms of reuse of such Open Access shall be governed by a Creative Commons licence – details of these licences and which <u>Creative Commons</u> licence will apply to this Work are set out in our licence referred to above.

Other than as permitted in any relevant BMJ Author's Self Archiving Policies, I confirm this Work has not been accepted for publication elsewhere, is not being considered for publication elsewhere and does not duplicate material already published. I confirm all authors consent to publication of this Work and authorise the granting of this licence.

Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.	MJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Departmen
---	--

Research quality and dissemination of paediatric randomized controlled trials with and without
patient and family engagement: systematic review
Cornelia M. Borkhoff, MSc, PhD ^{1,2,3} Nayantara Hattangadi, MHSc ³ , Kimberly M. Nurse, MSc ^{2,3} , Tatjana Kay, MSc ³ , Manav Bhalla, BSc ³ , Quenby Mahood, MI ⁴ , Francine Buchanan, MLiS, PhD ³ , Monica Taljaard, MSc, PhD, ^{5,6} , Eyal Cohen, MD, MSc, ^{1,2,3,7,8} Patricia C. Parkin, MD ^{1,2,3,7} , Colin Macarthur, MBChB, PhD ^{1,2,3,7}
 Affiliations: ¹ Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT), Hospital for Sick Children, Toronto, Ontario, Canada; ² Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health, University of Toronto, Toronto, Ontario, Canada; ³ Child Health Evaluative Sciences, SickKids Research Institute, Toronto, Ontario, Canada; ⁴ Division of Hospital Library and Archives, Hospital for Sick Children, Toronto, Ontario, Canada; ⁵ Clinical Epidemiology Program, Ottawa Hospital Research Institute (OHRI), The Ottawa Hospital, General Campus, 501 Smyth Road, Ottawa, Ontario, Canada; ⁶ School of Epidemiology and Public Health, University of Ottawa, Ottawa, Ontario, Canada; ⁷ Department of Paediatrics, Temerty Faculty of Medicine, University of Toronto, Toronto, Ontario, Canada; ⁸ Edwin S.H. Leong Centre for Healthy Children, University of Toronto, Toronto, ON M5G 0A4, Canada
Address correspondence to: Cornelia M. Borkhoff, PhD, The Hospital for Sick Children Research Institute, Peter Gilgan Centre for Research and Learning, 686 Bay St, Toronto, ON, Canada M5G 0A4. E-mail: <u>cory.borkhoff@sickkids.ca</u>
Short title: Patient engagement, quality and dissemination of paediatric randomized controlled trials
Keywords: patient engagement; paediatrics; child; randomized controlled trials; research quality; dissemination; social media

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

Objectives: While there is good evidence of a positive impact of patient and family engagement on the research process, there are few empirical data on the impact of patient and family engagement on research quality and dissemination. The objective of this study was to compare research quality and dissemination metrics for paediatric randomized controlled trials (RCTs) that engaged patients and families in the research, with trials that did not.

Design and setting: Paediatric RCTs involving children and youth (<18 years of age) and published in a peer-reviewed general medical journal (*The BMJ*) from 2011 to 2020 were identified using an Ovid MEDLINE search strategy. Trials were categorized as those engaging patients and families (PE+) and those that did not (PE-). Two reviewers screened trials for eligibility, extracted data, and assessed research quality using the modified Cochrane Risk of Bias Tool. Dissemination metrics were determined using measures of academic and non-academic citation.

Results: Of 45 paediatric RCTs, only 10/45 RCTs (22%) reported engaging patients and families in the research process. Research quality for PE+ and PE- paediatric RCTs was similar; 4/10 (40%) of PE+ trials, and 13/35 (37%) of PE- trials were rated as 'fair' or 'good' (p=1.00). Academic citation frequency per year was similar for PE+ and PE- trials: Web of Science (median 6.6 versus 7.1, respectively; p=0.84). Non-academic dissemination measures were generally higher among PE+ trials, for example, median PlumX Social Media score per year for PE+ trials was 46.6, compared with a median score of 7.6 for PE- trials (p=0.02).

Conclusions: Few paediatric RCTs reported patient engagement activity. Research quality was similar for trials engaging patients and families compared with those that did not. Patient and

1 ว	
3	family engagement in the trial however, was associated with higher metrics for social media
4	family engagement in the trial, nowever, was associated with higher metrics for social media
5	attention, compared with trials with no engagement.
7	
8	
9 10	
11	
12	
13 14	
15	
16	
17	
18 19	
20	
21	
22	
24	
25	
26 27	
27	
29	
30	
31	
33	
34	
35 36	
37	
38	
39	
40	
42	
43	
44 45	
46	
47	
48 49	
50	
51	
52	
53 54	
55	
56	
57 58	3
59	
60	For peer review only - http://bmjopen.bmj.com/site/about/guidelines.xhtml

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

BMJ Open

Strengths and limitations of this study

- While there is good evidence of a positive impact of patient engagement on the research process, on research teams, and on patient partners, there are few quantitative data on the impact of patient and family engagement on research quality and dissemination.
- We performed a systematic review and compared research quality and dissemination metrics for paediatric randomized controlled trials (RCTs) published in *The BMJ* that engaged patients and families in the research process with trials that did not.
- We only sampled paediatric RCTs published in one journal, but *The BMJ* was selected deliberately, given the requirement of submitting authors to report patient and public involvement in their research.
- RCTs were categorized as those engaging patients and families (PE+) and those that did not (PE-) based on information reported in the published article (and/or study protocol where available). There may have been misclassification of trials, particularly those trials published prior to *The BMJ* policy in 2015 that mandated reporting of patient engagement.

BMJ Open

INTRODUCTION

Patient-oriented research has been defined as a continuum of research that engages patients as equal partners, focuses on patient-identified priorities and outcomes, and integrates the knowledge generated into policy and practice to improve health care outcomes.¹ The term 'patient' is overarching and includes those with lived experience of a health issue, as well as informal caregivers, such as family and friends. Patient-oriented research is hypothesized to improve the quality, relevance, and uptake of health research.²

Authentic patient engagement in health research involves an equal partnership between patients and researchers working together on any or all part(s) of the research process, including choosing the research question, selecting the study design, tailoring the intervention, devising study procedures, and dissemination of study findings. Barriers to patient engagement, however, such as parenting commitments, work schedules, and long research timelines, can make patient and family engagement in child health research challenging.³

Aubin *et al*⁴ proposed a framework for measuring *impact* related to patient-oriented research. The framework identifies potential impact metrics across four domains: improvements to research process, impact on policies, impact on health outcomes, and contribution to social change. In the context of child health research, there is qualitative evidence of a positive impact of patient and family engagement on the research process (refinement of the research question, intervention, materials), on research teams (enhanced knowledge, cultural competency), and on patient partners (empowerment, skills development).⁵⁻¹⁰ There are, however, few quantitative data on the impact of patient-oriented research, and to our knowledge, the impact of patient and family engagement on research quality and dissemination has not been examined.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

Therefore, the objective of this study was to conduct a systematic review of paediatric RCTs published in *The BMJ* (a peer-reviewed general medical journal with a high impact factor), examine measures of research quality and dissemination (academic and non-academic), and compare RCTs that reported engaging patients and families in the research process with RCTs that did not.

METHODS

This study followed the Preferred Reporting Items for Systematic Reviews and Metaanalyses (PRISMA) reporting guidelines.¹¹ We did not register a protocol prior to conducting the review.

Search Strategy and Information Sources

An information science specialist (QM) developed an Ovid MEDLINE electronic search strategy to identify RCTs involving children and youth (<18 years of age) published in *The BMJ* over the 10-year period January 01, 2011, through to December 31, 2020. The search strategy was optimized for sensitivity and specificity using The Cochrane Highly Sensitive Search to identify RCTs.¹² A search end date of 2020 was chosen, given the widespread impact of the COVID-19 pandemic on health research. **Supplementary Table 1** describes the search strategy. **Inclusion and Exclusion Criteria**

RCTs published in *The BMJ* over the 10-year period that assessed a specific intervention were eligible. Only primary reports of trial results related to the trial primary outcome were included, i.e., publications reporting a secondary analysis of RCT data were excluded. Trials were included if the study population was limited to children and youth from birth to less than 18 years of age. Excluded were clinical trial study protocols; non-randomized comparative trials;

BMJ Open

Study Selection and Data Extraction

A standardized review form was developed to confirm trial eligibility and extract data on study characteristics. Two reviewers (two of NH, KMN, TK, MB) independently performed an eligibility assessment for each article using the inclusion and exclusion criteria, first screening titles and abstracts and then full texts of potentially relevant articles. Any discrepancies were resolved through discussion and adjudication with a third reviewer (CMB).

For this review, patient and family engagement (as previously defined)¹⁻³ was considered the 'exposure of interest' and research quality and dissemination were the 'outcomes of interest.' Therefore, trials were categorized into two groups: the PE+ group (trials that reported engaging patients and families in the research process) and the PE- group (trials that <u>did not report</u> engaging patients and families in the research process). Recognizing that information on patient and family engagement might not necessarily be reported in *The BMJ* publication, we also reviewed information in published trial protocols (where available), and clinical trial registries such as ClinicalTrials.gov.

Two reviewers independently extracted data on RCT characteristics and methods, including author name; year of publication; trial setting; trial type; multicenter (yes/no); multinational (yes/no); participant age; primary outcome; sample size; number lost to follow-up; and patient and/or family engagement (yes/no). For the PE+ trials, additional data were collected: number of patient/family/caregivers engaged; youth engagement (yes/no); and area of engagement in the research process.

Outcomes

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

'Research quality' was assessed using the modified Cochrane Risk of Bias Tool for RCTs.¹³ The Cochrane tool appraises 7 methodological quality criteria: 1) random sequence generation (selection bias); 2) allocation concealment (selection bias); 3) selective reporting (reporting bias); 4) other bias; 5) blinding of participants and researchers (performance bias); 6) blinding of outcome assessment (detection bias); and 7) incomplete outcome data (attrition bias). Two reviewers independently evaluated the research quality of eligible trials, and any discrepancies were resolved through discussion with a third reviewer. An overall quality rating for each trial based on a summation of the 7 criteria was also determined; trials were categorized as 'Good,' 'Fair,' or 'Poor,' based on the Risk of Bias Tool guide.

Dissemination of trial results was determined using measures of academic and nonacademic citation. Data on citation numbers and citation frequencies from Web of Science and Scopus were collected. Web of Science has a narrower coverage of biomedical journals than Scopus and may therefore give a more conservative citation count.¹⁴ The primary academic dissemination metric captured was citation frequency per year (citation count divided by the number of years since publication).

PlumX data from Scopus (<u>https://plumanalytics.com/learn/about-metrics/</u>) and altmetric data from Web of Science (<u>https://www.altmetric.com/research-access/</u>) were used to determine non-academic citation.¹⁵ PlumX data capture interactions with a research output in the online environment across five domains: Citations (indexes, clinical or policy citations); Usage (URL clicks, downloads, views); Captures (bookmarks, favourites, follows); Mentions (news media, blog posts, Wikipedia references); and Social Media (shares, likes, comments). The Altmetric Attention Score is a weighted count of the public attention a research article has received based on a variety of sources (citations, news media, social media mentions, blogs etc.). PlumX and

BMJ Open

Altmetric Attention Scores were described by year (total interactions divided by the number of years since publication of the trial).

Statistical Analysis

Descriptive statistics were used to describe the frequency of paediatric RCTs published in *The BMJ* (by calendar year and patient engagement) and other RCT characteristics. PE+ and PE-trials were compared on research quality and dissemination metrics using Fisher's Exact test for categorical outcomes and the Mann-Whitney test for continuous variables. Statistical significance was defined as p<0.05; all statistical tests were 2-sided.

Ethics

Ethics approval for the study was not required as no data were collected from human subjects and only peer-reviewed published studies in the public domain were reviewed.

Patient and public involvement

This systematic review was conducted in partnership with a patient partner (FB) who provided input on framing the research question, research methods, interpreting the research findings, writing the manuscript, and preparing dissemination plans.

RESULTS

The literature search strategy (see **Supplementary Table 1**) identified 29,944 citations in *The BMJ* up to 2020, with 818 (2.7%) classified as potential RCTs. Of these, 132 (16%) were indexed as including children, and were assessed for eligibility. After review, 87 records were excluded, leaving 45 paediatric trials included in the review (the search flowchart and reasons for exclusion are described in **Supplementary Figure 1**).

Table 1. Number of paediatric RCTs published in *The BMJ* by patient engagement status and year of publication

Over the j	period 2	011 to 2	2020, <i>Th</i>	he BMJ	publish	ed 45 R	CTs inv	olving o	children	and	
youth. ¹⁶⁻⁶⁰ As sho	own in T	able 1,	only 10) of the	45 RCT	s (22%)) reporte	ed engag	ging pat	ients an	d
families in the tri	al. (Add	litional	informa	tion on	the 10 F	PE+ trial	ls is pro	vided in	Supple	ementa	ry
Table 2). In align	nment w	ith the 2	2015 ma	andate f	rom The	e <i>BMJ</i> in	n <i>Instru</i> d	ctions to	o Author	rs, all	
seven of the PE+	trials pu	ublished	l from 2	015 onv	wards in	cluded	a Patien	t and P	ublic In	volveme	ent
(PPI) paragraph i	n the M	ethods s	section of	of the m	anuscri	pt. Of th	ne PE- p	aediatri	c trials,	seven	
were published ir	n 2015 o	r later, I	howeve	r, only t	hree inc	cluded a	PPI par	agraph;	the fou	r trials	
without a PPI par	ragraph	were all	l publish	ned in 2	015. Ful	ll imple	mentatio	on of the	e new re	eporting	
policy may have	been slo	wer tha	n antici	pated.							
1 5 5				1							
able 1. Number of pae	ediatric R	CTs put	blished in	n <i>The BN</i>	∕∕J by pa	tient eng	gagement	t status a	nd year	of public	cation
		0010	2013	2014	2015	2016	2017	2018	2019	2020	2011-2020
BMJ	2011	2012	2010				-				
BMJ Published RCTs	2011 11	10	4	6	6	0	1	3	3	1	45
BMJ Published RCTs PE+	2011 11 1	2012 10 1	4 0	6 1	6 1	0 0	1 1	3 2	3 2	1 1	45 10
BMJ Published RCTs PE+ PPI paragraph, yes	2011 11 <i>1</i> 0	2012 10 1 0	4 0 0	6 1 0	6 1 1	0 0 0	1 1 1	3 2 2	3 2 2	1 1 1	45 10 7
BMJPublished RCTsPE+PPI paragraph, yesPPI paragraph, no	2011 11 1 0 1	2012 10 1 0 1	4 0 0 0	6 1 0 1	6 1 1 0	0 0 0 0	1 1 1 0	3 2 2 0	3 2 2 0	1 1 1 0	45 10 7 3
BMJPublished RCTsPE+PPI paragraph, yesPPI paragraph, noPE -	2011 11 1 0 1 10	2012 10 1 0 1 9	4 0 0 0 4	6 1 0 1 5	6 1 1 0 5	0 0 0 0 0	1 1 1 0 0	3 2 2 0 1	3 2 2 0 1	1 1 1 0 0	45 10 7 3 35
BMJPublished RCTsPE+PPI paragraph, yesPPI paragraph, noPE -PPI paragraph, yes	2011 11 1 0 1 10 0	2012 10 1 0 1 9 0	4 0 0 0 0 0 0 0 0 0	6 1 0 1 5 0	6 1 1 0 5 1	0 0 0 0 0 0	1 1 0 0 0	3 2 2 0 1 1	3 2 2 0 1 1	1 1 0 0 0	45 10 7 3 35 3 3
BMJPublished RCTsPE+PPI paragraph, yesPPI paragraph, noPE -PPI paragraph, yesPPI paragraph, no	2011 11 1 0 1 10 0 10 10	2012 10 1 0 1 9 0 9	$ \begin{array}{c} 2010 \\ 4 \\ 0 \\ $	6 1 0 1 5 0 5	6 1 0 5 1 4	0 0 0 0 0 0 0	1 1 0 0 0 0	3 2 2 0 1 1 0	3 2 2 0 1 1 0	1 1 0 0 0 0	45 10 7 3 35 3 32
BMJ Published RCTs PE+ PPI paragraph, yes PPI paragraph, no PE - PPI paragraph, yes PPI paragraph, yes PPI paragraph, no As described and families were one aspect of the	2011 11 1 0 1 10 0 10 bed in F e engage study. F	2012 10 1 0 1 9 0 9 igure 1 ed acros	4 0 0 4 0 4 0 4 s the en nple, of	6 1 0 1 5 0 5 ppleme tire rese	6 1 0 5 1 4 entary T earch pro- PE+ RC	0 0 0 0 0 0 0 5 able 2, 5 ccess, at Ts, 8/10	1 1 0 0 0 0 among nd often 0 (80%)	3 2 0 1 1 0 the 10 H involve	$\begin{array}{c} 3\\ 2\\ 0\\ 1\\ 1\\ 0\\ \end{array}$ PE+ tria ed in mo	1 1 0 1 1 1 1 1 0 0 0 0 0 0 0 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 <td< td=""><td>45 10 7 3 35 3 32 ents</td></td<>	45 10 7 3 35 3 32 ents
BMJ Published RCTs PE+ PPI paragraph, yes PPI paragraph, no PE - PPI paragraph, yes PPI paragraph, yes PPI paragraph, yes PPI paragraph, no As descrift and families were one aspect of the input on the development	2011 11 1 0 1 10 0 10 bed in F e engage study. F	2012 10 1 0 1 9 0 9 igure 1 ed across For example to f the state	4000404and Sus the ennple, ofinterven	6 1 0 1 5 0 5 ppleme tire rese the 10 1 ation, an	6 1 0 5 1 4 earch property T earch property T PE+ RC ad 5/10 (0 0 0 0 0 0 5 able 2 , 50%) h	1 1 0 0 0 0 among nd often 0 (80%) ad input	$\begin{array}{c} 3 \\ 2 \\ 2 \\ 0 \\ 1 \\ 1 \\ 0 \end{array}$ the 10 H involve had pate to the	320110PE+ triaed in modelient anddissemi	1 1 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 1 0 0 0 0 0 0 1 0 1 <td< td=""><td>45 10 7 3 35 35 32 ents</td></td<>	45 10 7 3 35 35 32 ents
BMJ Published RCTs PE+ PPI paragraph, yes PPI paragraph, no PE - PPI paragraph, yes PPI paragraph, yes PPI paragraph, no As descrift and families were one aspect of the input on the deve trial results. Only	2011 11 1 0 1 10 0 10 10 bed in F e engage study. F lopment	$\begin{array}{c} 2012 \\ 10 \\ 1 \\ 0 \\ 1 \\ 9 \\ 0 \\ 9 \\ 0 \\ 9 \\ 0 \\ 9 \\ 0 \\ 0 \\ 9 \\ 0 \\ 0$	4 0 0 4 0 4 and Su s the en nple, of interven	6 1 0 1 5 0 5 ppleme tire rese the 10 1 ntion, an patients	6 1 0 5 1 4 earch pro PE+ RC od 5/10 (s and fa:	0 0 0 0 0 0 5 able 2 , 5 0 5 5 8/10 (50%) h milies in	1 1 0	$\begin{array}{c} 3 \\ \hline 2 \\ \hline 0 \\ \hline 1 \\ \hline 0 \\ \end{array}$ the 10 H involve had pate to the pping the	3 2 0 1 1 0 PE+ tria ed in mo ient and dissemi e resear	1 1 0 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 <td< td=""><td>45 10 7 3 35 3 32 ents</td></td<>	45 10 7 3 35 3 32 ents
BMJ Published RCTs PE+ PPI paragraph, yes PPI paragraph, no PE - PPI paragraph, yes PPI paragraph, yes PPI paragraph, no As descrift and families were one aspect of the input on the deve trial results. Only question. The three	2011 11 1 0 1 10 0 10 bed in F e engage study. F clopment r one PE ee PE+ 1	$\frac{2012}{10}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$	$\begin{array}{c} 2010 \\ \hline \\ 4 \\ \hline \\ 0 \\ \hline \\ 0 \\ \hline \\ 4 \\ \hline \\ 0 \\ \hline \\ 4 \\ \hline \\ and Su \\ \hline \\ s the en \\ nple, of \\ interven \\ engaged \\ nat inclu$	6 1 0 1 5 0 5 ppleme tire rese the 10 1 ntion, and patients ided par	6 1 0 5 1 4 earch property T earch property T pE+ RC of 5/10 (s and faither the second se	0 0 0 0 0 0 0 5 able 2 , 5 0 5 5 8/10 (50%) h milies in s 12 to 1	1 1 0	$\frac{3}{2}$ $\frac{2}{0}$ $\frac{1}{1}$ $\frac{1}{0}$ $\frac{1}$	3 2 0 1 1 0 PE+ tria ed in mo ient and dissemi e researd include	1 1 0 <td< td=""><td>45 10 7 3 35 3 32 ents</td></td<>	45 10 7 3 35 3 32 ents
BMJ Published RCTs PE+ PPI paragraph, yes PPI paragraph, no PE - PPI paragraph, yes PPI paragraph, yes PPI paragraph, yes PPI paragraph, no As descrift and families were one aspect of the input on the deve trial results. Only question. The three engagement of you	2011 11 1 0 1 10 0 10 0 10 0 10 0 10 0 10 0 10 bed in F e engage study. F clopment r one PE ee PE+ 1 outh part	$\frac{2012}{10}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$	and Su and Su and Su s the en nple, of interven engaged hat inclu 48,59 Info	6 1 0 1 5 0 5 ppleme tire rese the 10 1 ation, and patients uded particular	$\begin{array}{c} 6 \\ 1 \\ 0 \\ 5 \\ 1 \\ 4 \end{array}$ entary T earch property PE+ RC ed 5/10 (s and faither the second se	0 0 0 0 0 0 0 0 0 0 0 0 0 0	1 1 0 17 17 0 0 0	$\frac{3}{2}$ $\frac{2}{0}$ $\frac{1}{1}$ $\frac{1}{0}$ $\frac{1}$	3 2 0 1 1 0 PE+ tria ed in mo ient and dissemi e researd include	1 1 0 <td< td=""><td>45 10 7 3 35 3 32 ents</td></td<>	45 10 7 3 35 3 32 ents
BMJ Published RCTs PE+ PPI paragraph, yes PPI paragraph, no PE - PPI paragraph, yes PPI paragraph, yes PPI paragraph, yes PPI paragraph, no As descril and families were one aspect of the input on the deve trial results. Only question. The thre engagement of yo engaged was prov	20111110110010bed in Fe engagestudy. Felopmentr one PEee PE+1outh partvided by	$\frac{2012}{10}$ $\frac{1}{7}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$ $\frac{1}{9}$ $\frac{9}{9}$ $\frac{1}{9}$	$\begin{array}{c} 2010 \\ \hline \\ 4 \\ \hline \\ 0 \\ \hline \\ 0 \\ \hline \\ 4 \\ \hline \\ 0 \\ \hline \\ 0 \\ \hline \\ 4 \\ \hline \\ 0 \\ \hline \\ 0 \\ \hline \\ 4 \\ \hline \\ 0 \\ \hline 0 \\ \hline \\ 0 \\ \hline 0 $	6 1 0 1 5 0 5 ppleme tire rese the 10 b ation, and patients ided par priation trial; in	$\begin{array}{c} 6 \\ 1 \\ 0 \\ 5 \\ 1 \\ 4 \end{array}$ entary T earch pro PE+ RC d 5/10 (s and fat ticipant n on the that tria	0 0 0 0 0 0 0 0 0 5 able 2, 0 cess, at Ts, 8/10 (50%) h milies in s 12 to 1 number al parent	1 1 0 17 years r of pati ts and cand canon	$\frac{3}{2}$ $\frac{2}{0}$ $\frac{1}{1}$ $\frac{1}{0}$ $\frac{1}$	3 2 0 1 0 PE+ tria ed in modilient and ient and disseminet ereseard include l/or fam 40 child	1 1 0 <td< td=""><td>45 10 7 3 35 3 32 ents</td></td<>	45 10 7 3 35 3 32 ents

contributed to refinement of the research question, selection of outcome measures, and feedback on the intervention.⁵³

Table 2 describes the characteristics of PE+ RCTs (n=10) and PE- RCTs (n=35). Given the small sample size, formal statistical testing was not conducted. The descriptive data, however, suggest that PE+ RCTs were more likely to use a patient-reported outcome measure (PROM) as the primary outcome, have more than one primary outcome, have a larger sample size, and a lower percentage of loss to follow up, compared with PE- RCTs.

TRIAL CHARACTERISTICS	TOTAL	PE +	PE -
Number of trials	45	10	35
Age of study participants ² , median (IQR)	5.1 (1.8, 10.2)	5.1 (3.7, 7.9)	5.5 (1.5, 10.3)
Trial setting, n (%)			
Primary Care	9 (20.0)	1 (10.0)	8 (22.9)
Hospital	18 (40.0)	4 (40.0)	14 (40.0)
Community/Population	18 (40.0)	5 (50.0)	13 (37.1)
Trial type, n (%)			
Prevention	16 (35.6)	4 (40.0)	12 (34.3)
Treatment	29 (64.4)	6 (60.0)	23 (65.7)
Multicenter trial, yes, n (%)	39 (86.7)	10 (100.0)	29 (82.9)
Multinational trial, yes, n (%)	10 (22.2)	4 (40.0)	6 (17.1)
Multiple primary outcomes, yes, n (%)	5 (11.1)	3 (30.0)	2 (5.7)
Primary outcome:			
Biomedical, yes, n (%)	14 (31.1)	4 (40.0)	10 (28.6)
Clinical, yes, n (%)	29 (64.4)	6 (60.0)	23 (65.7)
PROM, yes, n (%)	7 (15.6)	3 (30.0)	4 (11.4)
Sample size, median (IQR)	433 (237, 1420)	671 (354, 1467)	366 (185, 1420)
Percentage lost to follow up, median (IQR)	9.6 (3.8, 13.3)	4.7 (2.8, 12.7)	10.5 (3.9, 16.5)

 Table 2. Characteristics of PE+ and PE- paediatric RCTs published in The BMJ, 2011-20201

¹ Values are shown as n (%) or median (inter quartile range, IQR).

² 13 trials were excluded from mean age analysis (2 PE+ and 11 PE-) as only median age or age range of participants was reported: Freedman 2011, Kumar 2011, Porto 2011, Green 2011, Gill 2011, Bhandari 2012, Little 2013, Stremler 2013, Dodd 2014, Andersson 2015, Hyttel-Sorensen 2015, Skoog Stahlgren 2019, and Blair 2019.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

BMJ Open

Research Quality - assessed using the modified Cochrane Risk of Bias Tool - was similar for PE+ and PE- RCTs (see Figure 2). Only 4/10 (40%) of PE+ trials, and 13/35 (37%) of PEtrials were rated as 'fair' or 'good' (p=1.00). Additional data on the quality scores for PE+ and PE- trials is shown in Supplementary Table 3.

Table 3. Measures of academic and non-	academic citation for PE+ and PE- p	aediatric RCTs published in The BMJ,
2011-2020		

Table 3 describes academic and	l non-academic me	easures of dissemir	nation for the PE+					
and PE- trials. With respect to academic measures of dissemination, citation frequency per year								
was similar for PE+ trials and PE- trials: Web of Science (median 6.6 versus 7.1, respectively)								
and Scopus (median 9.3 versus 9.5, respectively). Non-academic measures of dissemination,								
however, tended to be higher for PE+ tr	rials, compared wi	th PE- trials. For e	xample, the media	ın				
Altmetric Attention Score per year was	23.0 for PE+ trial	s compared with 5	.4 for PE- trials.					
Likewise, the median PlumX Social Me	edia score per year	r for PE+ trials was	s 46.6, compared v	vith				
a median score of 7.6 for PE- trials. Las	st, while median P	lumX Captures sco	ores per year were					
			1 1					
nigher for PE+ trials (41.5), compared v	with PE- trials (29	$\Pi \Pi P H + \Pi \eta R \eta \eta \eta$						
-	($(0), 1 \ge 1$ that had	a lower median					
PlumX Usage score per year (3.9), com	pared with PE- tri	als (41.9).	a lower median					
PlumX Usage score per year (3.9), com `able 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR)	pared with PE- trinic citation for PE+	als (41.9). and PE- paediatric F PE +	CTs published in <i>T</i>	<i>he BMJ</i> ,				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n	pared with PE- tri nic citation for PE+ Total 45	als (41.9). and PE- paediatric R PE + 10	CTs published in <i>T</i> PE - 35	he BMJ, p-value				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science)	pared with PE- tri nic citation for PE+ Total 45 6.9 (4.5, 13.1)	als (41.9). and PE- paediatric F PE + 10 6.6 (6.0, 8.6)	PE - 35 7.1 (4.2, 14.0)	<i>he BMJ</i> , p-value 0.84				
PlumX Usage score per year (3.9), com Fable 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science) Citation frequency per year (Scopus)	Total 45 6.9 (4.5, 13.1) 9.5 (6.1, 15.5)	als (41.9). and PE- paediatric R PE + 10 6.6 (6.0, 8.6) 9.3 (8.0, 15.3)	PE - 35 7.1 (4.2, 14.0) 9.5 (5.0, 17.5)	<i>he BMJ</i> , p-value 0.84 0.77				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science) Citation frequency per year (Scopus) Altmetric Attention Score per year	Total 45 6.9 (4.5, 13.1) 9.5 (6.1, 15.5) 7.3 (1.2, 21.8)	als (41.9). and PE- paediatric F PE + 10 6.6 (6.0, 8.6) 9.3 (8.0, 15.3) 23.0 (3.9, 40.0)	PE - 35 7.1 (4.2, 14.0) 9.5 (5.0, 17.5) 5.4 (1.0, 17.8)	<i>he BMJ</i> , p-value 0.84 0.77 0.13				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science) Citation frequency per year (Scopus) Altmetric Attention Score per year PlumX Citations per year	Total 45 6.9 (4.5, 13.1) 9.5 (6.1, 15.5) 7.3 (1.2, 21.8) 9.8 (6.6, 18.3)	als (41.9). and PE- paediatric R PE + 10 6.6 (6.0, 8.6) 9.3 (8.0, 15.3) 23.0 (3.9, 40.0) 9.5 (8.9, 16.8)	PE - 35 7.1 (4.2, 14.0) 9.5 (5.0, 17.5) 5.4 (1.0, 17.8) 10.2 (5.5, 19.5)	<i>he BMJ</i> , p-value 0.84 0.77 0.13 0.88				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science) Citation frequency per year (Scopus) Altmetric Attention Score per year PlumX Citations per year PlumX Usage per year	Total 45 6.9 (4.5, 13.1) 9.5 (6.1, 15.5) 7.3 (1.2, 21.8) 9.8 (6.6, 18.3) 37.7 (10.8, 75.8)	als (41.9). and PE- paediatric F PE + 10 6.6 (6.0, 8.6) 9.3 (8.0, 15.3) 23.0 (3.9, 40.0) 9.5 (8.9, 16.8) 3.9 (0.0, 69.8)	PE - 35 7.1 (4.2, 14.0) 9.5 (5.0, 17.5) 5.4 (1.0, 17.8) 10.2 (5.5, 19.5) 41.9 (23.1, 78.7)	<i>he BMJ</i> , <i>p</i> -value 0.84 0.77 0.13 0.88 0.04				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science) Citation frequency per year (Scopus) Altmetric Attention Score per year PlumX Citations per year PlumX Usage per year PlumX Captures per year	Total 45 6.9 (4.5, 13.1) 9.5 (6.1, 15.5) 7.3 (1.2, 21.8) 9.8 (6.6, 18.3) 37.7 (10.8, 75.8) 30.9 (22.0, 44.3)	als (41.9). and PE- paediatric R PE + 10 6.6 (6.0, 8.6) 9.3 (8.0, 15.3) 23.0 (3.9, 40.0) 9.5 (8.9, 16.8) 3.9 (0.0, 69.8) 41.5 (27.0, 80.8)	PE - 35 7.1 (4.2, 14.0) 9.5 (5.0, 17.5) 5.4 (1.0, 17.8) 10.2 (5.5, 19.5) 41.9 (23.1, 78.7) 29.0 (17.2, 40.9)	<i>he BMJ</i> , p-value 0.84 0.77 0.13 0.88 0.04 0.04				
PlumX Usage score per year (3.9), com Table 3. Measures of academic and non-academ 011-2020 Measures of citation (median, IQR) n Citation frequency per year (Web of Science) Citation frequency per year (Scopus) Altmetric Attention Score per year PlumX Citations per year PlumX Usage per year PlumX Captures per year PlumX Mentions per year	Total 45 6.9 (4.5, 13.1) 9.5 (6.1, 15.5) 7.3 (1.2, 21.8) 9.8 (6.6, 18.3) 37.7 (10.8, 75.8) 30.9 (22.0, 44.3) 0.2 (0.1, 0.7)	als (41.9). and PE- paediatric F PE + 10 6.6 (6.0, 8.6) 9.3 (8.0, 15.3) 23.0 (3.9, 40.0) 9.5 (8.9, 16.8) 3.9 (0.0, 69.8) 41.5 (27.0, 80.8) 0.3 (0.2, 0.9)	PE - 35 7.1 (4.2, 14.0) 9.5 (5.0, 17.5) 5.4 (1.0, 17.8) 10.2 (5.5, 19.5) 41.9 (23.1, 78.7) 29.0 (17.2, 40.9) 0.2 (0.1, 0.7)	<i>he BMJ</i> , <i>p</i> -value 0.84 0.77 0.13 0.88 0.04 0.04 0.37				

¹ Citation measures for PE+ and PE- groups were compared using the Mann-Whitney test for continuous variables.

BMJ Open

DISCUSSION

This systematic review collected and compared research quality and dissemination metrics for paediatric RCTs that engaged patients and families in the research process with trials that did not. Over a ten-year period, 10 of 45 RCTs published in *The BMJ* reported engaging patients and families in the research process. Descriptive data suggested that PE+ trials were more likely to use a PROM as the primary outcome, have larger sample sizes, and fewer participants lost to follow up. Research quality and academic dissemination metrics were similar for PE+ and PE- trials. PE+ trials, however, had higher social media scores compared with PEtrials, suggesting that patient and family engagement in a paediatric RCT may increase the likelihood of dissemination of trial research findings to the public via social media networks.

In total, 22% of paediatric trials in this review reported patient and family engagement. This compares favourably with data on patient engagement in adult RCTs. For example, a systematic review by Benizri *et al*⁶¹ that examined 50 RCTs published in 2021 in three leading medical journals (not including *The BMJ*) noted that only 5% of the RCTs reported patient engagement. A previous systematic review of RCTs published between 2011 and 2016, suggested that less than 1% of trials reported any patient engagement.⁶²

Paediatric care is child and family-centered, therefore, patient and family engagement in the research process may be more likely in this clinical milieu. Second, the increasing frequency of patient and family engagement may be related to the fact that several national research funding agencies mandate patient engagement in research proposal submissions. Last, an important factor, was the introduction of a new policy in 2015 by *The BMJ* that required authors to provide a *Patient and Public Involvement* paragraph in the Methods section of submitted manuscripts.⁶³ This requirement provides authors with a mandate and corollary word count to

report patient and family engagement in their research. In addition, the policy may encourage researchers to engage patients and families in their research, if *The BMJ* is the target journal. It is unsurprising that PE+ paediatric trials in the review were more likely to use a PROM as the primary outcome, compared with PE- trials. In addition, the data suggested that PE+ trials had larger sample sizes and lower loss to follow up, compared with PE- trials. Crocker et al⁶⁴ have shown that patient and public involvement in clinical trials significantly improves participant enrolment and may improve retention. Patient and public involvement in health research has long been hypothesized to improve

research quality and dissemination of findings, however, there are few empirical data on the topic.^{4,65,66} Barriers to precise measurement of engagement impact include inconsistent terminology for engagement, unpredictable reporting of engagement in the published literature, and the difficulty in accurately measuring the direct impact of engagement, given the role of other factors such as context, policy, and culture. This systematic review showed no material difference in the research quality of PE+ and PE- paediatric RCTs. Measures of academic dissemination - based on citation frequency per year - also showed no difference between PE+ and PE- trials. Non-academic dissemination measures, however, were generally higher among PE+ paediatric trials, compared with PE-trials. PlumX Usage scores were higher for PE- RCTs compared with PE+ RCTs; however, Usage scores (URL clicks, downloads, views) may reflect academic dissemination. PlumX Social Media scores (shares, likes, comments) more likely represent non-academic dissemination. To our knowledge, this is the first empirical study to examine the impact of patient and family engagement in paediatric RCTs on research quality and dissemination.

Strengths and Limitations

BMJ Open

Strengths of this study included a comprehensive and rigorous search strategy and eligibility review process. In addition, published trial protocols and clinical trial registries such as ClinicalTrials.gov were also searched for information on engagement for eligible trials.

There were limitations to our study. First, only one database (MEDLINE) and one journal (*The BMJ*) were searched. *The BMJ* was selected deliberately, given the requirement of submitting authors to report patient and public involvement in their research. In addition, the a priori sampling strategy was to examine high quality research and The BMJ is a high impact journal. Second, there may have been misclassification of trials. Categorization of RCTs as PE+ or PE- was based on information reported in the published article (and/or study protocol where available). This issue is particularly relevant for those trials published prior to *The BMJ* policy in 2015 that mandated reporting of patient engagement. Other authors have noted that patient and family engagement is under reported in the published literature.^{67,68} Given the *a priori* hypothesis, and the finding of no difference in research quality between PE+ and PE- trials in our review, any bias because of misclassification, i.e., PE+ trials misclassified as PE- would likely have been towards the null. Likewise, the academic citation frequency for any publication accumulates as the length of time from publication increases. Proportionately more PE- trials were published at the beginning of the study period, compared with PE+ trials. Therefore, given that academic citation frequencies per year were similar between PE+ and PE- trials, any bias would again have likely been towards the null.

CONCLUSION

Despite widespread recommendation for patient and family engagement in child health research, this systematic review showed that only one in five paediatric RCTs published in *The*

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ over a ten-year period reported patient and family engagement. Research quality was similar for trials engaging patients and families compared with trials that did not. Patient and family engagement, however, was associated with higher non-academic dissemination metrics, in particular, social media attention, compared to trials with no patient and family engagement.

Acknowledgements: We are thankful to the *The BMJ* authors and their collaborators of the papers included in this review who made this research possible.

Author Contributions

Dr. Borkhoff conceptualized and designed the study, designed the data collection instruments, coordinated and supervised the data extraction, performed the statistical analysis, interpreted the data, drafted the manuscript, critically reviewed and revised the manuscript.

Dr. Macarthur conceptualized and designed the study, designed the data collection instruments, interpreted the data, drafted the manuscript, critically reviewed and revised the manuscript.

Ms. Hattangadi performed the data extraction, performed the statistical analysis, interpreted the data, critically reviewed the manuscript for important intellectual content.

Ms. Nurse, Ms. Kay and Mr. Bhalla performed the data extraction, interpreted the data, critically reviewed the manuscript for important intellectual content.

Ms. Mahood developed the search strategy, performed data curation, interpreted the data, critically reviewed the manuscript for important intellectual content.

Drs. Buchanan, Taljaard, Cohen, Parkin interpreted the data, critically reviewed the manuscript for important intellectual content.

All of the authors approved the final manuscript as submitted and agreed to be accountable for all aspects of the work.

Funding: This research received no specific grant from any funding agency in the public, commercial or not-for-profit sectors.

Competing interests: None declared.

Patient consent: Not required.

Additional information: Data extracted from included studies and data used for all analyses are available on request. This review was not registered, and a review protocol was not prepared.

REFERENCES

- Canada's Strategy for Patient Oriented Research 2011. https://cihrirsc.gc.ca/e/44000.html#a1.1. Accessed February 9, 2024.
- Bate J, Ranasinghe N, Ling R, *et al.* Public and patient involvement in paediatric research. *Arch Dis Child Educ Pract* 2016;101(3):158–61.
- 3. Amirav I, Vandall-Walker V, Rasiah J, *et al.* Patient and researcher engagement in health research: a parent's perspective. *Pediatrics* 2017;140:e20164127.
- 4. Aubin D, Hebert M, Eurich D. The importance of measuring the impact of patient-oriented research. *CMAJ* 2019;191:e860-864.
- 5. Vanderhout S, Bhalla M, Van A, *et al.* The impact of patient and family engagement in child health research: a scoping review. *J Pediatr* 2023;253:115-128.
- Flynn R, Walton S, Scott SD. Engaging children and families in pediatric health research. *Research Involvement & Engagement* 2019 5, 32. https://doi.org/10.1186/s40900-019-0168-9.
- 7. Bailey S, Boddy K, Briscoe S, *et al.* Involving disabled children and young people as partners in research: a systematic review. *Child Care Health Dev* 2014;41:505-514.
- Shen S, Doyle-Thomas KAR, Beesley L, *et al.* How and why should we engage patients as co-researchers in health research? A scoping review of current practices. *Health Expectations* 2016;20:543-54.
- Bird M, Ouellette C, Whitmore C, *et al.* Preparing for patient partnership: a scoping review of patient partner engagement and evaluation in research. *Health Expectations* 2020;23:523-539.

BMJ Open

et al. Children and young people's contributions
ivities in health-related research: A scoping
https://doi.org/10.1371/journal.pone.0252774
SMA Group. Preferred reporting items for
PRISMA statement. Int J Surg 2010;8(5):336-
echnical Supplement to Chapter 4: Searching for
omas J, Chandler J, Cumpston M, Li T, Page MJ,
for Systematic Reviews of Interventions Version
024. Available from
al. Cochrane Bias Methods Group, Cochrane
Collaboration's tool for assessing risk of bias in
oi:10.1136/bmj.d5928
rage of Web of Science and Scopus: A
;106:213–228.
netrics and citation counts: an empirical analysis
ngs of the Joint Conference on Digital Libraries
Rapid versus standard intravenous rehydration
ded randomised clinical trial. BMJ
19
n.bmj.com/site/about/guidelines.xhtml

 Andersson O, Hellström-Westas L, Andersson D, *et al.* Effect of delayed versus early umbilical cord clamping on neonatal outcomes and iron status at 4 months: a randomized controlled trial. *BMJ* 2011;343:d7157.

- Puder JJ, Marques-Vidal P, Schindler C, *et al.* Effect of multidimensional lifestyle intervention on fitness and adiposity in predominantly migrant preschool children (Ballabeina): cluster randomised controlled trial. *BMJ* 2011;343:d6195.
- van den Aardweg MTA, Boonacker CWB, Rovers MM, *et al.* Effectiveness of adenoidectomy in children with recurrent upper respiratory tract infections: open randomised controlled trial. *BMJ* 2011;343:d5154.
- Wake M, Tobin S, Girolametto L, *et al.* Outcomes of population based language promotion for slow to talk toddlers at ages 2 and 3 years: Let's Learn Language cluster randomised controlled trial. *BMJ* 2011;343:d4741.
- Kumar GT, Sachdev HS, Chellani H, *et al.* Effect of weekly vitamin D supplements on mortality, morbidity, and growth of low birthweight term infants in India up to age 6 months: randomised controlled trial. *BMJ* 2011;342:d2975.
- Gault EJ, Perry RJ, Cole TJ, *et al.* Effect of oxandrolone and timing of pubertal induction on final height in Turner's syndrome: randomised, double blind, placebo controlled trial. *BMJ* 2011;342:d1980.
- Porto AMF, Coutinho IC, Correia JB, *et al.* Effectiveness of antenatal corticosteroids in reducing respiratory disorders in late preterm infants: randomised clinical trial. *BMJ* 2011;342:d1696.
- 24. Green JM, Wood AJ, Kerfoot MJ, *et al.* Group therapy for adolescents with repeated self harm: randomised controlled trial with economic evaluation. *BMJ* 2011;342:d682.

BMJ Open

25.	Sandison TG, Homsy J, Arinaitwe E, et al. Protective efficacy of co-trimoxazole
	prophylaxis against malaria in HIV exposed children in rural Uganda: a randomised
	clinical trial. <i>BMJ</i> 2011;342:d1617.
26.	Gill CJ, Phiri-Mazala G, Guerina NG, et al. Effect of training traditional birth attendants on
	neonatal mortality (Lufwanyama Neonatal Survival Project): randomised controlled study.
	<i>BMJ</i> 2011;342:d346.
27.	Gringras P, Gamble C, Jones A P, et al. Melatonin for sleep problems in children with
	neurodevelopmental disorders: randomised double masked placebo controlled trial. BMJ
	2012;345:e6664.
28.	Stallard P, Sayal K, Phillips R, et al. Classroom based cognitive behavioural therapy in
	reducing symptoms of depression in high risk adolescents: pragmatic cluster randomised
	controlled trial. <i>BMJ</i> 2012;345:e6058.
29.	Miller G, Luo R, Zhang L, et al. Effectiveness of provider incentives for anaemia reduction
	in rural China: a cluster randomised trial. BMJ 2012;345:e4809.
30.	Wen LM, Baur LA, Simpson JM, et al. Effectiveness of home based early intervention on
	children's BMI at age 2: randomised controlled trial. BMJ 2012;344:e3732.
31.	Waldén M, Atroshi I, Magnusson H, et al. Prevention of acute knee injuries in adolescent
	female football players: cluster randomised controlled trial. BMJ 2012;344:e3042.
32.	Robling M, McNamara R, Bennert K, et al. The effect of the Talking Diabetes consulting
	skills intervention on glycaemic control and quality of life in children with type 1 diabetes:
	cluster randomised controlled trial (DEPICTED study). BMJ 2012;344:e2359.
	21
	21

33.	Merry SN, Stasiak K, Shepherd M, et al. The effectiveness of SPARX, a computerised s	elf-
	help intervention for adolescents seeking help for depression: randomised controlled non	1-
	inferiority trial. BMJ 2012;344:e2598.	
34.	Bhandari N, Mazumder S, Taneja S, et al. Effect of implementation of Integrated	
	Management of Neonatal and Childhood Illness (IMNCI) programme on neonatal and	
	infant mortality: cluster randomised controlled trial. BMJ 2012;344:e1634.	
35.	Day C, Michelson D, Thomson S, et al. Evaluation of a peer led parenting intervention f	or
	disruptive behaviour problems in children: community based randomised controlled trial	Ι.
	<i>BMJ</i> 2012;344:e1107.	
36.	Palmer DJ, Sullivan T, Gold MS, et al. Effect of n-3 long chain polyunsaturated fatty acid	id
	supplementation in pregnancy on infants' allergies in first year of life: randomised	
	controlled trial. BMJ 2012;344:e184.	
37.	Wake M, Lycett K, Clifford SA, et al. Shared care obesity management in 3-10 year old	
	children: 12 month outcomes of HopSCOTCH randomised trial. BMJ 2013;346:f3092.	
38.	Little P, Moore M, Kelly J, et al. Ibuprofen, paracetamol, and steam for patients with	
	respiratory tract infections in primary care: pragmatic randomised factorial trial. BMJ	
	2013;347:f6041.	
39.	South East Asia Infectious Disease Clinical Research Network. Effect of double dose	
	oseltamivir on clinical and virological outcomes in children and adults admitted to hospi	tal
	with severe influenza: double blind randomised controlled trial. BMJ 2013;346:f3039.	
40.	Stremler R, Hodnett E, Kenton L, et al. Effect of behavioural-educational intervention of	n
	sleep for primiparous women and their infants in early postpartum: multisite randomised	l
	controlled trial. BMJ 2013;346:f1164.	
		22

BMJ Open

41.	Kipping RR, Howe LD, Jago R, et al. Effect of intervention aimed at increasing physical
	activity, reducing sedentary behaviour, and increasing fruit and vegetable consumption in
	children: Active for Life Year 5 (AFLY5) school based cluster randomised controlled trial.
	<i>BMJ</i> 2014;348:g3256.
42.	Attanasio OP, Bentham J, Fernández C, et al. Using the infrastructure of a conditional cash
	transfer program to deliver a scalable integrated early child development program in
	Colombia: cluster randomized controlled trial. BMJ 2014;349:g5785.
43.	Ma X, Zhou Z, Yi H, et al. Effect of providing free glasses on children's educational
	outcomes in China: cluster randomized controlled trial. BMJ 2014;349:g5740.
44.	van Wijk RM, van Vlimmeren LA, Groothuis-Oudshoorn CGM, et al. Helmet therapy in
	infants with positional skull deformation: randomised controlled trial. BMJ
	2014;348:g2741.
45.	Sung V, Hiscock H, Tang MLK, et al. Treating infant colic with the probiotic Lactobacillus
	reuteri: double blind, placebo controlled randomised trial. BMJ 2014;348:g2107.
46.	Dodd JM, Turnbull D, McPhee AJ, et al. Antenatal lifestyle advice for women who are
	overweight or obese: LIMIT randomised trial. BMJ 2014;348:g1285.
47.	Le Noury J, Nardo JM, Healy D, et al. Restoring Study 329: efficacy and harms of
	paroxetine and imipramine in treatment of major depression in adolescence. BMJ
	2015;351:h4320.
48.	Andersson N, Nava-Aguilera E, Arosteguí J, et al. Evidence based community mobilization
	for dengue prevention in Nicaragua and Mexico (Camino Verde, the Green Way): cluster
	randomized controlled trial. BMJ 2015;351:h3267.

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open

Pace D, Khatami A, McKenna J, et al. Immunogenicity of reduced dose priming schedules of serogroup C meningococcal conjugate vaccine followed by booster at 12 months in infants: open label randomised controlled trial. BMJ 2015;350:h1554. 50. He FJ, Wu Y, Feng X, et al. School based education programme to reduce salt intake in children and their families (School-EduSalt): cluster randomised controlled trial. BMJ 2015;350:h770. Hiscock H, Sciberras E, Mensah F, et al. Impact of a behavioural sleep intervention on 51. symptoms and sleep in children with attention deficit hyperactivity disorder, and parental mental health: randomised controlled trial. BMJ 2015:350:h68. Hyttel-Sorensen S, Pellicer A, Alderliesten T, et al. Cerebral near infrared spectroscopy 52. oximetry in extremely preterm infants: phase II randomised clinical trial. BMJ 2015;350:g7635. Kaufman J, Fitzpatrick P, Tosif S, et al. Faster clean catch urine collection (Quick-Wee 53. method) from infants: randomised controlled trial. BMJ 2017;357:j1341. Vinding RK, Stokholm J, Sevelsted A, et al. Effect of fish oil supplementation in 54. pregnancy on bone, lean, and fat mass at six years: randomised clinical trial. BMJ 2018;362:k3312. 55. Santer M, Ridd MJ, Francis NA, et al. Emollient bath additives for the treatment of childhood eczema (BATHE): multicentre pragmatic parallel group randomised controlled trial of clinical and cost effectiveness. BMJ 2018;361:k1332. Adab P, Pallan MJ, Lancashire ER, et al. Effectiveness of a childhood obesity prevention 56. programme delivered through schools, targeting 6 and 7 year olds: cluster randomised controlled trial (WAVES study). BMJ 2018;360:k211.

BMJ Open

57.	Skoog Ståhlgren G, Tyrstrup M, Edlund C, et al. Penicillin V four times daily for five days
	versus three times daily for 10 days in patients with pharyngotonsillitis caused by group A
	streptococci: randomised controlled, open label, non-inferiority study. BMJ 2019;
	367:15337.
58.	Webb NJA, Woolley RL, Lambe T, et al. Long term tapering versus standard prednisolone
	treatment for first episode of childhood nephrotic syndrome: phase III randomised
	controlled trial and economic evaluation. BMJ 2019;365:11800.
59.	Blair JC, McKay A, Ridyard C, et al. for the SCIPI investigators. Continuous subcutaneous
	insulin infusion versus multiple daily injection regimens in children and young people at
	diagnosis of type 1 diabetes: pragmatic randomised controlled trial and economic
	evaluation. <i>BMJ</i> 2019;365:11226.
60.	Roberts SB, Franceschini MA, Silver RE, et al. Effects of food supplementation on
	cognitive function, cerebral blood flow, and nutritional status in young children at risk of
	undernutrition: randomized controlled trial. BMJ 2020;370:m2397.
61.	Benizri N, Hallot S, Burns K, et al. Patient and Family Representation in Randomized
	Clinical Trials Published in 3 Medical and Surgical Journals: A Systematic Review. JAMA
	Network Open 2022;5(9):e2230858.
62.	Fergusson D, Monfaredi Z, Pussegoda K, et al. The prevalence of patient engagement in
	published trials: a systematic review. Research Involvement & Engagement 2018;4:17.
63.	Richards T, Schroter S, Price A, et al. Better together: patient partnership in medical
	journals. BMJ 2018;362:k3798 doi: 10.1136/bmj.k3798.

 Crocker J, Ricci-Cabello I, Parker A, <i>et al.</i> Impact of patient and public involvement on enrolment and retention in clinical trials: systematic review and meta-analysis. <i>BMJ</i> 2018;363:k4738. Petit-Zeman S, Locock L. Bring on the evidence. <i>Nature</i> 2013;501:160-61. Snape D, Kirkham J, Britten N, <i>et al.</i> Exploring perceived barriers, drivers, impacts and the need for evaluation of public involvement in health and social care research: a modified Delphi study. <i>BMJ Open</i> 2014;4:e004943. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		
 enrolment and retention in clinical trials: systematic review and meta-analysis. <i>BMJ</i> 2018;363:k4738. 65. Petit-Zeman S, Locock L. Bring on the evidence. <i>Nature</i> 2013;501:160-61. 66. Snape D, Kirkham J, Britten N, <i>et al.</i> Exploring perceived barriers, drivers, impacts and the need for evaluation of public involvement in health and social care research: a modified Delphi study. <i>BMJ Open</i> 2014;4:e004943. 67. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 	64.	Crocker J, Ricci-Cabello I, Parker A, et al. Impact of patient and public involvement on
 2018;363:k4738. 65. Petit-Zeman S, Locock L. Bring on the evidence. <i>Nature</i> 2013;501:160-61. 66. Snape D, Kirkham J, Britten N, <i>et al.</i> Exploring perceived barriers, drivers, impacts and the need for evaluation of public involvement in health and social care research: a modified Delphi study. <i>BMJ Open</i> 2014;4:e004943. 67. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		enrolment and retention in clinical trials: systematic review and meta-analysis. BMJ
 Petit-Zeman S, Locock L. Bring on the evidence. <i>Nature</i> 2013;501:160-61. Snape D, Kirkham J, Britten N, <i>et al.</i> Exploring perceived barriers, drivers, impacts and the need for evaluation of public involvement in health and social care research: a modified Delphi study. <i>BMJ Open</i> 2014;4:e004943. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		2018;363:k4738.
 Snape D, Kirkham J, Britten N, <i>et al.</i> Exploring perceived barriers, drivers, impacts and the need for evaluation of public involvement in health and social care research: a modified Delphi study. <i>BMJ Open</i> 2014;4:e004943. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 	65.	Petit-Zeman S, Locock L. Bring on the evidence. Nature 2013;501:160-61.
 need for evaluation of public involvement in health and social care research: a modified Delphi study. <i>BMJ Open</i> 2014;4:e004943. 67. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 	66.	Snape D, Kirkham J, Britten N, et al. Exploring perceived barriers, drivers, impacts and the
 Delphi study. <i>BMJ Open</i> 2014;4:e004943. 67. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		need for evaluation of public involvement in health and social care research: a modified
 67. Price A, Schroter S, Snow R, <i>et al.</i> Frequency of reporting on patient and public involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		Delphi study. BMJ Open 2014;4:e004943.
 involvement (PPI) in a general medical journal: a descriptive study. <i>BMJ Open</i> 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 	67.	Price A, Schroter S, Snow R, et al. Frequency of reporting on patient and public
 2018;8:e020452. doi:10.1136/bmjopen-2017-020452. 68. Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		involvement (PPI) in a general medical journal: a descriptive study. BMJ Open
 Vanderhout S, Nevins P, Nicholls S, <i>et al.</i> Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115. 		2018;8:e020452. doi:10.1136/bmjopen-2017-020452.
trials: online survey of corresponding authors of published trials. <i>CMAJ Open</i> 2023 Sep 19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115.	68.	Vanderhout S, Nevins P, Nicholls S, et al. Patient and public involvement in pragmatic
19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115.		trials: online survey of corresponding authors of published trials. CMAJ Open 2023 Sep
		19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115.

Figure Legend

Figure 1. Areas of patient and family engagement in PE+ paediatric trials published in *The BMJ*¹

Figure 2. Cochrane Risk of Bias Scores for PE+ and PE- trials

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.



¹Patients and families could be engaged in more than one aspect of the research process in a single study

For peer review only - http://bmjopen.bmj.com/site/about/guidelines.xhtml

BMJ Open

PE+RCTs



BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool Protected by copyright, instlightion for heres seleted fourth and idats/mithing, but the initian technologies.

2
2
2
4
5
6
7
/
8
9
10
11
11
12
13
14
1 5
15
16
17
18
10
19
20
21
22
22
23
24
25
26
20
27
28
29
20
30
31
32
33
24
34
35
36
37
20
38
39
40
11
42
42
43
44
45
45
46
47
48
⊿0
77
50
51
52
52
22
54
55
56
57
5/
58
59

1

Supplementary Table 1. Ovid MEDLINE search strategy to identify indexed pediatric randomize	d
controlled trials (RCTs) published in <i>The BMJ</i>	

Search Strategy	Description	Results
1. randomized controlled trial.pt.		574707
2. controlled clinical trial.pt.		94982
3. randomized.ab.		571502
4. placebo.ab.		230695
5. clinical trials as topic.sh.	Cochrane Highly Sensitive Search	200252
6. randomly.ab.	(Steps 1 to 10)	388759
7. trial.ti.		268047
8. 1 or 2 or 3 or 4 or 5 or 6 or 7		1465478
9. exp animals/not humans.sh.		5035319
10. 8 not 9		1348058
11. (BMJ or British Medical	Limit by BMJ	182994
Journal).jn		
12. limit 11 to yr="2015"	Limit by year*	3335
13. limit 12 to (address or comment or		1214
editorial or letter or observational study	Filter out addresses, comments, editorials, letters,	
or meta-analysis or review or	observational studies, meta-analyses, reviews, and	
14 12 not 13	systematic reviews	2121
15 10 and 14	All RCTs meeting above criteria	97
16 limit 15 to all child <0 to 18 years>	Limit to RCTs indexed as including children	22
17 limit 15 to all adult <19 plus years>	Limit to RCTs indexed as including adults	30
18 15 not (16 or 17)	RCTs not indexed by any age group	57
19 16 not 17	RCTs indexed as only including children	10
20. 17 not 16	RCTs indexed as only including adults	18
21. (16 or 17) not (19 or 20)	RCTs indexed as including both children and	12
	adults	

*Example shows the search query for the year 2015. This search strategy was repeated for each calendar year from 2011 to 2020, inclusive.



			BMJ Open	cted by co
1 2 3 4 5	Supplementary Tab	le 2. Paediatric RCTs reporting patient and family o	engagement published in 2	The BMJ (2011-2020); Areas of Engager
6	Country	1.00	Chine Curegory	in cus of Eligage dente jo 3
7 8 9 10	Puder, 2011 Switzerland	Effect of multidimensional lifestyle intervention on fitness and adiposity in predominantly migrant preschool children (Ballabeina): cluster randomised controlled trial.	Cardiology	• Intervention development/refinement
11 12 13 14 15 16	Robling, 2012 United Kingdom	The effect of the Talking Diabetes consulting skills intervention on glycaemic control and quality of life in children with type 1 diabetes: cluster randomised controlled trial (DEPICTED study).	Endocrinology	 Engagement of the participants Intervention development/refinement text an escilation
17 18 19 20 21	Kipping, 2014 England	Effect of intervention aimed at increasing physical activity, reducing sedentary behaviour, and increasing fruit and vegetable consumption in children: active for Life Year 5 (AFLY5) school- based cluster randomised controlled trial.	Public health or preventive medicine	 Intervention der geweinent/refinement Intervention der geweine intervention de
22 23 24 25 26 27	Andersson, 2015 Nicaragua and Mexico	Evidence based community mobilization for dengue prevention in Nicaragua and Mexico (Camino Verde, the Green Way): cluster randomized controlled trial.	Infectious diseases and vaccines	 Engagement of youth participants Intervention development/refinement Input on study lesson Intervention delivery Dissemination and implementation of results
28 29 30 31	Kaufman, 2017 Australia	Faster clean catch urine collection (Quick-Wee method) from infants: randomised controlled trial.	Urology	 Development of the research question Selection of our comes Intervention development/refinement Intervention development/refinement
32 33 34 35 36	Santer, 2018 England, Wales	Emollient bath additives for the treatment of childhood eczema (BATHE): multicentre pragmatic parallel group randomised controlled trial of clinical and cost effectiveness.	Dermatology	 Intervention development/refinement Input on study glestin Recruitment of participants Dissemination and implementation of results
37 38 39 40	Adab, 2018 United Kingdom	Effectiveness of a childhood obesity prevention programme delivered through schools, targeting 6- and 7-year-olds: cluster randomised controlled trial (WAVES study).	Public health or preventive medicine	 Intervention development/refinement Input on study design
41 42 43 44 45 46 47		For peer review only - http://	bmjopen.bmj.com/site/abou	μ/guidelines.xhtml

Page 35 of 35		BMJ Open	0.1136/bmjc cted by cop	
2 3 Webb, 2019 4 United Kingdom 5	Long term tapering versus standard prednisolone treatment for first episode of childhood nephrotic syndrome: phase III randomised controlled trial and economic evaluation.	Nephrology	 Input on study design Input on study conduct Results interpretation of results 	
 7 8 Blair, 2019 9 England, Wales 10 11 12 13 14 15 16 	Continuous subcutaneous insulin infusion versus multiple daily injection regimens in children and young people at diagnosis of type 1 diabetes: pragmatic randomised controlled trial and economic evaluation.	Endocrinology	 Engagement of youth participants Engagement of youth participants Selection of our comes Input on study design Recruitment of the cipants Intervention deligible With the cipants Input on study for the cipants Input on study for the cipants Input on study for the cipants Dissemination and make the cipants 	
 17 Roberts, 2020 18 Guinea-Bissau 19 20 	Effects of food supplementation on cognitive function, cerebral blood flow, and nutritional status in young children at risk of undernutrition: randomized controlled trial.	Neurology	 Intervention de grade ment/refinement Input on study al segn Intervention de ivery 	
21 22 23 24 25 26 27 28 29 30 31 32 33 34 35 36 37 38 39 40 41 42 43 44 45 46	For peer review only - http://	bmjopen.bmj.com/site/abour	, Al training, and similar technologies.	
	BMJ Open		0.1136/bmjop cted by copy	
--	-----------------------------------	---------------------------	---	------------------------------
Supplementary Table 3. Quality assessment of PE+ and PE-	paediatric RCTs in	<i>The BMJ</i> , 2011-202	en-2024- right, inc	
Criteria / Risk of bias	Total	PE +	P#d :- 086	<i>p</i> -value ²
n	45	10	135 934	
Q1. Random Sequence Generation – selection bias			or on	
Low risk of	bias 43 (95.6)	9 (90.0)	34 (@ 7. b	
Unc	lear 1 (2.2)	0 (0.0)	1 (3.9)Aar	0.40
High risk of	bias 1 (2.2)	1 (10.0)		
Q2. Allocation Concealment – selection bias			025 imu d to	
Low risk of	bias 37 (82.2)	9 (90.0)	28 (3)	
Unc	lear 4 (8.9)	0 (0.0)		0.80
High risk of	bias 4 (8.9)	1 (10.0)	3 (8.5)	
Q3. Selective Reporting – reporting bias			ata ded	
Low risk of	bias 12 (26.7)	2 (20.0)	10 (28.6)	
Unc	lear 31 (68.9)	7 (70.0)	24 🛃 8.6	0.54
High risk of	bias 2 (4.4)	1 (10.0)	1 (2.9)	
Q4. Other Bias – bias not covered elsewhere			trai	
Low risk of	bias 31 (68.9)	7 (70.0)	24 (58.6	
Unc	lear 5 (11.1)	1 (10.0)	4 (المالية 1.4	1.00
High risk of	bias 9 (20.0)	2 (20.0)	7 (20.02.	
Q5. Blinding of Participants and Personnel – performance b	pias		mi g	
Low risk of	bias 22 (48.9)	4 (40.0)	18 (5) 1.40	
Unc	lear 5 (11.1)	1 (10.0)	4 (<u><u><u></u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u><u></u></u>	0.87
High risk of	bias 18 (40.0)	5 (50.0) 📨	13 (d 7. h	
Q6. Blinding of Outcome Assessment – detection bias			7, 2 ogie	
Low risk of	bias 31 (68.9)	6 (60.0)	25 (71.4)	
Unc	lear 9 (20.0)	3 (30.0)	6 (17.1 °)	0.74
High risk of	bias 5 (11.1)	1 (10.0)	4 (11.4	
	, , , , , , , , , , , , , , , , ,		artn	
			nen	
			GE CE	
	I		Z-LTA	

		BMJ Open		1136/bmjope ed by copyr	
07. Incomplete Outcome Data – attrition b	ias			ight, i	
	Low risk of bias	31 (68.9)	6 (60.0)	25 (71.45	
	Unclear	13 (28.9)	4 (40.0)	9 (\$5.78	0.5
	High risk of bias	1 (2.2)	0 (0.0)	1 (2.9)	1
Overall Quality Rating				r us	
	GOOD or FAIR	17 (37.8)	4 (40.0)	13 (87.13)	1.0
	POOR	28 (62.2)	6 (60.0)	22 (🖧 🎢 🦣	- 1.0
				ning	

BMJ Open

Research quality and dissemination of paediatric randomized controlled trials with and without patient and family engagement: systematic review

Journal:	BMJ Open
Manuscript ID	bmjopen-2024-086934.R1
Article Type:	Original research
Date Submitted by the Author:	25-Jan-2025
Complete List of Authors:	Borkhoff, Cornelia; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health, Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Hattangadi, Nayantara ; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Nurse, Kimberly ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Kay, Tatjana; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Bhalla, Manav ; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Mahood, Quenby; The Hospital for Sick Children, Division of Hospital Library and Archives Buchanan, Francine; Hospital for Sick Children Research Institute, Child Health and Evaluative Sciences Taljaard, Monica; Ottawa Hospital Research Institute, Clinical Epidemiology Program; University of Ottawa, School of Epidemiology and Public Health Cohen, Eyal; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences; University of Toronto, Edwin S.H. Leong Centre for Healthy Children Parkin, Patricia; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) ; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Chil

2	
- २	
1	
4	
5	
6	
7	
8	
9	
10	
10	
11	
12	
13	
14	
15	
16	
17	
18	
10	
20	
20	
21	
22	
23	
24	
25	
26	
27	
28	
20	
29	
30	
31	
32	
33	
34	
35	
36	
37	
38	
39	
40	
/1	
40	
42	
43	
44	
45	
46	
47	
48	
49	
50	
50	
51	
52	
53	
54	
55	
56	

57 58 59

Primary Subject Heading :	Paediatrics
Secondary Subject Heading:	Research methods
Keywords:	PAEDIATRICS, Child, Social Media, Randomized Controlled Trial



Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, AI training, and similar technologies



I, the Submitting Author has the right to grant and does grant on behalf of all authors of the Work (as defined in the below author licence), an exclusive licence and/or a non-exclusive licence for contributions from authors who are: i) UK Crown employees; ii) where BMJ has agreed a CC-BY licence shall apply, and/or iii) in accordance with the terms applicable for US Federal Government officers or employees acting as part of their official duties; on a worldwide, perpetual, irrevocable, royalty-free basis to BMJ Publishing Group Ltd ("BMJ") its licensees and where the relevant Journal is co-owned by BMJ to the co-owners of the Journal, to publish the Work in this journal and any other BMJ products and to exploit all rights, as set out in our <u>licence</u>.

The Submitting Author accepts and understands that any supply made under these terms is made by BMJ to the Submitting Author unless you are acting as an employee on behalf of your employer or a postgraduate student of an affiliated institution which is paying any applicable article publishing charge ("APC") for Open Access articles. Where the Submitting Author wishes to make the Work available on an Open Access basis (and intends to pay the relevant APC), the terms of reuse of such Open Access shall be governed by a Creative Commons licence – details of these licences and which <u>Creative Commons</u> licence will apply to this Work are set out in our licence referred to above.

Other than as permitted in any relevant BMJ Author's Self Archiving Policies, I confirm this Work has not been accepted for publication elsewhere, is not being considered for publication elsewhere and does not duplicate material already published. I confirm all authors consent to publication of this Work and authorise the granting of this licence.

Resea	arch quality and dissemination of paediatric randomized controlled trials with and without patient and family engagement: systematic review
Corr Franc	nelia M. Borkhoff, MSc, PhD ^{1,2,3} Nayantara Hattangadi, MHSc ³ , Kimberly M. Nurse, MSc ^{2,4} Tatjana Kay, MSc ³ , Manav Bhalla, BSc ³ , Quenby Mahood, MI ⁴ , ine Buchanan, MLiS, PhD ³ , Monica Taljaard, MSc, PhD, ^{5,6} , Eyal Cohen, MD, MSc, ^{1,2,3,7,8} Patricia C. Parkin, MD ^{1,2,3,7} , Colin Macarthur, MBChB, PhD ^{1,2,3,7}
Affilia ¹ Divis Sick C ² Instit Univer ³ Chilo ⁴ Divis Canad ⁵ Clini Hospit ⁶ Scho ⁷ Depa Ontari ⁸ Edwi Canad	 httons: hin of Paediatric Medicine and Paediatric Outcomes Research Team (PORT), Hospital for Children, Toronto, Ontario, Canada; hute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health, rsity of Toronto, Toronto, Ontario, Canada; health Evaluative Sciences, SickKids Research Institute, Toronto, Ontario, Canada; hospital Library and Archives, Hospital for Sick Children, Toronto, Ontario, a; cal Epidemiology Program, Ottawa Hospital Research Institute (OHRI), The Ottawa al, General Campus, 501 Smyth Road, Ottawa, Ontario, Canada; ol of Epidemiology and Public Health, University of Ottawa, Ottawa, Ontario, Canada; rtment of Paediatrics, Temerty Faculty of Medicine, University of Toronto, Toronto, ON M5G 0A4, a
Addre Reseau Canad	ess correspondence to: Cornelia M. Borkhoff, PhD, The Hospital for Sick Children rch Institute, Peter Gilgan Centre for Research and Learning, 686 Bay St, Toronto, ON, a M5G 0A4. E-mail: cory.borkhoff@sickkids.ca
Short trials	title: Patient engagement, quality and dissemination of paediatric randomized controlled
Keyw quality	ords: patient engagement; paediatrics; child; randomized controlled trials; research y; dissemination; social media
	1

ABSTRACT

Objectives: Authentic patient and family engagement in child health research is defined as researchers working in partnership with patients and families on all aspects of the research process, including refining the research question, tailoring the intervention, devising study procedures, and disseminating study findings. While there is good evidence of a positive impact of patient engagement on the research process, on research teams, and on patient partners, there are few empirical data on the impact of patient and family engagement on research quality and dissemination. We conducted a systematic review to compare research quality and dissemination metrics for paediatric randomized controlled trials (RCTs) that engaged patients and families in the research process, with trials that did not.

Design: Systematic review using The Cochrane Highly Sensitive Search to identify RCTs. **Data sources:** Ovid MEDLINE from 1 January 2011 through to 31 December 2020.

Eligibility criteria: We included RCTs involving children and youth (<18 years of age) published in *The BMJ* (a peer-reviewed general medical journal).

Data extraction and synthesis: Trials were categorized as those engaging patients and families (PE+) and those that did not (PE-). A standardized review form was used to confirm trial eligibility and extract data on study characteristics. Two reviewers independently screened and sorted RCTs into PE+ and PE- groups, extracted data, and assessed research quality using the modified Cochrane Risk of Bias Tool (based on 7 methodologic criteria). Dissemination of RCT findings was determined using measures of academic and non-academic citation collected from Web of Science and Scopus.

Results: From 2011 to 2020, *The BMJ* published 45 RCTs involving children and youth. Only 10/45 RCTs (22%) reported engaging patients and families in the research process. Research

quality for PE+ and PE- paediatric RCTs was similar; 4/10 (40%) of PE+ trials, and 13/35 (37%) of PE- trials were rated as 'fair' or 'good' (p=1.00). Academic citation frequency per year was similar for PE+ trials and PE- trials: Web of Science (median 6.6 versus 7.1, respectively; p=0.84). Non-academic dissemination measures were generally higher among PE+ trials, for example, median PlumX Social Media score per year for PE+ trials was 46.6, compared with a median score of 7.6 for PE- trials (p=0.02).

Conclusions: Despite increasing interest in patient and family engagement in child health research, this review showed that few paediatric RCTs report patient engagement activity. Research quality was similar for trials engaging patients and families compared with those that did not. Patient and family engagement in the trial, however, was associated with higher metrics for social media attention, compared with trials with no engagement.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

All data relevant to the study are included in the article or uploaded as supplementary

information. Extracted data are available on request to the corresponding author.

BMJ Open

Strengths and limitations of this study

- We conducted a systematic review of paediatric randomized controlled trials (RCTs) published in *The BMJ* to compare research quality and dissemination metrics for trials that engaged patients and families in the research process with trials that did not.
- We assessed research quality using the modified Cochrane Risk of Bias Tool for RCTs (based on 7 methodological quality criteria) and dissemination metrics using measures of academic and non-academic citation collected from Web of Science and Scopus.
- We only sampled paediatric RCTs published in one journal, but *The BMJ* was selected deliberately, given the requirement of submitting authors to report patient and public involvement in their research.
- RCTs were categorized as those engaging patients and families (PE+) and those that did not (PE-) based on information reported in the published article (and/or study protocol where available). There may have been misclassification of trials, particularly those trials published prior to *The BMJ* policy in 2015 that mandated reporting of patient engagement.

INTRODUCTION

Patient-oriented research has been defined as a continuum of research that engages patients as partners, focuses on patient-identified priorities and outcomes, and integrates the knowledge generated into policy and practice to improve health care outcomes.¹ The term 'patient' is overarching and includes those with lived experience of a health issue, as well as informal caregivers, such as family and friends. Patient-oriented research is hypothesized to improve the quality, relevance, and uptake of health research.²

Authentic patient engagement in health research involves a partnership between patients and researchers working together on any or all aspects of the research process, including choosing the research question, selecting the study design, tailoring the intervention, devising study procedures, and dissemination of study findings. Barriers to patient engagement, however, such as parenting commitments, work schedules, and long research timelines, can make patient and family engagement in child health research challenging.³

Aubin *et al*⁴ proposed a framework for measuring *impact* related to patient-oriented research. The framework identifies potential impact metrics across four domains: improvements to research process, impact on policies, impact on health outcomes, and contribution to social change. In the context of child health research, there is qualitative evidence of a positive impact of patient and family engagement on the research process (refinement of the research question, intervention, materials), on research teams (enhanced knowledge, cultural competency), and on patient partners (empowerment, skills development).⁵⁻¹⁰ There are, however, few quantitative data on the impact of patient-oriented research, and to our knowledge, the impact of patient and family engagement on the search, and to our knowledge, the impact of patient and family engagement on research quality and dissemination has not been examined.

BMJ Open

Therefore, the objective of this study was to conduct a systematic review of paediatric RCTs published in *The BMJ* (a peer-reviewed general medical journal with a high impact factor), examine measures of research quality and dissemination (academic and non-academic), and compare RCTs that reported engaging patients and families in the research process with RCTs that did not.

METHODS

This study followed the Preferred Reporting Items for Systematic Reviews and Metaanalyses (PRISMA) reporting guidelines.¹¹ We did not register a protocol prior to conducting the review.

Search Strategy and Information Sources

An information science specialist (QM) developed an Ovid MEDLINE electronic search strategy to identify RCTs involving children and youth (<18 years of age) published in *The BMJ* over the 10-year period January 01, 2011, through to December 31, 2020. The search strategy was optimized for sensitivity and specificity using The Cochrane Highly Sensitive Search to identify RCTs.¹² A search end date of 2020 was chosen, given the widespread impact of the COVID-19 pandemic on health research. **Supplementary Table 1** describes the search strategy. **Inclusion and Exclusion Criteria**

RCTs published in *The BMJ* over the 10-year period that assessed a specific intervention were eligible. Only primary reports of trial results related to the trial primary outcome were included, i.e., publications reporting a secondary analysis of RCT data were excluded. Trials were included if the study population was limited to children and youth from birth to less than 18 years of age. Excluded were clinical trial study protocols; non-randomized comparative trials;

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

cross-sectional studies; non-comparative studies; systematic, scoping, and narrative reviews; conference abstracts; and editorials/commentaries.

Study Selection and Data Extraction

A standardized review form was developed to confirm trial eligibility and extract data on study characteristics. Two reviewers (two of NH, KMN, TK, MB) independently performed an eligibility assessment for each article using the inclusion and exclusion criteria, first screening titles and abstracts and then full texts of potentially relevant articles. Any discrepancies were resolved through discussion and adjudication with a third reviewer (CMB).

For this review, patient and family engagement in research (a continuum of research that engages patients as partners, focuses on patient-identified priorities, and aims to improve patient outcomes)¹ was considered the 'exposure of interest' and research quality and dissemination were the 'outcomes of interest'. Therefore, trials were categorized into two groups: the PE+ group (trials that reported engaging patients and families in the research process) and the PE- group (trials that <u>did not report</u> engaging patients and families in the research process). Recognizing that information on patient and family engagement might not necessarily be reported in *The BMJ* publication, we also reviewed information in published trial protocols (when available), and clinical trial registries such as ClinicalTrials.gov (when available) for all studies.

Two reviewers independently extracted data on RCT characteristics and methods, including author name; year of publication; trial setting; trial type; multicenter (yes/no); multinational (yes/no); participant age; primary outcome; sample size; number lost to follow-up; and patient and/or family engagement (yes/no). For the PE+ trials, additional data were

BMJ Open

Outcomes

'Research quality' was assessed using the modified Cochrane Risk of Bias Tool for RCTs.¹³ The Cochrane tool appraises 7 methodological quality criteria: 1) random sequence generation (selection bias); 2) allocation concealment (selection bias); 3) selective reporting (reporting bias); 4) other bias; 5) blinding of participants and researchers (performance bias); 6) blinding of outcome assessment (detection bias); and 7) incomplete outcome data (attrition bias). Two reviewers independently evaluated the research quality of eligible trials, and any discrepancies were resolved through discussion with a third reviewer. An overall quality rating for each trial based on a summation of the 7 criteria was also determined; trials were categorized as 'Good,' 'Fair,' or 'Poor,' based on the Risk of Bias Tool guide.

Dissemination of trial results was determined using measures of academic and nonacademic citation. Data on citation numbers and citation frequencies from Web of Science and Scopus were collected up to February 14, 2024. Web of Science has a narrower coverage of biomedical journals than Scopus and may therefore give a more conservative citation count.¹⁴ The primary academic dissemination metric captured was citation frequency per year (citation count divided by the number of years since publication).

PlumX data from Scopus (<u>https://plumanalytics.com/learn/about-metrics/</u>) and altmetric data from Web of Science (<u>https://www.altmetric.com/research-access/</u>) were used to determine non-academic citation.¹⁵ PlumX data capture interactions with a research output in the online environment across five domains: Citations (indexes, clinical or policy citations); Usage (URL clicks, downloads, views); Captures (bookmarks, favourites, follows); Mentions (news media,

blog posts, Wikipedia references); and Social Media (shares, likes, comments). The Altmetric Attention Score is a weighted count of the public attention a research article has received based on a variety of sources (citations, news media, social media mentions, blogs etc.). PlumX and Altmetric Attention Scores were described by year (total interactions divided by the number of years since publication of the trial).

Statistical Analysis

Descriptive statistics were used to describe the frequency of paediatric RCTs published in *The BMJ* (by calendar year and patient engagement) and other RCT characteristics. Medians and interquartile ranges (IQRs) were used to describe continuous variables that were non-normally distributed. PE+ and PE- trials were compared on research quality and dissemination metrics using Fisher's Exact test for categorical outcomes and the Mann-Whitney test for continuous variables. Statistical significance was defined as p<0.05; all statistical tests were 2-sided.

Ethics

Ethics approval for the study was not required as no data were collected from human subjects and only peer-reviewed published studies in the public domain were reviewed.

Patient and public involvement

This systematic review was conducted in partnership with a patient partner (FB, coauthor) who provided input on framing the research question, research methods, interpreting the research findings, writing the manuscript, and preparing dissemination plans.

RESULTS

The literature search strategy (see **Supplementary Table 1**) identified 29,944 citations in *The BMJ* up to 2020, with 818 (2.7%) classified as potential RCTs. Of these, 132 (16%) were

BMJ Open

indexed as including children, and were assessed for eligibility. After review, 87 records were excluded, leaving 45 paediatric trials included in the review (the search flowchart and reasons for exclusion are described in **Supplementary Figure 1**).

Over the period 2011 to 2020, *The BMJ* published 45 RCTs involving children and youth.¹⁶⁻⁶⁰ As shown in **Table 1**, only 10 of the 45 RCTs (22%) reported engaging patients and/or families in the trial. (Additional information on the 10 PE+ trials is provided in **Supplementary Table 2**). In alignment with the 2015 mandate from *The BMJ* in *Instructions to Authors*, all seven of the PE+ trials published from 2015 onwards included a *Patient and Public Involvement* (PPI) paragraph in the Methods section of the manuscript. Of the PE- paediatric trials, seven were published in 2015 or later, however, only three included a PPI paragraph; the four trials without a PPI paragraph were all published in 2015. Full implementation of the new reporting policy may have been slower than anticipated.

BMJ	Published RCTs	Patient engagement		No patient	engagement
		PPI paragraph	PPI paragraph	PPI paragraph	PPI paragraph
		yes	no	yes	no
2011	11	0	1	0	10
2012	10	0	1	0	9
2013	4	0	0	0	4
2014	6	0	1	0	5
2015	6	1	0	1	4
2016	0	0	0	0	0
2017	1	1	0	0	0
2018	3	2	0	1	0
2019	3	2	0	1	0
2020	1	1	0	0	0
2011-2020	45	7	3	3	32

Table 1. Number of paediatric RCTs published in *The BMJ* by patient engagement status and year of publication

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

As described in **Figure 1** and **Supplementary Table 2**, among the 10 PE+ trials, studies described a spectrum of engagement, including engagement in the research preparation phase (9 [90%]), execution phase (8 [80%]), and translation phase (5 [50%]), and often across more than one aspect of the study. For example, 8 PE+ trials had patient and/or family input on the development of the intervention, ^{18,32,41,48,53,55,56,60} and 5 PE+ trials had input on the dissemination of trial results.^{41,48,55,58,59} Of the 10 PE+ RCTs, three trials that included study participants 12 to 17 years of age, specifically engaged youth partners;^{32,48,59} the remaining seven trials engaged only parents or caregivers. Only one PE+ trial engaged families in developing the research question and only this trial provided information on the numbers engaged; parents and caregivers of 40 children contributed to refinement of the research question, selection of outcome measures, and feedback on the intervention.⁵³

Table 2 describes the characteristics of PE+ RCTs (n=10) and PE- RCTs (n=35). Across the 45 trials, the most common clinical categories were mental health (7 [16%]), endocrinology (4 [9%]), neonatology (4 [9%]), public health/preventative medicine (3 [7%]), infectious diseases and vaccines (3 [7%]), neurology (3 [7%]), and respirology (3 [7%]). Given the small sample size, formal statistical testing was not conducted. The descriptive data, however, suggest that PE+ RCTs were somewhat more likely to use a patient-reported outcome measure (PROM) as the primary outcome. Of the 7 trials using a PROM, three trials used a parent proxy measure (2 PE+ and 1 PE-) as participating children were as young as 1 to 3 years of age. Likewise, based on descriptive data, PE+ RCTs were also more likely to have more than one primary outcome, a larger sample size, and a lower percentage of loss to follow up, compared with PE- RCTs.

TRIAL CHARACTERISTICS	TOTAL	PE +	PE -
Number of trials	45	10	35
Age of study participants ² , median (IQR)	5.1 (1.8, 10.2)	5.1 (3.7, 7.9)	5.5 (1.5, 10.3)
Trial setting, n (%)			
Primary Care	9 (20.0)	1 (10.0)	8 (22.9)
Hospital	18 (40.0)	4 (40.0)	14 (40.0)
Community/Population	18 (40.0)	5 (50.0)	13 (37.1)
Trial type, n (%)			
Prevention	16 (35.6)	4 (40.0)	12 (34.3)
Treatment	29 (64.4)	6 (60.0)	23 (65.7)
Multicenter trial, yes, n (%)	39 (86.7)	10 (100.0)	29 (82.9)
Multinational trial, yes, n (%)	10 (22.2)	4 (40.0)	6 (17.1)
Multiple primary outcomes, yes, n (%)	5 (11.1)	3 (30.0)	2 (5.7)
Primary outcome:			
Biomedical, yes, n (%)	14 (31.1)	4 (40.0)	10 (28.6)
Clinical, yes, n (%)	29 (64.4)	6 (60.0)	23 (65.7)
PROM, yes, n (%)	7 (15.6)	3 (30.0)	4 (11.4)
Sample size, median (IQR)	433 (237, 1420)	671 (354,	366 (185,
		1467)	1420)
Percentage lost to follow up, median (IQR)	9.6 (3.8, 13.3)	4.7 (2.8, 12.7)	10.5 (3.9,
			16.5)

Table 2. Characteristics of PE	+ and PE- naediatr	ic RCTs published ir	The BML 2011-20201
Table 2. Characteristics of TE	⁻ and 1 L ⁻ paculati	ic ite i s published il	1 <i>I ne Divis</i> , 2011-2020

¹Values are shown as n (%) or median (inter quartile range, IQR).

² 13 trials were excluded from mean age analysis (2 PE+ and 11 PE-) as only median age or age range of participants was reported: Freedman 2011, Kumar 2011, Porto 2011, Green 2011, Gill 2011, Bhandari 2012, Little 2013, Stremler 2013, Dodd 2014, Andersson 2015, Hyttel-Sorensen 2015, Skoog Stahlgren 2019, and Blair 2019.

Research Quality - assessed using the modified Cochrane Risk of Bias Tool - was similar for PE+ and PE- RCTs (see **Figure 2**). Only 4/10 (40%) of PE+ trials, and 13/35 (37%) of PEtrials were rated as 'fair' or 'good' (p=1.00). Additional data on the quality scores for PE+ and PE- trials is shown in **Supplementary Table 3**.

Table 3 describes academic and non-academic measures of dissemination for the PE+

and PE- trials. With respect to academic measures of dissemination, citation frequency per year

was similar for PE+ trials and PE- trials: Web of Science (median 6.6 versus 7.1, respectively)

and Scopus (median 9.3 versus 9.5, respectively). Non-academic measures of dissemination,

however, tended to be higher for PE+ trials, compared with PE- trials. For example, the median

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

Altmetric Attention Score per year was 23.0 for PE+ trials compared with 5.4 for PE- trials. Likewise, the median PlumX Social Media score per year for PE+ trials was 46.6, compared with a median score of 7.6 for PE- trials. Last, while median PlumX Captures scores per year were higher for PE+ trials (41.5), compared with PE- trials (29.0), PE+ trials had a lower median PlumX Usage score per year (3.9), compared with PE- trials (41.9). For all dissemination metrics, the data showed a positively skewed (or right-skewed) distribution with most values clustering at smaller values; there were large IQRs for non-academic measures of dissemination indicating greater variability.

Table 3. Measures of academic and non-academic citation for PE+ and PE- paediatric RCTs published in *The BMJ*, 2011-2020

Measures of citation (median, IQR)	Total	PE +	PE -	<i>p</i> -value ¹
n	45	10	35	
Citation frequency per year (Web of Science)	6.9 (4.5, 13.1)	6.6 (6.0, 8.6)	7.1 (4.2, 14.0)	0.84
Citation frequency per year (Scopus)	9.5 (6.1, 15.5)	9.3 (8.0, 15.3)	9.5 (5.0, 17.5)	0.77
Altmetric Attention Score per year	7.3 (1.2, 21.8)	23.0 (3.9, 40.0)	5.4 (1.0, 17.8)	0.13
PlumX Citations per year	9.8 (6.6, 18.3)	9.5 (8.9, 16.8)	10.2 (5.5, 19.5)	0.88
PlumX Usage per year	37.7 (10.8, 75.8)	3.9 (0.0, 69.8)	41.9 (23.1, 78.7)	0.04
PlumX Captures per year	30.9 (22.0, 44.3)	41.5 (27.0, 80.8)	29.0 (17.2, 40.9)	0.04
PlumX Mentions per year	0.2 (0.1, 0.7)	0.3 (0.2, 0.9)	0.2 (0.1, 0.7)	0.37
PlumX Social Media per year	9.2 (5.3, 41.0)	46.6 (21.7, 128.5)	7.6 (4.2, 34.0)	0.02

¹ Citation measures for PE+ and PE- groups were compared using the Mann-Whitney test for continuous variables.

DISCUSSION

This systematic review collected and compared research quality and dissemination metrics for paediatric RCTs that engaged patients and families in the research process with trials that did not. Over a ten-year period, 10 of 45 RCTs published in *The BMJ* reported engaging patients and families in the research process. Descriptive data suggested that PE+ trials were somewhat more likely to use a PROM as the primary outcome, and were more likely to have

BMJ Open

more than one primary outcome, a larger sample size, and fewer participants lost to follow up, compared with PE- trials. Research quality and academic dissemination metrics were similar for PE+ and PE- trials. PE+ trials, however, had higher social media scores compared with PE- trials, suggesting that patient and family engagement in a paediatric RCT may increase the likelihood of dissemination of trial research findings to the public via social media networks.

In total, 22% of paediatric trials in this review reported patient and family engagement. This compares favourably with data on patient engagement in adult RCTs. For example, a systematic review by Benizri *et al*⁶¹ that examined 50 RCTs published in 2021 in three leading medical journals (not including *The BMJ*) noted that only 5% of the RCTs reported patient engagement. A previous systematic review of RCTs published between 2011 and 2016, suggested that less than 1% of trials reported any patient engagement.⁶²

Paediatric care is child and family-centered, therefore, patient and family engagement in the research process may be more likely in this clinical milieu. Second, the increasing frequency of patient and family engagement may be related to the fact that several national research funding agencies mandate patient engagement in research proposal submissions. Last, an important factor, was the introduction of a new policy in 2015 by *The BMJ* that required authors to provide a *Patient and Public Involvement* paragraph in the Methods section of submitted manuscripts.⁶³ This requirement provides authors with a mandate and corollary word count to report patient and family engagement in their research. In addition, the policy may encourage researchers to engage patients and families in their research, if *The BMJ* is the target journal.

It is unsurprising that PE+ paediatric trials in the review were somewhat more likely to use a PROM as the primary outcome, compared with PE- trials. In addition, the data suggested that PE+ trials had larger sample sizes and lower loss to follow up, compared with PE- trials.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open

Crocker *et al*⁶⁴ have shown that patient and public involvement in clinical trials significantly improves participant enrolment and may improve retention.

Patient and public involvement in health research has long been hypothesized to improve research quality and dissemination of findings, however, there are few empirical data on the topic.^{4,65,66} Barriers to precise measurement of engagement impact include inconsistent terminology for engagement, unpredictable reporting of engagement in the published literature, and the difficulty in accurately measuring the direct impact of engagement, given the role of other factors such as context, policy, and culture. This systematic review showed no material difference in the research quality of PE+ and PE- paediatric RCTs. Of note, relatively few trials in the sample were considered 'fair' or 'good' quality. The measure of research quality, however, was of the trial itself; we did not measure the "quality" of patient engagement. In this context, none of the RCTs in the sample (neither PE+ nor PE-) used a reporting tool, such as the Guidance for Reporting Involvement of Patients and Public (GRIPP2) checklist to describe patient and public involvement.⁶⁷ This lack of standardized reporting of patient engagement limits the analysis of the impact of patient and family engagement in paediatric RCTs.

Measures of academic dissemination - based on citation frequency per year - also showed no difference between PE+ and PE- trials. Non-academic dissemination measures, however, were generally higher among PE+ paediatric trials, compared with PE-trials. PlumX Usage scores were higher for PE- RCTs compared with PE+ RCTs; however, Usage scores (URL clicks, downloads, views) may reflect academic dissemination. PlumX Social Media scores (shares, likes, comments) more likely represent non-academic dissemination. To our knowledge, this is the first empirical study to examine the impact of patient and family engagement in paediatric RCTs on research quality and dissemination.

Strengths and Limitations

Strengths of this study included a comprehensive and rigorous search strategy and eligibility review process. In addition, published trial protocols and clinical trial registries such as ClinicalTrials.gov were also searched for information on engagement for eligible trials.

There were limitations to our study. First, only one database (MEDLINE) and one journal (*The BMJ*) were searched, limiting generalizability. *The BMJ* was selected deliberately, given the mandatory reporting requirement of submitting authors to report patient and public involvement in their research. In addition, the *a priori* sampling strategy was to examine high quality child health research and *The BMJ* is a high impact journal. Second, a ten-year sampling period with a 2011 start date was selected to align with national initiatives in patient and public involvement in health research (Canada's Strategy for Patient-Oriented Research and the Patient-Centered Outcomes Research Institute in the United States), as well as the 2015 The BMJ mandatory PPI reporting requirement. The small sample size of paediatric trials, however, limited a formal assessment of the impact of these initiatives. Third, there may have been misclassification of PE+ trials as PE- trials if patient and public involvement was not reported by manuscript authors. This issue is particularly relevant for those trials published prior to *The BMJ* policy in 2015 that mandated reporting of patient engagement. To mitigate this risk of misclassification, in addition to reviewing information reported in the published article, we also reviewed information in published trial protocols (when available) and clinical trial registries (when available) for all studies, when categorizing trials as PE+ or PE-. Other authors have noted that patient and family engagement is under reported in the published literature.^{68,69} Given the *a priori* hypothesis, and the finding of no difference in research quality between PE+ and PE- trials in our review, any bias because of misclassification, i.e., PE+ trials misclassified as PE- would likely have been

towards the null. Likewise, the academic citation frequency for any publication accumulates as the length of time from publication increases. Proportionately more PE- trials were published at the beginning of the study period, compared with PE+ trials. Therefore, given that academic citation frequencies per year were similar between PE+ and PE- trials, any bias would again have likely been towards the null.

CONCLUSION

Despite widespread recommendation for patient and family engagement in child health research, this systematic review showed that only one in five paediatric RCTs published in *The BMJ* over a ten-year period reported patient and family engagement. Research quality was similar for trials engaging patients and families compared with trials that did not. Patient and family engagement, however, was associated with higher non-academic dissemination metrics, in particular, social media attention, compared to trials with no patient and family engagement. Next steps include the development and application of standardized tools and methods to better measure the quantity, quality, and impact of patient engagement in paediatric RCTs.

CMB conceptualized and designed the study, designed the data collection instruments, coordinated and supervised the data extraction, performed the statistical analysis, interpreted the data, drafted the manuscript, critically reviewed and revised the manuscript. CM and FB conceptualized and designed the study, designed the data collection instruments, interpreted the data, drafted the manuscript, critically reviewed and revised the manuscript. NH performed the data extraction, performed the statistical analysis, interpreted the data, critically reviewed the manuscript for important intellectual content. KMN, TK, MB performed the data extraction, interpreted the data, critically reviewed the manuscript for important intellectual content. KMN, TK, MC performed the data, critically reviewed the manuscript for important intellectual content. KMN, TK, MB performed the data, critically reviewed the manuscript for important intellectual content. KMN, TK, MC performed the data, critically reviewed the manuscript for important intellectual content. All authors approved the final manuscript as submitted and agreed to be accountable for all aspects of the work. The corresponding author, as guarantor, accepts full responsibility for the finished article and attests that all listed authors meet the authorship criteria and that no others meeting the criteria have been omitted.

Funding: This research received no specific grant from any funding agency in the public, commercial or not-for-profit sectors.

Competing interests: None declared.

Patient consent: Not required.

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

REFERENCES

- 1. Canada's Strategy for Patient Oriented Research 2011. https://cihrirsc.gc.ca/e/44000.html#a1.1. Accessed February 9, 2024.
- Bate J, Ranasinghe N, Ling R, *et al.* Public and patient involvement in paediatric research. *Arch Dis Child Educ Pract* 2016;101(3):158–61.
- 3. Amirav I, Vandall-Walker V, Rasiah J, *et al.* Patient and researcher engagement in health research: a parent's perspective. *Pediatrics* 2017;140:e20164127.
- 4. Aubin D, Hebert M, Eurich D. The importance of measuring the impact of patient-oriented research. *CMAJ* 2019;191:e860-864.
- 5. Vanderhout S, Bhalla M, Van A, *et al.* The impact of patient and family engagement in child health research: a scoping review. *J Pediatr* 2023;253:115-128.
- Flynn R, Walton S, Scott SD. Engaging children and families in pediatric health research. *Research Involvement & Engagement* 2019 5, 32. https://doi.org/10.1186/s40900-019-0168-9.
- 7. Bailey S, Boddy K, Briscoe S, *et al.* Involving disabled children and young people as partners in research: a systematic review. *Child Care Health Dev* 2014;41:505-514.
- Shen S, Doyle-Thomas KAR, Beesley L, *et al.* How and why should we engage patients as co-researchers in health research? A scoping review of current practices. *Health Expectations* 2016;20:543-54.
- Bird M, Ouellette C, Whitmore C, *et al.* Preparing for patient partnership: a scoping review of patient partner engagement and evaluation in research. *Health Expectations* 2020;23:523-539.

2		
- 3 4	10.	Rouncefield-Swales A, Harris J, Carter B, et al. Children and young people's contribu
5 6		to public involvement and engagement activities in health-related research: A scoping
7 8		review. PLoS ONE 2021;16(6):e0252774. https://doi.org/10.1371/journal.pone.025277
9 10	11.	Moher D, Liberati A, Tetzlaff J, et al. PRISMA Group. Preferred reporting items for
11 12		systematic reviews and meta-analyses: the PRISMA statement. Int J Surg 2010;8(5):3.
13 14 15		341
16 17	12	Lefebure C. Glanville I. Briscoe S. at al. Technical Supplement to Chapter 4: Searchin
18	12.	Letebvie C, Gianvine J, Briscoe S, <i>et al.</i> Teeninear Supplement to Chapter 4. Searchin
19 20		and selecting studies. In: Higgins JPT, Thomas J, Chandler J, Cumpston M, Li T, Page
21 22		Welch VA (editors). Cochrane Handbook for Systematic Reviews of Interventions Ver
23 24 25		6.4 (updated February 2024). Cochrane, 2024. Available from
25 26 27		www.training.cochrane.org/handbook.
28 29	13.	Higgins JPT, Altman DG, Gøtzsche PC, et al. Cochrane Bias Methods Group, Cochran
30 31		Statistical Methods Group. The Cochrane Collaboration's tool for assessing risk of bia
32 33		randomised trials. <i>BMJ</i> 2011:343:d5928. doi:10.1136/bmi.d5928
34 35	14	Mongeon P. Paul-Hus A. The journal coverage of Web of Science and Sconus: A
36 37	17.	
38		comparative analysis. Scientometrics 2016;106:213–228.
40 41	15.	Shakeel Y, Alchokr R, Kruger J, et al. Altmetrics and citation counts: an empirical ana
42 43		of the computer science domain. Proceedings of the Joint Conference on Digital Libra
44 45		2022;17:1–11.
46 47	16.	Freedman SB, Parkin PC, Willan AR, et al. Rapid versus standard intravenous rehydra
48 49		in paediatric gastroenteritis: pragmatic blinded randomised clinical trial. BMJ
50 51		
52		2011;343:d6976.
53		
54 55		
56		
57 59		
оо 59		
60		For peer review only - http://bmjopen.bmj.com/site/about/guidelines.xhtml

- Andersson O, Hellström-Westas L, Andersson D, et al. Effect of delayed versus early umbilical cord clamping on neonatal outcomes and iron status at 4 months: a randomized controlled trial. BMJ 2011;343:d7157. Puder JJ, Marques-Vidal P, Schindler C, et al. Effect of multidimensional lifestyle 18. intervention on fitness and adiposity in predominantly migrant preschool children (Ballabeina): cluster randomised controlled trial. BMJ 2011;343:d6195. 19. van den Aardweg MTA, Boonacker CWB, Rovers MM, et al. Effectiveness of adenoidectomy in children with recurrent upper respiratory tract infections: open randomised controlled trial. BMJ 2011;343:d5154. Wake M, Tobin S, Girolametto L, et al. Outcomes of population based language promotion 20. for slow to talk toddlers at ages 2 and 3 years: Let's Learn Language cluster randomised controlled trial. BMJ 2011;343:d4741. 21. Kumar GT, Sachdev HS, Chellani H, et al. Effect of weekly vitamin D supplements on mortality, morbidity, and growth of low birthweight term infants in India up to age 6 months: randomised controlled trial. BMJ 2011;342:d2975. Gault EJ, Perry RJ, Cole TJ, et al. Effect of oxandrolone and timing of pubertal induction 22. on final height in Turner's syndrome: randomised, double blind, placebo controlled trial. BMJ 2011;342:d1980. 23. Porto AMF, Coutinho IC, Correia JB, et al. Effectiveness of antenatal corticosteroids in
 - Porto AMF, Coutinio IC, Correla JB, *et al.* Effectiveness of antenatal controsteroids in reducing respiratory disorders in late preterm infants: randomised clinical trial. *BMJ* 2011;342:d1696.
 - 24. Green JM, Wood AJ, Kerfoot MJ, *et al*. Group therapy for adolescents with repeated self harm: randomised controlled trial with economic evaluation. *BMJ* 2011;342:d682.

BMJ Open

25.	Sandison TG, Homsy J, Arinaitwe E, et al. Protective efficacy of co-trimoxazole
	prophylaxis against malaria in HIV exposed children in rural Uganda: a randomised
	clinical trial. <i>BMJ</i> 2011;342:d1617.
26.	Gill CJ, Phiri-Mazala G, Guerina NG, et al. Effect of training traditional birth attendants on
	neonatal mortality (Lufwanyama Neonatal Survival Project): randomised controlled study.
	<i>BMJ</i> 2011;342:d346.
27.	Gringras P, Gamble C, Jones A P, et al. Melatonin for sleep problems in children with
	neurodevelopmental disorders: randomised double masked placebo controlled trial. BMJ
	2012;345:e6664.
28.	Stallard P, Sayal K, Phillips R, et al. Classroom based cognitive behavioural therapy in
	reducing symptoms of depression in high risk adolescents: pragmatic cluster randomised
	controlled trial. <i>BMJ</i> 2012;345:e6058.
29.	Miller G, Luo R, Zhang L, et al. Effectiveness of provider incentives for anaemia reduction
	in rural China: a cluster randomised trial. BMJ 2012;345:e4809.
30.	Wen LM, Baur LA, Simpson JM, et al. Effectiveness of home based early intervention on
	children's BMI at age 2: randomised controlled trial. BMJ 2012;344:e3732.
31.	Waldén M, Atroshi I, Magnusson H, et al. Prevention of acute knee injuries in adolescent
	female football players: cluster randomised controlled trial. BMJ 2012;344:e3042.
32.	Robling M, McNamara R, Bennert K, et al. The effect of the Talking Diabetes consulting
	skills intervention on glycaemic control and quality of life in children with type 1 diabetes:
	cluster randomised controlled trial (DEPICTED study). BMJ 2012;344:e2359.
	23

33.	Merry SN, Stasiak K, Shepherd M, et al. The effectiveness of SPARX, a computerised self-
	help intervention for adolescents seeking help for depression: randomised controlled non-
	inferiority trial. BMJ 2012;344:e2598.
34.	Bhandari N, Mazumder S, Taneja S, et al. Effect of implementation of Integrated
	Management of Neonatal and Childhood Illness (IMNCI) programme on neonatal and
	infant mortality: cluster randomised controlled trial. BMJ 2012;344:e1634.
35.	Day C, Michelson D, Thomson S, et al. Evaluation of a peer led parenting intervention for
	disruptive behaviour problems in children: community based randomised controlled trial.
	<i>BMJ</i> 2012;344:e1107.
36.	Palmer DJ, Sullivan T, Gold MS, et al. Effect of n-3 long chain polyunsaturated fatty acid
	supplementation in pregnancy on infants' allergies in first year of life: randomised
	controlled trial. BMJ 2012;344:e184.
37.	Wake M, Lycett K, Clifford SA, et al. Shared care obesity management in 3-10 year old
	children: 12 month outcomes of HopSCOTCH randomised trial. BMJ 2013;346:f3092.
38.	Little P, Moore M, Kelly J, et al. Ibuprofen, paracetamol, and steam for patients with
	respiratory tract infections in primary care: pragmatic randomised factorial trial. BMJ
	2013;347:f6041.
39.	South East Asia Infectious Disease Clinical Research Network. Effect of double dose
	oseltamivir on clinical and virological outcomes in children and adults admitted to hospital
	with severe influenza: double blind randomised controlled trial. BMJ 2013;346:f3039.
40.	Stremler R, Hodnett E, Kenton L, et al. Effect of behavioural-educational intervention on
	sleep for primiparous women and their infants in early postpartum: multisite randomised
	controlled trial. BMJ 2013;346:f1164.
	24

BMJ Open

41.	Kipping RR, Howe LD, Jago R, et al. Effect of intervention aimed at increasing physical
	activity, reducing sedentary behaviour, and increasing fruit and vegetable consumption in
	children: Active for Life Year 5 (AFLY5) school based cluster randomised controlled trial.
	<i>BMJ</i> 2014;348:g3256.
42.	Attanasio OP, Bentham J, Fernández C, et al. Using the infrastructure of a conditional cash
	transfer program to deliver a scalable integrated early child development program in
	Colombia: cluster randomized controlled trial. BMJ 2014;349:g5785.
43.	Ma X, Zhou Z, Yi H, et al. Effect of providing free glasses on children's educational
	outcomes in China: cluster randomized controlled trial. BMJ 2014;349:g5740.
44.	van Wijk RM, van Vlimmeren LA, Groothuis-Oudshoorn CGM, et al. Helmet therapy in
	infants with positional skull deformation: randomised controlled trial. BMJ
	2014;348:g2741.
45.	Sung V, Hiscock H, Tang MLK, et al. Treating infant colic with the probiotic Lactobacillus
	reuteri: double blind, placebo controlled randomised trial. BMJ 2014;348:g2107.
46.	Dodd JM, Turnbull D, McPhee AJ, et al. Antenatal lifestyle advice for women who are
	overweight or obese: LIMIT randomised trial. BMJ 2014;348:g1285.
47.	Le Noury J, Nardo JM, Healy D, et al. Restoring Study 329: efficacy and harms of
	paroxetine and imipramine in treatment of major depression in adolescence. BMJ
	2015;351:h4320.
48.	Andersson N, Nava-Aguilera E, Arosteguí J, et al. Evidence based community mobilization
	for dengue prevention in Nicaragua and Mexico (Camino Verde, the Green Way): cluster
	randomized controlled trial. BMJ 2015;351:h3267.

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open

Pace D, Khatami A, McKenna J, et al. Immunogenicity of reduced dose priming schedules of serogroup C meningococcal conjugate vaccine followed by booster at 12 months in infants: open label randomised controlled trial. BMJ 2015;350:h1554. He FJ, Wu Y, Feng X, et al. School based education programme to reduce salt intake in 50. children and their families (School-EduSalt): cluster randomised controlled trial. BMJ 2015;350:h770. Hiscock H, Sciberras E, Mensah F, et al. Impact of a behavioural sleep intervention on 51. symptoms and sleep in children with attention deficit hyperactivity disorder, and parental mental health: randomised controlled trial. BMJ 2015:350:h68. Hyttel-Sorensen S, Pellicer A, Alderliesten T, et al. Cerebral near infrared spectroscopy 52. oximetry in extremely preterm infants: phase II randomised clinical trial. BMJ 2015;350:g7635. Kaufman J, Fitzpatrick P, Tosif S, et al. Faster clean catch urine collection (Quick-Wee 53. method) from infants: randomised controlled trial. BMJ 2017;357:j1341. Vinding RK, Stokholm J, Sevelsted A, et al. Effect of fish oil supplementation in 54. pregnancy on bone, lean, and fat mass at six years: randomised clinical trial. BMJ 2018;362:k3312. 55. Santer M, Ridd MJ, Francis NA, et al. Emollient bath additives for the treatment of childhood eczema (BATHE): multicentre pragmatic parallel group randomised controlled trial of clinical and cost effectiveness. BMJ 2018;361:k1332. 56. Adab P, Pallan MJ, Lancashire ER, *et al.* Effectiveness of a childhood obesity prevention programme delivered through schools, targeting 6 and 7 year olds: cluster randomised controlled trial (WAVES study). BMJ 2018;360:k211.

BMJ Open

57.	Skoog Ståhlgren G, Tyrstrup M, Edlund C, et al. Penicillin V four times daily for five days
	versus three times daily for 10 days in patients with pharyngotonsillitis caused by group A
	streptococci: randomised controlled, open label, non-inferiority study. BMJ 2019;
	367:15337.
58.	Webb NJA, Woolley RL, Lambe T, et al. Long term tapering versus standard prednisolone
	treatment for first episode of childhood nephrotic syndrome: phase III randomised
	controlled trial and economic evaluation. BMJ 2019;365:11800.
59.	Blair JC, McKay A, Ridyard C, et al. for the SCIPI investigators. Continuous subcutaneous
	insulin infusion versus multiple daily injection regimens in children and young people at
	diagnosis of type 1 diabetes: pragmatic randomised controlled trial and economic
	evaluation. BMJ 2019;365:11226.
60.	Roberts SB, Franceschini MA, Silver RE, et al. Effects of food supplementation on
	cognitive function, cerebral blood flow, and nutritional status in young children at risk of
	undernutrition: randomized controlled trial. BMJ 2020;370:m2397.
61.	Benizri N, Hallot S, Burns K, et al. Patient and Family Representation in Randomized
	Clinical Trials Published in 3 Medical and Surgical Journals: A Systematic Review. JAMA
	Network Open 2022;5(9):e2230858.
52.	Fergusson D, Monfaredi Z, Pussegoda K, et al. The prevalence of patient engagement in
	published trials: a systematic review. Research Involvement & Engagement 2018;4:17.
63.	Richards T, Schroter S, Price A, et al. Better together: patient partnership in medical
	journals. BMJ 2018;362:k3798 doi: 10.1136/bmj.k3798.

64.	Crocker J, Ricci-Cabello I, Parker A, et al. Impact of patient and public involvement on
	enrolment and retention in clinical trials: systematic review and meta-analysis. BMJ
	2018;363:k4738.
65.	Petit-Zeman S, Locock L. Bring on the evidence. Nature 2013;501:160-61.
66.	Snape D, Kirkham J, Britten N, et al. Exploring perceived barriers, drivers, impacts and the
	need for evaluation of public involvement in health and social care research: a modified
	Delphi study. BMJ Open 2014;4:e004943.
67.	Staniszewska S, Brett J, Simera I, et al. GRIPP2 reporting checklists: tools to improve
	reporting of patient and public involvement in research. BMJ 2017;358:j3453.
68.	Price A, Schroter S, Snow R, et al. Frequency of reporting on patient and public
	involvement (PPI) in a general medical journal: a descriptive study. BMJ Open
	2018;8:e020452. doi:10.1136/bmjopen-2017-020452.
69.	Vanderhout S, Nevins P, Nicholls S, et al. Patient and public involvement in pragmatic
	trials: online survey of corresponding authors of published trials. CMAJ Open 2023 Sep
	19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115.

Figure Legend					
Figure 1. Areas of patient and family engagement in PE+ paediatric trials published in <i>The BMJ</i>					
Figure 2. Cochrane Risk of Bias Scores for PE+ and PE- trials					
2					

15	
16	
17	
18	
19	
20	
21	
22	
23	
24	
25	
26	
27	
28	
29	
30	
31	
32	
33	
34	
35	
36	
37	
38	
39	
40	
41	
42	
43	
44	
45	
46	
47	
48	
49	
50	
51	
52	
53	
54	
55	
56	
57	
58	
59	

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

BMJ Open



¹Patients and families could be engaged in more than one aspect of the research process in a single study

For peer review only - http://bmjopen.bmj.com/site/about/guidelines.xhtml

 BMJ Open

PE+RCTs



BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool Protected by copyright, instlightion for heres seleted fourth and idats/mithing, but the initian technologies.
2
з
ر ۸
4
5
6
7
8
a
10
10
11
12
13
14
15
16
10
17
18
19
20
21
22
22
23
24
25
26
27
20
20
29
30
31
32
33
24
34
35
36
37
38
39
40
40
41
42
43
44
45
46
47
4/
48
49
50
51
52
52
55
54
55
56
57
58
50
27

1

Supplementary Table 1. Ovid MEDLINE search strategy to identify indexed pediatric randomized controlled trials (RCTs) published in *The BMJ*

Search Strategy	Description	Results
1. randomized controlled trial.pt.		574707
2. controlled clinical trial.pt.		94982
3. randomized.ab.		571502
4. placebo.ab.		230695
5. clinical trials as topic.sh.	Cochrane Highly Sensitive Search	200252
6. randomly.ab.	(Steps 1 to 10)	388759
7. trial.ti.		268047
8. 1 or 2 or 3 or 4 or 5 or 6 or 7		1465478
9. exp animals/not humans.sh.		5035319
10. 8 not 9		1348058
11. (BMJ or British Medical	Limit by BMJ	182994
Journal).jn		
12. limit 11 to yr="2015"	Limit by year*	3335
13. limit 12 to (address or comment or		1214
editorial or letter or observational study	Filter out addresses, comments, editorials, letters,	
or meta-analysis or review or systematic review)	observational studies, meta-analyses, reviews, and	
14 12 not 13	systematic reviews	2121
15 10 and 14	All RCTs meeting above criteria	97
16. limit 15 to all child <0 to 18 years>	Limit to RCTs indexed as including children	22
17 limit 15 to all adult <19 plus years>	Limit to RCTs indexed as including adults	30
18. 15 not (16 or 17)	RCTs not indexed by any age group	57
19 16 not 17	RCTs indexed as only including children	10
20 17 not 16	RCTs indexed as only including adults	18
21 (16 or 17) not (19 or 20)	RCTs indexed as including both children and	12
	adults	

*Example shows the search query for the year 2015. This search strategy was repeated for each calendar year from 2011 to 2020, inclusive.



1				BMJ Open	0.1136/bmjc
1 2 3 4	Supplementary '	Table 2. Paediatric RCTs report	ting patient and fa	amily engagement published in <i>The BMJ</i> (2	yright 2011-2020) <u>, inc. 4</u>
5 6	Author, year Country	Title	Clinical Category	Areas of Engagement	Descriptize Symmary of Patient and Pamily Input
7 8 9 10 11 12 13	Puder, 2011 Switzerland	Effect of multidimensional lifestyle intervention on fitness and adiposity in predominantly migrant preschool children (Ballabeina): cluster randomised controlled trial.	Cardiology	 Preparation Intervention development/refinement 	"The intervention was developed with input from exercise physiologists, preschool and primary school teachers, paediatrications dietitians, psychologists, and variors and keholders including experts for the primary families."
14 15 16 17 18 19 20 21 22 23 24 25 26 27 28 29	Robling, 2012 United Kingdom	The effect of the Talking Diabetes consulting skills intervention on glycaemic control and quality of life in children with type 1 diabetes: cluster randomised controlled trial (DEPICTED study).	Endocrinology	 Engagement of youth participants Preparation Intervention development/refinement 	In Acknowle generats: "We than to be all principal investigators, members are each clinical team, and local UKCRN research staff participating in the 26 trial centres, and to all the practitioners and families who contributed to the development of the Talking Drabetes intervention. We particularly thank the dedicated input of our parent and patient representative CC (co-applicant in particular during the development shase of the study. Other contributers to programme development: students and saff at Whitchurch High School (drame club) "
30 31 32 33 34 35 36 37 38 39 40 41 42	Kipping, 2014 England	Effect of intervention aimed at increasing physical activity, reducing sedentary behaviour, and increasing fruit and vegetable consumption in children: active for Life Year 5 (AFLY5) school-based cluster randomised controlled trial.	Public health or preventive medicine	 Preparation Intervention development/refinement Execution Intervention delivery Input on study conduct Translation Dissemination and implementation of results 	"We worked with primary school teachers, the local primary care trust (public halth commissioners), and the local council government) in South Gloucestershipe, in the south west of England, to determine whether this intervention could be adapted for use in the UK, whether delivering the adapted intervention within the National Curriculum was feasible, and whether a pilot randomined controlled trial provided
∠ 43 44					LTA

For peer review only - http://bmjopen.bmj.com/site/about/guidelines.xhtml

 Andersson, Andersson, Andersson, Distance based community Nicaragua and Nicara	Page 37 of 40			BMJ Open	.1136/bmj
 Input on study design Intervention delivery Translation Dissemination and implementation of results Dissemination and implementation of results distribution distrest distribution	Andersson, Ev Andersson, Ev 2015 21 22 23 24 25 26 27 2015 29 Nicaragua and pr Mexico 31 32 33 34 35 36 37 38 39 40 50 50 60 71 80 90 90 90 90 90 90 90 90 90 9	vidence based community nobilization for dengue revention in Nicaragua and fexico (Camino Verde, the freen Way): cluster andomized controlled trial.	Infectious diseases and vaccines	 ✓ Engagement of youth participants Preparation Intervention development/refinement Execution Input on study design Intervention delivery Translation Dissemination and implementation of results 	evidence of promise for the intervention sufficient to justify a full scale trial. We undertook qualitative work with parents and teachers to develop the intervention is such a way that it involved parents; this showed that child- parent interactive homework would be feasible and macceptable to them. Furthermore, our work with teachers and parents clearly showed that a more intensive prevention in school would not have the process evaluations will be undertakent thoughout the study to identify any issues that might impact dissemination Process evaluation will be conducted using focus groups with children, face to-face interviews with teachers and school administrators, and telephone, interviews with parents." "Patients who previously had dengue and their families were intimately involved in design of the intervention. Facilitators convened and ran intervention design groups with & 10 people, usually separatel for men and women, to discuss survey realls, cost implication, and specific prevention strategies in each community. Patients and their families were also central to dissemination of the baseline information (i.e., assessment of risk of dengue in their community), which helped o motivate community

				BMJ Open	0.1136/b cted by •
1 2					mjopen- copyrigh
3 4 5 6 7 8 9 10 11 12 13 14 15 16	Kaufman, 2017 Australia	Faster clean catch urine collection (Quick-Wee method) from infants: randomised controlled trial.	Urology	 Preparation Development of the research question Selection of outcomes Intervention development/refinement Execution Intervention delivery 	"Feedback from the parents and carers of 40 particinants in the preceding pilot study contributed to refinement and confirmation of the study research question and outcome measures. Parents and carers were asked to rate their satisfaction with the intervention and could provide additional comments. Parental statisfaction with the intervention in the pilot by was high and no responders were dissatisfied with the intervention 20 demonstrating feasibility for this lager definitive trial."
17 18 19 20 21 22 23 24 25 26 27 28 29 30 31 32 33	Santer, 2018 England, Wales	Emollient bath additives for the treatment of childhood eczema (BATHE): multicentre pragmatic parallel group randomised controlled trial of clinical and cost effectiveness.	Dermatology	 Preparation Intervention development/refinement Execution Input on study design Recruitment of participants Translation Dissemination and implementation of results 	"The trial management group included an experienced patient and public involvement @PI) co-applicant (AR) who participated in all phases of the trial design, including planning recruitment and recruitment materials. We also consulted members of the Centre of Evidence Based Dermatology patient panel at the trial design stage, and we sought additional PPI representation when plasming how to disseminate findings. The independent trial steering committee included a PPI member. The results will be emailed to all trial participants and published on the trial website."
34 35 36 37 38 39 40 41	Adab, 2018 United Kingdom	Effectiveness of a childhood obesity prevention programme delivered through schools, targeting 6- and 7-year-olds: cluster randomised controlled trial (WAVES study).	Public health or preventive medicine	 Preparation Selection of outcomes Intervention development/refinement Execution Input on study design Recruitment of participants 	"Public in volgement was a key feature of the early phases of trial development and feasibility testing before this main trial. Intervention development was informed by detailed consultation with parents, teachers, and gether school staff. The intervention was further refined and the
41 42 43 44					ŧΕΖ-LTA

Pag	je 39 of 40			BMJ Open	0.1136/b
1 2					mjopen. copyrigl
$\begin{array}{c} 3\\ 4\\ 5\\ 6\\ 7\\ 8\\ 9\\ 10\\ 11\\ 12\\ 13\\ 14\\ 15\\ 16\\ 17\\ 18\\ 19\\ 20\\ 21\\ 22\\ 23\\ 24\\ 25\\ 26\\ 27\\ 28\\ 29\\ 30\\ 31\\ 32\\ 33\\ 34 \end{array}$	Webb, 2019 United Kingdom	Long term tapering versus standard prednisolone treatment for first episode of childhood nephrotic syndrome: phase III randomised controlled trial and economic evaluation.	Nephrology	 Execution Input on study design Input on study conduct Results interpretation Translation Dissemination and implementation of results 	process for measuring outcomes tested and adapted by asking the children, parents, and to cher about their experiences during the feasibility study. Measures of wellbeing and body dissatisfaction, were included as outcomes based on meisperceived importance among scher staff. Our research team includes and duisor at the Health Education Service, who has regular connect with schools and advised on school meter participant recruitment." "The trial proposed was reviewed by represent for of the UK Nephrotic Syndrome Trust (NeST) and the UK Renal Patient of upport Group, who provided valuable input about trial design, acceptibility of trial visit frequency; and adverse event monitoring. A NeST representative participated on the trial specing committee. After publication, the trial results will be disseminated to all study collaborators. The plain English summary of the study results will be sent to the participants and/or their perents through their responsible clinician. The summary will also be available on the NeST website and the Participants acuk/prednos)."
35 36 37 38 39 40	England, Wales	Continuous subcutaneous insulin infusion versus multiple daily injection regimens in children and young people at diagnosis of type 1 diabetes: pragmatic	Endocrinology	 Engagement of youth participants Preparation Selection of outcomes Execution Input on study design Recruitment of participants 	interpretation over undertaken in close discussion with patients and their families. Young people were consulted on the design of the study including impact of participation outcome
41 42 43 44 45	<u> </u>	Fo	r peer review only -	http://bmjopen.bmj.com/site/about/guidelines	.xhtml

aterials. Parents of ople with type 1 s of the trial e and trial steering l on recruitment and their s and their families il with parent
involvement held to obtain ort for village onal discussions nembers in study health workers iffic control ed the plan for all ir supplement at te feeding centers d also asked for Supplementation esponsibilities. rkers and parents ulted about ation of NEWSUP r pilot; based on ed the amount of UP recipe and n from a baked (to prevent aste), and l increase in e first study week come accustomed
コン 3 いわ ii シロ む 1 ご e d ひ a r シし n (a l .e c .

Page 41 of 40

	, ,		incl	
Criteria / Risk of bias	Total	PE +	P# - 86	<i>p</i> -value ²
n	45	10	35 34	-
Q1. Random Sequence Generation – selection bias			or on	
Low risk of bias	43 (95.6)	9 (90.0)	34 (@7.1)	-
Unclear	1 (2.2)	0 (0.0)	1 (2.9)a	0.40
High risk of bias	1 (2.2)	1 (10.0)		
Q2. Allocation Concealment – selection bias			1 to	
Low risk of bias	37 (82.2)	9 (90.0)	28 (8)	-
Unclear	4 (8.9)	0 (0.0)		0.80
High risk of bias	4 (8.9)	1 (10.0)	3 (8 B) 8	
Q3. Selective Reporting – reporting bias			ata l	
Low risk of bias	12 (26.7)	2 (20.0)	10 (38.6 -	_
Unclear	31 (68.9)	7 (70.0)	24 👼 8.6	0.54
High risk of bias	2 (4.4)	1 (10.0)	1 (2.9)	
Q4. Other Bias – bias not covered elsewhere			bm	
Low risk of bias	31 (68.9)	7 (70.0)	24 (58.6	
Unclear	5 (11.1)	1 (10.0)	4 (b 1.4 b	1.00
High risk of bias	9 (20.0)	2 (20.0)	7 (20.02.	
Q5. Blinding of Participants and Personnel – performance bias		O_{h}	imil	
Low risk of bias	22 (48.9)	4 (40.0)	18 (51.46	
Unclear	5 (11.1)	1 (10.0)	4 (별.4)	0.87
High risk of bias	18 (40.0)	5 (50.0)	13 (0 7. b	
Q6. Blinding of Outcome Assessment – detection bias			7, 2(
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.43	
Unclear	9 (20.0)	3 (30.0)	6 (17.1 g	0.74
High risk of bias	5 (11.1)	1 (10.0)	4 (11.4 §]

			mjope copyri		
Q7. Incomplete Outcome Data – attrition bias			n-202 ght, ir		
Low risk of bias	31 (68.9)	6 (60.0)	25 (2 1.4)		
Unclear	13 (28.9)	4 (40.0)	9 (\$5.7 8	0.57	
High risk of bias	1 (2.2)	0 (0.0)	1 (2 .9)		
Overall Quality Rating			n 12 r us		
GOOD or FAIR	17 (37.8)	4 (40.0)	13 (87.13	1.00	
POOR	28 (62.2)	6 (60.0)	22 (2 1 9 1 1 1 2 2 (2 1 9 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1	1.00	
¹ Values are shown as n (%). ² PE+ and PE- groups were compared using Fisher's Exact test.			2025, Downlo asmushogesci led to text and		

beer review only

BMJ Open

cted by 0.1136/b

2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA

data mining, Al training, and similar technologies

BMJ Open

Research quality and dissemination of paediatric randomized controlled trials with and without patient and family engagement: systematic review

Journal:	BMJ Open
Manuscript ID	bmjopen-2024-086934.R2
Article Type:	Original research
Date Submitted by the Author:	12-Feb-2025
Complete List of Authors:	Borkhoff, Cornelia; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT); University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Hattangadi, Nayantara; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Nurse, Kimberly; University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Kay, Tatjana; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Bhalla, Manav; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences Mahood, Quenby; The Hospital for Sick Children, Division of Hospital Library and Archives Buchanan, Francine; Hospital for Sick Children Research Institute, Child Health and Evaluative Sciences Taljaard, Monica; Ottawa Hospital Research Institute, Clinical Epidemiology Program; University of Ottawa, School of Epidemiology and Public Health Cohen, Eyal; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT); University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT); University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Research Institute, Child Health Evaluative Sciences; University of Toronto, Edwin S.H. Leong Centre for Healthy Children Parkin, Patricia; The Hospital for Sick Children, Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT); University of Toronto, Institute of Health Policy, Management and Evaluation, Dalla Lana School of Public Health; Hospital for Sick Children Re

1	
2	
3	
1	
5	
5	
0	
/	
8	
9	
10	
11	
12	
13	
14	
15	
16	
17	
18	
19	
20	
20	
21 22	
∠∠ วว	
∠3 24	
24	
25	
26	
27	
28	
29	
30	
31	
32	
33	
34	
35	
36	
37	
38	
39	
40	
41	
42	
<u>4</u> 2	
ΔΛ	
<u>⊿</u> 5	
4-) /6	
40 47	
4/	
48	
49	
50	
51	
52	
53	
54	
55	

Primary Subject Heading :	Paediatrics
Secondary Subject Heading:	Research methods
Keywords:	PAEDIATRICS, Child, Social Media, Randomized Controlled Trial



Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, AI training, and similar technologies



I, the Submitting Author has the right to grant and does grant on behalf of all authors of the Work (as defined in the below author licence), an exclusive licence and/or a non-exclusive licence for contributions from authors who are: i) UK Crown employees; ii) where BMJ has agreed a CC-BY licence shall apply, and/or iii) in accordance with the terms applicable for US Federal Government officers or employees acting as part of their official duties; on a worldwide, perpetual, irrevocable, royalty-free basis to BMJ Publishing Group Ltd ("BMJ") its licensees and where the relevant Journal is co-owned by BMJ to the co-owners of the Journal, to publish the Work in this journal and any other BMJ products and to exploit all rights, as set out in our <u>licence</u>.

The Submitting Author accepts and understands that any supply made under these terms is made by BMJ to the Submitting Author unless you are acting as an employee on behalf of your employer or a postgraduate student of an affiliated institution which is paying any applicable article publishing charge ("APC") for Open Access articles. Where the Submitting Author wishes to make the Work available on an Open Access basis (and intends to pay the relevant APC), the terms of reuse of such Open Access shall be governed by a Creative Commons licence – details of these licences and which <u>Creative Commons</u> licence will apply to this Work are set out in our licence referred to above.

Other than as permitted in any relevant BMJ Author's Self Archiving Policies, I confirm this Work has not been accepted for publication elsewhere, is not being considered for publication elsewhere and does not duplicate material already published. I confirm all authors consent to publication of this Work and authorise the granting of this licence.

2	
3	Desearch quality and dissemination of prediatric randomized controlled trials with and without
4	Research quality and dissemination of pactitatic fandomized controlled thats with and without
5	patient and family engagement: systematic review
6	
7	Cornelia M. Borkhoff, MSc, PhD ^{1,2,3} Nayantara Hattangadi, MHSc ³ , Kimberly M. Nurse, MSc ^{2,3} ,
8	Tatiana Kay MSc ³ Manay Bhalla BSc ³ Ouenby Mahood MI ⁴
9	Eranging Buchangn ML is DhD ³ Maniag Taligard MSg DhD ^{5,6} Eval Cohon MD MSg 123.7.8
10	Tranchie Duchanan, MILIS, FIID', Monica Taijaaru, MiSc, FIID, ³⁷ , Eyar Conen, MID, MiSc, ^{37,000}
11	Patricia C. Parkin, MD ^{1,2,3,7} , Colin Macarthur, MBChB, PhD ^{1,2,3,7}
12	
13	Affiliations:
14	¹ Division of Paediatric Medicine and Paediatric Outcomes Research Team (PORT) Hospital for
15	Sick Children Toronto Ontario Canada:
16	² Institute of Health Deline Management and Eschaption Delle Long Calcelle Collect of Dellin Health
17	² Institute of Health Policy, Management and Evaluation, Daha Lana School of Public Health,
18	University of Toronto, Toronto, Ontario, Canada;
10	³ Child Health Evaluative Sciences, SickKids Research Institute, Toronto, Ontario, Canada;
20	⁴ Division of Hospital Library and Archives, Hospital for Sick Children, Toronto, Ontario,
20	Canada [.]
21	5 Clinical Enidemiology Program Ottawa Hagnital Pagaarah Instituta (OHPI) The Ottawa
22	Userite1 Conversion 501 Sworth David Ottoma Outomic Conversion, The Ottawa
23	Hospital, General Campus, 501 Smyth Road, Ottawa, Ontario, Canada;
24	⁶ School of Epidemiology and Public Health, University of Ottawa, Ottawa, Ontario, Canada;
25	⁷ Department of Paediatrics, Temerty Faculty of Medicine, University of Toronto, Toronto,
20	Ontario. Canada:
27	⁸ Edwin S.H. Leong Centre for Healthy Children University of Toronto, Toronto, ON M5G 0A4
28	Conodo
29	Callaua
30	
31	Address correspondence to: Cornelia M. Borkhoff, PhD, The Hospital for Sick Children
32	Research Institute, Peter Gilgan Centre for Research and Learning, 686 Bay St. Toronto, ON.
33	Canada M5G $0A4$ E-mail: corv borkhoff@sickkids ca
34	Canada WISO 0/14. L-man. Cory.corknon@sickRids.ca
35	Short title: Patient engagement quality and dissemination of paediatric randomized controlled
36	trials
37	ullais
38	
39	Keywords: patient engagement; paediatrics; child; randomized controlled trials; research
40	quality; dissemination; social media
41	
42	
43	
44	
45	
46	
47	
48	
49	
50	
51	
52	
53	
54	
55	
56	

ABSTRACT

Objectives: Authentic patient and family engagement in child health research is defined as researchers working in partnership with patients and families on all aspects of the research process, including refining the research question, tailoring the intervention, devising study procedures, and disseminating study findings. While there is good evidence of a positive impact of patient engagement on the research process, on research teams, and on patient partners, there are few empirical data on the impact of patient and family engagement on research quality and dissemination. We conducted a systematic review to compare research quality and dissemination metrics for paediatric randomized controlled trials (RCTs) that engaged patients and families in the research process, with trials that did not.

Design: Systematic review using The Cochrane Highly Sensitive Search to identify RCTs. **Data sources:** Ovid MEDLINE from 1 January 2011 through to 31 December 2020.

Eligibility criteria: We included RCTs involving children and youth (<18 years of age) published in *The BMJ* (a peer-reviewed general medical journal).

Data extraction and synthesis: Trials were categorized as those engaging patients and families (PE+) and those that did not (PE-). A standardized review form was used to confirm trial eligibility and extract data on study characteristics. Two reviewers independently screened and sorted RCTs into PE+ and PE- groups, extracted data, and assessed research quality using the modified Cochrane Risk of Bias Tool (based on 7 methodologic criteria). Dissemination of RCT findings was determined using measures of academic and non-academic citation collected from Web of Science and Scopus.

Results: From 2011 to 2020, *The BMJ* published 45 RCTs involving children and youth. Only 10/45 RCTs (22%) reported engaging patients and families in the research process. Research

quality for PE+ and PE- paediatric RCTs was similar; 4/10 (40%) of PE+ trials, and 13/35 (37%) of PE- trials were rated as 'fair' or 'good' (p=1.00). Academic citation frequency per year was similar for PE+ trials and PE- trials: Web of Science (median 6.6 versus 7.1, respectively; p=0.84). Non-academic dissemination measures were generally higher among PE+ trials, for example, median PlumX Social Media score per year for PE+ trials was 46.6, compared with a median score of 7.6 for PE- trials (p=0.02).

Conclusions: Despite increasing interest in patient and family engagement in child health research, this review showed that few paediatric RCTs report patient engagement activity. Research quality was similar for trials engaging patients and families compared with those that did not. Patient and family engagement in the trial, however, was associated with higher metrics for social media attention, compared with trials with no engagement.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

Data availability statement

All data relevant to the study are included in the article or uploaded as supplementary

information. Extracted data are available on request to the corresponding author.

Strengths and limitations of this study

- We conducted a systematic review of paediatric randomized controlled trials (RCTs) published in *The BMJ* to compare research quality and dissemination metrics for trials that engaged patients and families in the research process with trials that did not.
- We assessed research quality using the modified Cochrane Risk of Bias Tool for RCTs (based on 7 methodological quality criteria) and dissemination metrics using measures of academic and non-academic citation collected from Web of Science and Scopus.
- We only sampled paediatric RCTs published in one journal, but *The BMJ* was selected deliberately, given the requirement of submitting authors to report patient and public involvement in their research.
- There may have been misclassification of RCTs that engaged patients and families (PE+ trials) as trials that did not (PE-), particularly for trials published prior to *The BMJ* policy in 2015 that mandated reporting of patient engagement.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

INTRODUCTION

Patient-oriented research has been defined as a continuum of research that engages patients as partners, focuses on patient-identified priorities and outcomes, and integrates the knowledge generated into policy and practice to improve health care outcomes.¹ The term 'patient' is overarching and includes those with lived experience of a health issue, as well as informal caregivers, such as family and friends. Patient-oriented research is hypothesized to improve the quality, relevance, and uptake of health research.²

Authentic patient engagement in health research involves a partnership between patients and researchers working together on any or all aspects of the research process, including choosing the research question, selecting the study design, tailoring the intervention, devising study procedures, and dissemination of study findings. Barriers to patient engagement, however, such as parenting commitments, work schedules, and long research timelines, can make patient and family engagement in child health research challenging.³

Aubin *et al*⁴ proposed a framework for measuring *impact* related to patient-oriented research. The framework identifies potential impact metrics across four domains: improvements to research process, impact on policies, impact on health outcomes, and contribution to social change. In the context of child health research, there is qualitative evidence of a positive impact of patient and family engagement on the research process (refinement of the research question, intervention, materials), on research teams (enhanced knowledge, cultural competency), and on patient partners (empowerment, skills development).⁵⁻¹⁰ There are, however, few quantitative data on the impact of patient-oriented research, and to our knowledge, the impact of patient and family engagement on the search, and to our knowledge, the impact of patient and family engagement on the search and to search teams (enserch process for the search process).

BMJ Open

Therefore, the objective of this study was to conduct a systematic review of paediatric RCTs published in *The BMJ* (a peer-reviewed general medical journal with a high impact factor), examine measures of research quality and dissemination (academic and non-academic), and compare RCTs that reported engaging patients and families in the research process with RCTs that did not.

METHODS

This study followed the Preferred Reporting Items for Systematic Reviews and Metaanalyses (PRISMA) reporting guidelines.¹¹ We did not register a protocol prior to conducting the review.

Search Strategy and Information Sources

An information science specialist (QM) developed an Ovid MEDLINE electronic search strategy to identify RCTs involving children and youth (<18 years of age) published in *The BMJ* over the 10-year period January 01, 2011, through to December 31, 2020. The search strategy was optimized for sensitivity and specificity using The Cochrane Highly Sensitive Search to identify RCTs.¹² A search start date of 2011 was selected to align with national initiatives in patient and public involvement in health research (Canada's Strategy for Patient-Oriented Research and the Patient-Centered Outcomes Research Institute in the United States), as well as the 2015 *The BMJ* mandatory PPI reporting requirement. A search end date of 2020 was chosen, given the widespread impact of the COVID-19 pandemic on health research. **Supplementary Table 1** describes the search strategy.

Inclusion and Exclusion Criteria

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

RCTs published in *The BMJ* over the 10-year period that assessed a specific intervention were eligible. Only primary reports of trial results related to the trial primary outcome were included, i.e., publications reporting a secondary analysis of RCT data were excluded. Trials were included if the study population was limited to children and youth from birth to less than 18 years of age. Excluded were clinical trial study protocols; non-randomized comparative trials; cross-sectional studies; non-comparative studies; systematic, scoping, and narrative reviews; conference abstracts; and editorials/commentaries.

Study Selection and Data Extraction

A standardized review form was developed to confirm trial eligibility and extract data on study characteristics. Two reviewers (two of NH, KMN, TK, MB) independently performed an eligibility assessment for each article using the inclusion and exclusion criteria, first screening titles and abstracts and then full texts of potentially relevant articles. Any discrepancies were resolved through discussion and adjudication with a third reviewer (CMB).

For this review, patient and family engagement in research (a continuum of research that engages patients as partners, focuses on patient-identified priorities, and aims to improve patient outcomes)¹ was considered the 'exposure of interest' and research quality and dissemination were the 'outcomes of interest'. Therefore, trials were categorized into two groups: the PE+ group (trials that reported engaging patients and families in the research process) and the PE- group (trials that <u>did not report</u> engaging patients and families in the research process). Recognizing that information on patient and family engagement might not necessarily be reported in *The BMJ* publication, we also reviewed information in published trial protocols (when available), and clinical trial registries such as ClinicalTrials.gov (when available) for all studies.

Page 11 of 41

BMJ Open

Two reviewers independently extracted data on RCT characteristics and methods, including author name; year of publication; trial setting; trial type; multicenter (yes/no); multinational (yes/no); participant age; primary outcome; sample size; number lost to follow-up; and patient and/or family engagement (yes/no). For the PE+ trials, additional data were collected: number of patient/family/caregivers engaged; youth engagement (yes/no); and area of engagement in the research process.

Outcomes

'Research quality' was assessed using the modified Cochrane Risk of Bias Tool for RCTs.¹³ The Cochrane tool appraises 7 methodological quality criteria: 1) random sequence generation (selection bias); 2) allocation concealment (selection bias); 3) selective reporting (reporting bias); 4) other bias; 5) blinding of participants and researchers (performance bias); 6) blinding of outcome assessment (detection bias); and 7) incomplete outcome data (attrition bias). Two reviewers independently evaluated the research quality of eligible trials, and any discrepancies were resolved through discussion with a third reviewer. An overall quality rating for each trial based on a summation of the 7 criteria was also determined; trials were categorized as 'Good,' 'Fair,' or 'Poor,' based on the Risk of Bias Tool guide.

Dissemination of trial results was determined using measures of academic and nonacademic citation. Data on citation numbers and citation frequencies from Web of Science and Scopus were collected up to February 14, 2024. Web of Science has a narrower coverage of biomedical journals than Scopus and may therefore give a more conservative citation count.¹⁴ The primary academic dissemination metric captured was citation frequency per year (citation count divided by the number of years since publication).

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

PlumX data from Scopus (<u>https://plumanalytics.com/learn/about-metrics/</u>) and altmetric data from Web of Science (<u>https://www.altmetric.com/research-access/</u>) were used to determine non-academic citation.¹⁵ PlumX data capture interactions with a research output in the online environment across five domains: Citations (indexes, clinical or policy citations); Usage (URL clicks, downloads, views); Captures (bookmarks, favourites, follows); Mentions (news media, blog posts, Wikipedia references); and Social Media (shares, likes, comments). The Altmetric Attention Score is a weighted count of the public attention a research article has received based on a variety of sources (citations, news media, social media mentions, blogs etc.). PlumX and Altmetric Attention Scores were described by year (total interactions divided by the number of years since publication of the trial).

Statistical Analysis

Descriptive statistics were used to describe the frequency of paediatric RCTs published in *The BMJ* (by calendar year and patient engagement) and other RCT characteristics. Medians and interquartile ranges (IQRs) were used to describe continuous variables that were non-normally distributed. PE+ and PE- trials were compared on research quality and dissemination metrics using Fisher's Exact test for categorical outcomes and the Mann-Whitney test for continuous variables. Statistical significance was defined as p<0.05; all statistical tests were 2-sided.

Ethics

Ethics approval for the study was not required as no data were collected from human subjects and only peer-reviewed published studies in the public domain were reviewed.

Patient and public involvement

BMJ Open

This systematic review was conducted in partnership with a patient partner (FB, coauthor) who provided input on framing the research question, research methods, interpreting the research findings, writing the manuscript, and preparing dissemination plans.

RESULTS

The literature search strategy (see **Supplementary Table 1**) identified 29,944 citations in *The BMJ* up to 2020, with 818 (2.7%) classified as potential RCTs. Of these, 132 (16%) were indexed as including children, and were assessed for eligibility. After review, 87 records were excluded, leaving 45 paediatric trials included in the review (the search flowchart and reasons for exclusion are described in **Supplementary Figure 1**).

Over the period 2011 to 2020, *The BMJ* published 45 RCTs involving children and youth.¹⁶⁻⁶⁰ As shown in **Table 1**, only 10 of the 45 RCTs (22%) reported engaging patients and/or families in the trial. (Additional information on the 10 PE+ trials is provided in **Supplementary Table 2**). In alignment with the 2015 mandate from *The BMJ* in *Instructions to Authors*, all seven of the PE+ trials published from 2015 onwards included a *Patient and Public Involvement* (PPI) paragraph in the Methods section of the manuscript. Of the PE- paediatric trials, seven were published in 2015 or later, however, only three included a PPI paragraph; the four trials without a PPI paragraph were all published in 2015. Full implementation of the new reporting policy may have been slower than anticipated.

Table 1. Number of paediatric RCTs published in *The BMJ* by patient engagement status and year of publication

BMJ	Published RCTs	Patient engagement		No patient	engagement
		PPI paragraph	PPI paragraph	PPI paragraph	PPI paragraph
		yes	no	yes	no
2011	11	0	1	0	10

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

1	
2	
3	
4 5	
5 6	
7	
י 8	
9	
10	
11	
12	
13	
14	
15	
16	
17	
18	
19	
20	
21	
22	
23	
24	
25	
20	
28	
29	
30	
31	
32	
33	
34	
35	
36	
37	
38	
39 40	
40 //1	
42	
43	
44	
45	
46	
47	
48	
49	
50	
51	
52	
53	
54	
55	
56 57	
5/ 50	
50 50	
17	

60

2012	10	0	1	0	9
2013	4	0	0	0	4
2014	6	0	1	0	5
2015	6	1	0	1	4
2016	0	0	0	0	0
2017	1	1	0	0	0
2018	3	2	0	1	0
2019	3	2	0	1	0
2020	1	1	0	0	0
2011-2020	45	7	3	3	32

As described in **Figure 1** and **Supplementary Table 2**, among the 10 PE+ trials, studies described a spectrum of engagement, including engagement in the research preparation phase (9 [90%]), execution phase (8 [80%]), and translation phase (5 [50%]), and often across more than one aspect of the study. For example, 8 PE+ trials had patient and/or family input on the development of the intervention,^{18,32,41,48,53,55,56,60} and 5 PE+ trials had input on the dissemination of trial results.^{41,48,55,58,59} Of the 10 PE+ RCTs, three trials that included study participants 12 to 17 years of age, specifically engaged youth partners;^{32,48,59} the remaining seven trials engaged only parents or caregivers. Only one PE+ trial engaged families in developing the research question and only this trial provided information on the numbers engaged; parents and caregivers of 40 children contributed to refinement of the research question, selection of outcome measures, and feedback on the intervention.⁵³

Table 2 describes the characteristics of PE+ RCTs (n=10) and PE- RCTs (n=35). Across the 45 trials, the most common clinical categories were mental health (7 [16%]), endocrinology (4 [9%]), neonatology (4 [9%], public health/preventative medicine (3 [7%]), infectious diseases and vaccines (3 [7%]), neurology (3 [7%]), and respirology (3 [7%]). Given the small sample size, formal statistical testing was not conducted. The descriptive data, however, suggest that

PE+ RCTs were somewhat more likely to use a patient-reported outcome measure (PROM) as the primary outcome. Of the 7 trials using a PROM, three trials used a parent proxy measure (2 PE+ and 1 PE-) as participating children were as young as 1 to 3 years of age. Likewise, based on descriptive data, PE+ RCTs were also more likely to have more than one primary outcome, a larger sample size, and a lower percentage of loss to follow up, compared with PE- RCTs.

TRIAL CHARACTERISTICS	TOTAL	PE +	PE -
Number of trials	45	10	35
Age of study participants ² , median (IQR)	5.1 (1.8, 10.2)	5.1 (3.7, 7.9)	5.5 (1.5, 10.3)
Trial setting, n (%)			
Primary Care	9 (20.0)	1 (10.0)	8 (22.9)
Hospital	18 (40.0)	4 (40.0)	14 (40.0)
Community/Population	18 (40.0)	5 (50.0)	13 (37.1)
Trial type, n (%)			
Prevention	16 (35.6)	4 (40.0)	12 (34.3)
Treatment	29 (64.4)	6 (60.0)	23 (65.7)
Multicenter trial, yes, n (%)	39 (86.7)	10 (100.0)	29 (82.9)
Multinational trial, yes, n (%)	10 (22.2)	4 (40.0)	6 (17.1)
Multiple primary outcomes, yes, n (%)	5 (11.1)	3 (30.0)	2 (5.7)
Primary outcome:			
Biomedical, yes, n (%)	14 (31.1)	4 (40.0)	10 (28.6)
Clinical, yes, n (%)	29 (64.4)	6 (60.0)	23 (65.7)
PROM, yes, n (%)	7 (15.6)	3 (30.0)	4 (11.4)
Sample size, median (IQR)	433 (237, 1420)	671 (354,	366 (185,
		1467)	1420)
Percentage lost to follow up, median (IQR)	9.6 (3.8, 13.3)	4.7 (2.8, 12.7)	10.5 (3.9,
			16.5)

Table 2. Characteristics of PE+ and PE- paediatric RCTs published in *The BMJ*, 2011-2020¹

¹ Values are shown as n (%) or median (inter quartile range, IQR).

² 13 trials were excluded from mean age analysis (2 PE+ and 11 PE-) as only median age or age range of participants was reported: Freedman 2011, Kumar 2011, Porto 2011, Green 2011, Gill 2011, Bhandari 2012, Little 2013, Stremler 2013, Dodd 2014, Andersson 2015, Hyttel-Sorensen 2015, Skoog Stahlgren 2019, and Blair 2019.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

Research Quality - assessed using the modified Cochrane Risk of Bias Tool - was similar for PE+ and PE- RCTs (see **Figure 2**). Only 4/10 (40%) of PE+ trials, and 13/35 (37%) of PEtrials were rated as 'fair' or 'good' (p=1.00). Additional data on the quality scores for PE+ and PE- trials is shown in **Supplementary Table 3**.

Table 3 describes academic and non-academic measures of dissemination for the PE+ and PE- trials. With respect to academic measures of dissemination, citation frequency per year was similar for PE+ trials and PE- trials: Web of Science (median 6.6 versus 7.1, respectively) and Scopus (median 9.3 versus 9.5, respectively). Non-academic measures of dissemination, however, tended to be higher for PE+ trials, compared with PE- trials. For example, the median Altmetric Attention Score per year was 23.0 for PE+ trials compared with 5.4 for PE- trials. Likewise, the median PlumX Social Media score per year for PE+ trials was 46.6, compared with a median score of 7.6 for PE- trials. Last, while median PlumX Captures scores per year were higher for PE+ trials (41.5), compared with PE- trials (29.0), PE+ trials had a lower median PlumX Usage score per year (3.9), compared with PE- trials (41.9). For all dissemination metrics, the data showed a positively skewed (or right-skewed) distribution with most values clustering at smaller values; there were large IQRs for non-academic measures of dissemination indicating greater variability.

Table 3. Measures of academic and non-academic citation for PE+ and PE- paediatric RCTs published in *The BMJ*, 2011-2020

Measures of citation (median, IQR)	Total	PE +	PE -	<i>p</i> -value ¹
n	45	10	35	
Citation frequency per year (Web of Science)	6.9 (4.5, 13.1)	6.6 (6.0, 8.6)	7.1 (4.2, 14.0)	0.84
Citation frequency per year (Scopus)	9.5 (6.1, 15.5)	9.3 (8.0, 15.3)	9.5 (5.0, 17.5)	0.77
Altmetric Attention Score per year	7.3 (1.2, 21.8)	23.0 (3.9, 40.0)	5.4 (1.0, 17.8)	0.13
PlumX Citations per year	9.8 (6.6, 18.3)	9.5 (8.9, 16.8)	10.2 (5.5, 19.5)	0.88
PlumX Usage per year	37.7 (10.8, 75.8)	3.9 (0.0, 69.8)	41.9 (23.1, 78.7)	0.04
PlumX Captures per year	30.9 (22.0, 44.3)	41.5 (27.0, 80.8)	29.0 (17.2, 40.9)	0.04

PlumX Mentions per year	0.2 (0.1, 0.7)	0.3 (0.2, 0.9)	0.2 (0.1, 0.7)	0.37
PlumX Social Media per year	9.2 (5.3, 41.0)	46.6 (21.7, 128.5)	7.6 (4.2, 34.0)	0.02

¹ Citation measures for PE+ and PE- groups were compared using the Mann-Whitney test for continuous variables.

DISCUSSION

This systematic review collected and compared research quality and dissemination metrics for paediatric RCTs that engaged patients and families in the research process with trials that did not. Over a ten-year period, 10 of 45 RCTs published in *The BMJ* reported engaging patients and families in the research process. Descriptive data suggested that PE+ trials were somewhat more likely to use a PROM as the primary outcome, and were more likely to have more than one primary outcome, a larger sample size, and fewer participants lost to follow up, compared with PE- trials. Research quality and academic dissemination metrics were similar for PE+ and PE- trials. PE+ trials, however, had higher social media scores compared with PEtrials, suggesting that patient and family engagement in a paediatric RCT may increase the likelihood of dissemination of trial research findings to the public via social media networks.

In total, 22% of paediatric trials in this review reported patient and family engagement. This compares favourably with data on patient engagement in adult RCTs. For example, a systematic review by Benizri *et al*⁶¹ that examined 50 RCTs published in 2021 in three leading medical journals (not including *The BMJ*) noted that only 5% of the RCTs reported patient engagement. A previous systematic review of RCTs published between 2011 and 2016, suggested that less than 1% of trials reported any patient engagement.⁶²

Paediatric care is child and family-centered, therefore, patient and family engagement in the research process may be more likely in this clinical milieu. Second, the increasing frequency of patient and family engagement may be related to the fact that several national research

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

funding agencies mandate patient engagement in research proposal submissions. Last, an important factor, was the introduction of a new policy in 2015 by *The BMJ* that required authors to provide a *Patient and Public Involvement* paragraph in the Methods section of submitted manuscripts.⁶³ This requirement provides authors with a mandate and corollary word count to report patient and family engagement in their research. In addition, the policy may encourage researchers to engage patients and families in their research, if *The BMJ* is the target journal.

It is unsurprising that PE+ paediatric trials in the review were somewhat more likely to use a PROM as the primary outcome, compared with PE- trials. In addition, the data suggested that PE+ trials had larger sample sizes and lower loss to follow up, compared with PE- trials. Crocker *et al*⁶⁴ have shown that patient and public involvement in clinical trials significantly improves participant enrolment and may improve retention.

Patient and public involvement in health research has long been hypothesized to improve research quality and dissemination of findings, however, there are few empirical data on the topic.^{4,65,66} Barriers to precise measurement of engagement impact include inconsistent terminology for engagement, unpredictable reporting of engagement in the published literature, and the difficulty in accurately measuring the direct impact of engagement, given the role of other factors such as context, policy, and culture. This systematic review showed no material difference in the research quality of PE+ and PE- paediatric RCTs. Of note, relatively few trials in the sample were considered 'fair' or 'good' quality. The measure of research quality, however, was of the trial itself; we did not measure the "quality" of patient engagement. In this context, none of the RCTs in the sample (neither PE+ nor PE-) used a reporting tool, such as the Guidance for Reporting Involvement of Patients and Public (GRIPP2) checklist to describe

BMJ Open

patient and public involvement.⁶⁷ This lack of standardized reporting of patient engagement limits the analysis of the impact of patient and family engagement in paediatric RCTs.

Measures of academic dissemination - based on citation frequency per year - also showed no difference between PE+ and PE- trials. Non-academic dissemination measures, however, were generally higher among PE+ paediatric trials, compared with PE-trials. PlumX Usage scores were higher for PE- RCTs compared with PE+ RCTs; however, Usage scores (URL clicks, downloads, views) may reflect academic dissemination. PlumX Social Media scores (shares, likes, comments) more likely represent non-academic dissemination. To our knowledge, this is the first empirical study to examine the impact of patient and family engagement in paediatric RCTs on research quality and dissemination.

Strengths and Limitations

Strengths of this study included a comprehensive and rigorous search strategy and eligibility review process. In addition, published trial protocols and clinical trial registries such as ClinicalTrials.gov were also searched for information on engagement for eligible trials.

There were limitations to our study. First, only one database (MEDLINE) and one journal (*The BMJ*) were searched, limiting generalizability. *The BMJ* was selected deliberately, given the mandatory reporting requirement of submitting authors to report patient and public involvement in their research. In addition, the *a priori* sampling strategy was to examine high quality child health research and *The BMJ* is a high impact journal. Second, a ten-year sampling period with a 2011 start date was selected to align with national initiatives in patient and public involvement in health research (Canada's Strategy for Patient-Oriented Research and the Patient-Centered Outcomes Research Institute in the United States), as well as the 2015 *The BMJ* mandatory PPI reporting requirement. The small sample size of paediatric trials, however, limited a formal

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

assessment of the impact of these initiatives. Third, there may have been misclassification of PE+ trials as PE- trials if patient and public involvement was not reported by manuscript authors. This issue is particularly relevant for those trials published prior to *The BMJ* policy in 2015 that mandated reporting of patient engagement. To mitigate this risk of misclassification, in addition to reviewing information reported in the published article, we also reviewed information in published trial protocols (when available) and clinical trial registries (when available) for all studies, when categorizing trials as PE+ or PE-. Other authors have noted that patient and family engagement is under reported in the published literature.^{68,69} Given the *a priori* hypothesis, and the finding of no difference in research quality between PE+ and PE- trials in our review, any bias because of misclassification, i.e., PE+ trials misclassified as PE- would likely have been towards the null. Likewise, the academic citation frequency for any publication accumulates as the length of time from publication increases. Proportionately more PE- trials were published at the beginning of the study period, compared with PE+ trials. Therefore, given that academic citation frequencies per year were similar between PE+ and PE- trials, any bias would again have likely been towards the null.

CONCLUSION

Despite widespread recommendation for patient and family engagement in child health research, this systematic review showed that only one in five paediatric RCTs published in *The BMJ* over a ten-year period reported patient and family engagement. Research quality was similar for trials engaging patients and families compared with trials that did not. Patient and family engagement, however, was associated with higher non-academic dissemination metrics, in particular, social media attention, compared to trials with no patient and family engagement.

1		
3	Next steps include the development and application of standardized tools and methods to better	
4	Next steps menude the development and appreation of standardized tools and methods to better	
5	measure the quantity, quality, and impact of patient engagement in paediatric RCTs.	
6 7		
8		
9		
10 11		
12		
13		
14		
15		
17		
18		
19		
20		
21		
23		
24		
25		
20		
28		
29		
30 31		
32		
33		
34		
35 36		
37		
38		
39		
40 41		
42		
43		
44		
45 46		
47		
48		
49		
50 51		
52		
53		
54		
55 56		
57		
58	19	
59	For poor roviow only http://bmicnon.hmi.com/cita/about/cividalines.yhtml	
60	i or peer review only - http://binjopen.binj.com/site/about/guidelines.xhtml	

Contributors

CMB conceptualized and designed the study, designed the data collection instruments, coordinated and supervised the data extraction, performed the statistical analysis, interpreted the data, drafted the manuscript, critically reviewed and revised the manuscript. CM and FB conceptualized and designed the study, designed the data collection instruments, interpreted the data, drafted the manuscript, critically reviewed and revised the manuscript. NH performed the data extraction, performed the statistical analysis, interpreted the data, critically reviewed the manuscript for important intellectual content. KMN, TK, MB performed the data extraction, interpreted the data, critically reviewed the manuscript for important intellectual content. QM developed the search strategy, performed data curation, interpreted the data, critically reviewed the manuscript for important intellectual content. MT, EC, PCP interpreted the data, critically reviewed the manuscript as submitted and agreed to be accountable for all aspects of the work. The corresponding author, as guarantor, accepts full responsibility for the finished article and attests that all listed authors meet the authorship criteria and that no others meeting the criteria have been omitted.

Funding: This research received no specific grant from any funding agency in the public, commercial or not-for-profit sectors.

Competing interests: None declared.

Patient consent: Not required.

60

1		
2 3 4	RE	FERENCES
5 6	1.	Canada's Strategy for Patient Oriented Research 2011. https://cihr-
7 8		irsc.gc.ca/e/44000.html#a1.1. Accessed February 9, 2024.
9 10 11	2.	Bate J, Ranasinghe N, Ling R, et al. Public and patient involvement in paediatric research.
12 13		Arch Dis Child Educ Pract 2016;101(3):158–61.
14 15	3.	Amirav I, Vandall-Walker V, Rasiah J, et al. Patient and researcher engagement in health
16 17		research: a parent's perspective. Pediatrics 2017;140:e20164127.
19 20	4.	Aubin D, Hebert M, Eurich D. The importance of measuring the impact of patient-oriented
21 22		research. CMAJ 2019;191:e860-864.
23 24 25	5.	Vanderhout S, Bhalla M, Van A, et al. The impact of patient and family engagement in
25 26 27		child health research: a scoping review. J Pediatr 2023;253:115-128.
28 29	6.	Flynn R, Walton S, Scott SD. Engaging children and families in pediatric health research.
30 31		Research Involvement & Engagement 2019 5, 32. https://doi.org/10.1186/s40900-019-
32 33 34		0168-9.
35 36	7.	Bailey S, Boddy K, Briscoe S, et al. Involving disabled children and young people as
37 38		partners in research: a systematic review. Child Care Health Dev 2014;41:505-514.
39 40	8.	Shen S, Doyle-Thomas KAR, Beesley L, et al. How and why should we engage patients as
41 42 43		co-researchers in health research? A scoping review of current practices. Health
44 45		Expectations 2016;20:543-54.
46 47	9.	Bird M, Ouellette C, Whitmore C, et al. Preparing for patient partnership: a scoping review
48 49 50		of patient partner engagement and evaluation in research. Health Expectations
51 52		2020;23:523-539.
53 54		
55 56 57		
- ·		21

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool .

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies

BMJ Open

10.

review. PLoS ONE 2021;16(6):e0252774. https://doi.org/10.1371/journal.pone.0252774

- Moher D, Liberati A, Tetzlaff J, *et al.* PRISMA Group. Preferred reporting items for systematic reviews and meta-analyses: the PRISMA statement. *Int J Surg* 2010;8(5):336-341.
- Lefebvre C, Glanville J, Briscoe S, *et al.* Technical Supplement to Chapter 4: Searching for and selecting studies. In: Higgins JPT, Thomas J, Chandler J, Cumpston M, Li T, Page MJ, Welch VA (editors). Cochrane Handbook for Systematic Reviews of Interventions Version 6.4 (updated February 2024). Cochrane, 2024. Available from www.training.cochrane.org/handbook.
- Higgins JPT, Altman DG, Gøtzsche PC, *et al.* Cochrane Bias Methods Group, Cochrane Statistical Methods Group. The Cochrane Collaboration's tool for assessing risk of bias in randomised trials. *BMJ* 2011;343:d5928. doi:10.1136/bmj.d5928
- Mongeon P, Paul-Hus A. The journal coverage of Web of Science and Scopus: A comparative analysis. *Scientometrics* 2016;106:213–228.
- 15. Shakeel Y, Alchokr R, Kruger J, *et al.* Altmetrics and citation counts: an empirical analysis of the computer science domain. *Proceedings of the Joint Conference on Digital Libraries* 2022;17:1–11.
- Freedman SB, Parkin PC, Willan AR, *et al.* Rapid versus standard intravenous rehydration in paediatric gastroenteritis: pragmatic blinded randomised clinical trial. *BMJ* 2011;343:d6976.

BMJ Open

17.	Andersson O, Hellström-Westas L, Andersson D, et al. Effect of delayed versus early
	umbilical cord clamping on neonatal outcomes and iron status at 4 months: a randomized
	controlled trial. <i>BMJ</i> 2011;343:d7157.
18.	Puder JJ, Marques-Vidal P, Schindler C, et al. Effect of multidimensional lifestyle
	intervention on fitness and adiposity in predominantly migrant preschool children
	(Ballabeina): cluster randomised controlled trial. BMJ 2011;343:d6195.
19.	van den Aardweg MTA, Boonacker CWB, Rovers MM, et al. Effectiveness of
	adenoidectomy in children with recurrent upper respiratory tract infections: open
	randomised controlled trial. BMJ 2011;343:d5154.
20.	Wake M, Tobin S, Girolametto L, et al. Outcomes of population based language promotion
	for slow to talk toddlers at ages 2 and 3 years: Let's Learn Language cluster randomised
	controlled trial. BMJ 2011;343:d4741.
21.	Kumar GT, Sachdev HS, Chellani H, et al. Effect of weekly vitamin D supplements on
	mortality, morbidity, and growth of low birthweight term infants in India up to age 6
	months: randomised controlled trial. BMJ 2011;342:d2975.
22.	Gault EJ, Perry RJ, Cole TJ, et al. Effect of oxandrolone and timing of pubertal induction
	on final height in Turner's syndrome: randomised, double blind, placebo controlled trial.
	<i>BMJ</i> 2011;342:d1980.
23.	Porto AMF, Coutinho IC, Correia JB, et al. Effectiveness of antenatal corticosteroids in
	reducing respiratory disorders in late preterm infants: randomised clinical trial. BMJ
	2011;342:d1696.
24.	Green JM, Wood AJ, Kerfoot MJ, et al. Group therapy for adolescents with repeated self
	harm: randomised controlled trial with economic evaluation. BMJ 2011;342:d682.
	/ •

25. Sandison TG, Homsy J, Arinaitwe E, et al. Protective efficacy of co-trimoxazole prophylaxis against malaria in HIV exposed children in rural Uganda: a randomised clinical trial. BMJ 2011;342:d1617. Gill CJ, Phiri-Mazala G, Guerina NG, et al. Effect of training traditional birth attendants on 26. neonatal mortality (Lufwanyama Neonatal Survival Project): randomised controlled study. *BMJ* 2011;342:d346. Gringras P, Gamble C, Jones A P, et al. Melatonin for sleep problems in children with 27. neurodevelopmental disorders: randomised double masked placebo controlled trial. BMJ 2012;345:e6664. Stallard P, Sayal K, Phillips R, et al. Classroom based cognitive behavioural therapy in 28. reducing symptoms of depression in high risk adolescents: pragmatic cluster randomised controlled trial. BMJ 2012;345:e6058. 29. Miller G, Luo R, Zhang L, et al. Effectiveness of provider incentives for anaemia reduction in rural China: a cluster randomised trial. BMJ 2012;345:e4809. Wen LM, Baur LA, Simpson JM, et al. Effectiveness of home based early intervention on 30. children's BMI at age 2: randomised controlled trial. BMJ 2012;344:e3732. 31. Waldén M, Atroshi I, Magnusson H, et al. Prevention of acute knee injuries in adolescent female football players: cluster randomised controlled trial. *BMJ* 2012;344:e3042. 32. Robling M, McNamara R, Bennert K, et al. The effect of the Talking Diabetes consulting skills intervention on glycaemic control and quality of life in children with type 1 diabetes: cluster randomised controlled trial (DEPICTED study). BMJ 2012;344:e2359.
BMJ Open

33.	Merry SN, Stasiak K, Shepherd M, et al. The effectiveness of SPARX, a computerised self-
	help intervention for adolescents seeking help for depression: randomised controlled non-
	inferiority trial. BMJ 2012;344:e2598.
34.	Bhandari N, Mazumder S, Taneja S, et al. Effect of implementation of Integrated
	Management of Neonatal and Childhood Illness (IMNCI) programme on neonatal and
	infant mortality: cluster randomised controlled trial. BMJ 2012;344:e1634.
35.	Day C, Michelson D, Thomson S, et al. Evaluation of a peer led parenting intervention for
	disruptive behaviour problems in children: community based randomised controlled trial.
	<i>BMJ</i> 2012;344:e1107.
36.	Palmer DJ, Sullivan T, Gold MS, et al. Effect of n-3 long chain polyunsaturated fatty acid
	supplementation in pregnancy on infants' allergies in first year of life: randomised
	controlled trial. BMJ 2012;344:e184.
37.	Wake M, Lycett K, Clifford SA, et al. Shared care obesity management in 3-10 year old
	children: 12 month outcomes of HopSCOTCH randomised trial. BMJ 2013;346:f3092.
38.	Little P, Moore M, Kelly J, et al. Ibuprofen, paracetamol, and steam for patients with
	respiratory tract infections in primary care: pragmatic randomised factorial trial. BMJ
	2013;347:f6041.
39.	South East Asia Infectious Disease Clinical Research Network. Effect of double dose
	oseltamivir on clinical and virological outcomes in children and adults admitted to hospital
	with severe influenza: double blind randomised controlled trial. BMJ 2013;346:f3039.
40.	Stremler R, Hodnett E, Kenton L, et al. Effect of behavioural-educational intervention on
	sleep for primiparous women and their infants in early postpartum: multisite randomised
	controlled trial. BMJ 2013;346:f1164.
	25
	25

BMJ Open

- 41. Kipping RR, Howe LD, Jago R, *et al.* Effect of intervention aimed at increasing physical activity, reducing sedentary behaviour, and increasing fruit and vegetable consumption in children: Active for Life Year 5 (AFLY5) school based cluster randomised controlled trial. *BMJ* 2014;348:g3256.
- 42. Attanasio OP, Bentham J, Fernández C, *et al.* Using the infrastructure of a conditional cash transfer program to deliver a scalable integrated early child development program in Colombia: cluster randomized controlled trial. *BMJ* 2014;349:g5785.
- 43. Ma X, Zhou Z, Yi H, *et al.* Effect of providing free glasses on children's educational outcomes in China: cluster randomized controlled trial. *BMJ* 2014;349:g5740.
- 44. van Wijk RM, van Vlimmeren LA, Groothuis-Oudshoorn CGM, *et al.* Helmet therapy in infants with positional skull deformation: randomised controlled trial. *BMJ* 2014;348:g2741.
- 45. Sung V, Hiscock H, Tang MLK, *et al.* Treating infant colic with the probiotic Lactobacillus reuteri: double blind, placebo controlled randomised trial. *BMJ* 2014;348:g2107.
- 46. Dodd JM, Turnbull D, McPhee AJ, *et al*. Antenatal lifestyle advice for women who are overweight or obese: LIMIT randomised trial. *BMJ* 2014;348:g1285.
- Le Noury J, Nardo JM, Healy D, *et al.* Restoring Study 329: efficacy and harms of paroxetine and imipramine in treatment of major depression in adolescence. *BMJ* 2015;351:h4320.
- Andersson N, Nava-Aguilera E, Arosteguí J, *et al.* Evidence based community mobilization for dengue prevention in Nicaragua and Mexico (Camino Verde, the Green Way): cluster randomized controlled trial. *BMJ* 2015;351:h3267.

BMJ Open

49.	Pace D, Khatami A, McKenna J, et al. Immunogenicity of reduced dose priming schedules
	of serogroup C meningococcal conjugate vaccine followed by booster at 12 months in
	infants: open label randomised controlled trial. BMJ 2015;350:h1554.
50.	He FJ, Wu Y, Feng X, et al. School based education programme to reduce salt intake in
	children and their families (School-EduSalt): cluster randomised controlled trial. BMJ
	2015;350:h770.
51.	Hiscock H, Sciberras E, Mensah F, et al. Impact of a behavioural sleep intervention on
	symptoms and sleep in children with attention deficit hyperactivity disorder, and parental
	mental health: randomised controlled trial. BMJ 2015;350:h68.
52.	Hyttel-Sorensen S, Pellicer A, Alderliesten T, et al. Cerebral near infrared spectroscopy
	oximetry in extremely preterm infants: phase II randomised clinical trial. BMJ
	2015;350:g7635.
53.	Kaufman J, Fitzpatrick P, Tosif S, et al. Faster clean catch urine collection (Quick-Wee
	method) from infants: randomised controlled trial. BMJ 2017;357:j1341.
54.	Vinding RK, Stokholm J, Sevelsted A, et al. Effect of fish oil supplementation in
	pregnancy on bone, lean, and fat mass at six years: randomised clinical trial. BMJ
	2018;362:k3312.
55.	Santer M, Ridd MJ, Francis NA, et al. Emollient bath additives for the treatment of
	childhood eczema (BATHE): multicentre pragmatic parallel group randomised controlled
	trial of clinical and cost effectiveness. BMJ 2018;361:k1332.
56.	Adab P, Pallan MJ, Lancashire ER, et al. Effectiveness of a childhood obesity prevention
	programme delivered through schools, targeting 6 and 7 year olds: cluster randomised
	controlled trial (WAVES study). BMJ 2018;360:k211.
	27

- 57. Skoog Ståhlgren G, Tyrstrup M, Edlund C, *et al.* Penicillin V four times daily for five days versus three times daily for 10 days in patients with pharyngotonsillitis caused by group A streptococci: randomised controlled, open label, non-inferiority study. *BMJ* 2019; 367:15337.
- 58. Webb NJA, Woolley RL, Lambe T, *et al.* Long term tapering versus standard prednisolone treatment for first episode of childhood nephrotic syndrome: phase III randomised controlled trial and economic evaluation. *BMJ* 2019;365:11800.
- 59. Blair JC, McKay A, Ridyard C, *et al.* for the SCIPI investigators. Continuous subcutaneous insulin infusion versus multiple daily injection regimens in children and young people at diagnosis of type 1 diabetes: pragmatic randomised controlled trial and economic evaluation. *BMJ* 2019;365:11226.
- Roberts SB, Franceschini MA, Silver RE, *et al.* Effects of food supplementation on cognitive function, cerebral blood flow, and nutritional status in young children at risk of undernutrition: randomized controlled trial. *BMJ* 2020;370:m2397.
- Benizri N, Hallot S, Burns K, *et al.* Patient and Family Representation in Randomized Clinical Trials Published in 3 Medical and Surgical Journals: A Systematic Review. *JAMA Network Open* 2022;5(9):e2230858.
- 62. Fergusson D, Monfaredi Z, Pussegoda K, *et al.* The prevalence of patient engagement in published trials: a systematic review. *Research Involvement & Engagement* 2018;4:17.
- Richards T, Schroter S, Price A, *et al.* Better together: patient partnership in medical journals. *BMJ* 2018;362:k3798 doi: 10.1136/bmj.k3798.

BMJ Open

64.	Crocker J, Ricci-Cabello I, Parker A, et al. Impact of patient and public involvement on	
	enrolment and retention in clinical trials: systematic review and meta-analysis. BMJ	
	2018;363:k4738.	
65.	Petit-Zeman S, Locock L. Bring on the evidence. Nature 2013;501:160-61.	
66.	Snape D, Kirkham J, Britten N, et al. Exploring perceived barriers, drivers, impacts and t	he
	need for evaluation of public involvement in health and social care research: a modified	
	Delphi study. BMJ Open 2014;4:e004943.	
67.	Staniszewska S, Brett J, Simera I, et al. GRIPP2 reporting checklists: tools to improve	
	reporting of patient and public involvement in research. BMJ 2017;358:j3453.	
68.	Price A, Schroter S, Snow R, et al. Frequency of reporting on patient and public	
	involvement (PPI) in a general medical journal: a descriptive study. BMJ Open	
	2018;8:e020452. doi:10.1136/bmjopen-2017-020452.	
69.	Vanderhout S, Nevins P, Nicholls S, et al. Patient and public involvement in pragmatic	
	trials: online survey of corresponding authors of published trials. CMAJ Open 2023 Sep	
	19;11(5):E826-E837. doi: 10.9778/cmajo.20220198. PMID: 37726115.	
		20
		29

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool

Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.

Figure Legend

Figure 1. Areas of patient and family engagement in PE+ paediatric trials published in *The BMJ*

Figure 2. Cochrane Risk of Bias Scores for PE+ and PE- trials



¹Patients and families could be engaged in more than one aspect of the research process in a single study

BMJ Open

PE+RCTs



Supplementary Table 1. Ovid MEDLINE search strategy to identify indexed pediatric randomized

Search Strategy	Description	Results
1. randomized controlled trial.pt.		574707
2. controlled clinical trial.pt.		94982
3. randomized.ab.		571502
4. placebo.ab.		230695
5. clinical trials as topic.sh.	Cochrane Highly Sensitive Search	200252
6. randomly.ab.	(Steps 1 to 10)	388759
7. trial.ti.		268047
8. 1 or 2 or 3 or 4 or 5 or 6 or 7		1465478
9. exp animals/not humans.sh.		5035319
10. 8 not 9		1348058
11. (BMJ or British Medical	Limit by BMJ	182994
Journal).jn		
12. limit 11 to yr="2015"	Limit by year*	3335
13. limit 12 to (address or comment or		1214
editorial or letter or observational study	Filter out addresses, comments, editorials, letters,	
or meta-analysis or review or	observational studies, meta-analyses, reviews, and	
14 12 not 13	systematic reviews	2121
15 10 and 14	All RCTs meeting above criteria	97
16 limit 15 to all child <0 to 18 years>	Limit to RCTs indexed as including children	22
17 limit 15 to all adult <19 plus years>	Limit to RCTs indexed as including adults	30
18 15 not (16 or 17)	RCTs not indexed by any age group	57
19 16 not 17	RCTs indexed as only including children	10
20 17 not 16	RCTs indexed as only including adults	10
20. 17 not 10 21. $(16 \text{ or } 17) \text{ not } (19 \text{ or } 20)$	RCTs indexed as including both children and	10
21.(10011/)101(190120)	adults	14

*Example shows the search query for the year 2015. This search strategy was repeated for each calendar year from 2011 to 2020, inclusive.

BMJ Open: first published as 10.1136/bmjopen-2024-086934 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmushogeschool . Protected by copyright, including for uses related to text and data mining, Al training, and similar technologies.



Page 37 of 41

1

ge 37 of 41			BMJ Open).1136/bmjopen- cted by copyrigh
Supplementary	Table 2. Paediatric RCTs report Title	ing patient and fa	amily engagement published in <i>The BMJ</i> (Areas of Engagement	Descriptive Summary of
Country Puder, 2011 Switzerland	Effect of multidimensional lifestyle intervention on fitness and adiposity in predominantly migrant preschool children (Ballabeina): cluster randomised controlled trial.	Cardiology	 Preparation Intervention development/refinement 	Patient and Eamily Input "The intervention was developed with input from exercise physiologists, preschool and primary school teachers, paediatrictanse dietitians, psychologists, and variors and keholders including experts for magrant families."
Robling, 2012 United Kingdom	The effect of the Talking Diabetes consulting skills intervention on glycaemic control and quality of life in children with type 1 diabetes: cluster randomised controlled trial (DEPICTED study).	Endocrinology	 Engagement of youth participants Preparation Intervention development/refinement 	In Acknow we gements: "We than we get a principal investigators, members we get ch clinical team, and local UKCRN we get ch staff participating in the 26 trial centres, and to all the practitioners and families who contributed to the development of the Talking Drabetes intervention. We particularly thank the dedicated input of our parent and patient representative CC (co-applicant lin particular during the development shase of the study. Other contributes to programme development students and saff at Whitchurch High School (drame club) "
Kipping, 2014 England	Effect of intervention aimed at increasing physical activity, reducing sedentary behaviour, and increasing fruit and vegetable consumption in children: active for Life Year 5 (AFLY5) school-based cluster randomised controlled trial.	Public health or preventive medicine	 Preparation Intervention development/refinement Execution Intervention delivery Input on study conduct Translation Dissemination and implementation of results 	"We worked with primary school teachers, the local primary care trust (public hollth commissioners), and the local council bovernment) in South Gloucestershipe, in the south west of England, to determine whether this intervention could be adapted for use in the UK, whether delivering the adapted intervention within the National Curriculum was feasible, and whether a pilot randomined controlled trial provide

1				BMJ Open	0.1136/bmjope octed by copyri
$\begin{array}{c} -3\\ 3\\ 4\\ 5\\ 6\\ 7\\ 8\\ 9\\ 10\\ 11\\ 12\\ 13\\ 14\\ 15\\ 16\\ 17\\ 18\\ 19\\ 20\\ 22\\ 23\\ 24\\ 25\\ 26\\ 7\\ 28\\ 9\\ 30\\ 12\\ 33\\ 34\\ 35\\ 36\\ 37\\ 8\\ 9\\ 41\\ 42\end{array}$	Andersson, 2015 Nicaragua and Mexico	Evidence based community mobilization for dengue prevention in Nicaragua and Mexico (Camino Verde, the Green Way): cluster randomized controlled trial.	Infectious diseases and vaccines	 ✓ Engagement of youth participants Preparation Intervention development/refinement Execution 	evidence of promise for the intervention sufficient o justify a full scale trial. Weungertook qualitative work with parents and teachers to develop the intervention in such a way that it involved parents; this showed that child- parent interactive homework would be feasible and maceptable to them. Furthermore our work with teachers and parents classifies howed that a more intensive metric our work with teachers and parents classifies of the study to identify any issues that might impact dissemination. Process evaluations will be undertaken throughout the study to identify any issues that might impact dissemination. Process evaluation will be conducted using focus groups with children, need o-face interviews with teachers and school administrators, and telephone interviews with parents." "Patients whe previously had dengue and their families were intimately involved in design of the intervention. Facilitators convened and ran intervention design groups with & 10 people, usually separatelo formen and women, to discuss survey realits, cost implication, and specific prevention strategies in each community. Patients and their families were also central to dissemination of the baseline information (i.e., assessment of risk of dengue in their community), which helped o motivate community involvement beyond the study."
43 44					LTA

- 45 46
- 47

Pag	ge 39 of 41			BMJ Open	0.1136/b cted by
1 2					mjopen
3 4 5 6 7 8 9 10 11 12 13 14 15 16	Kaufman, 2017 Australia	Faster clean catch urine collection (Quick-Wee method) from infants: randomised controlled trial.	Urology	 Preparation Development of the research question Selection of outcomes Intervention development/refinement Execution Intervention delivery 	"Feedback from the parents and carers of 40 particinants in the preceding pilot study contaiting of the study research question and gutcome measures. Parents and carers were asked to rate their satisfaction with the intervention and could provide additional comments. Parental satisfaction with the intervention in the pilot gudy was high and no responders were dissatisfied with the intervention additional feasibility for this lager definitive trial."
 17 18 19 20 21 22 23 24 25 26 27 28 29 30 31 32 33 	Santer, 2018 England, Wales	Emollient bath additives for the treatment of childhood eczema (BATHE): multicentre pragmatic parallel group randomised controlled trial of clinical and cost effectiveness.	Dermatology	 Preparation Intervention development/refinement Execution Input on study design Recruitment of participants Translation Dissemination and implementation of results 	"The trial aggement group included an experienced patient and public involvement (PPI) co-applicant (AR) who participated in all phases of the trial design, including planning recruitment and recruitment materials. We also consulted members of the Centre of Evidence based Dermatology patient panel at the trial design stage, and we sought additional PPI representation when plasming how to disseminate findings. The independent trial steering committee induded a PPI member. The results will be emailed to all trial participates and published on the trial website."
34 35 36 37 38 39 40 41	Adab, 2018 United Kingdom	Effectiveness of a childhood obesity prevention programme delivered through schools, targeting 6- and 7-year-olds: cluster randomised controlled trial (WAVES study).	Public health or preventive medicine	 Preparation Selection of outcomes Intervention development/refinement Execution Input on study design Recruitment of participants 	"Public in volument was a key feature of the early phases of trial development and feasibility testing before this main trial. Intervention development was informed by detailed consultation with parents, teachers, and other school staff. The intervention was further refined and the
41 42 43 44 45 46 47		Fo	r peer review only -	http://bmjopen.bmj.com/site/about/guidelines.	EZ-LTA xhtml

.ong term tapering versus	0,		process for measuring outcomes tested and adapted by asking the children, parents, and teachers about their experiences during the feasibility study. Measures of gellbeing and body dissatisfaction; were included as outcomes based on meinsperceived importance among schones staff. Our research team includes and ducation advisor at the Health Education Service, who has regular comfact with schools and advised
.ong term tapering versus	0,-		process for masuring outcomes tested and adapted by asking the children, parents, and touchers about their experiences during the feasibility study. Measures of gellbeing and body dissatisfaction, were included as outcomes based on meisperceived importance among schools staff. Our research team includes and toucation advisor at the Health Eduration Service, who has regular conflict with schools and advised
tandard prednisolone reatment for first episode of hildhood nephrotic yndrome: phase III andomised controlled trial nd economic evaluation.	Nephrology	 Execution Input on study design Input on study conduct Results interpretation Translation Dissemination and implementation of results 	on school the participant recruitment." "The trial to be cold was reviewed by representation of the UK Nephrotic Syndrome IT est (NeST) and the UK Renal Patient Support Group, who provided saluable input about trial design, a ceptibility of trial visit frequency, and adverse event monitoring. A NeST representative participated on the trial specing committee. After publication, the trial results will be disseminated to all study collaborators. The plain English summary of the study results will be sent to the participants and/or their perents through their responsible clinician. The summary will also be available on the NeST website and the Patent NOS study website (www.birghingham.ac.uk/prednos)."
nultiple daily injection egimens in children and oung people at diagnosis of	Endocrinology	 Engagement of youth participants Preparation Selection of outcomes Execution Input on study design 	interpretation over undertaken in close discussion with patients and their families. Young people were consulted on the design of the study including
Con nul egiou	anood nephrotic drome: phase III domised controlled trial economic evaluation.	Intinuous subcutaneous alin infusion versus lin infusion versus lini nchildren and ing people at diagnosis of e 1 diabetes: pragmatic	Idiood nephrotic . Results interpretation drome: phase III . Dissemination and implementation of results economic evaluation. . Dissemination and implementation of results ntinuous subcutaneous . Endocrinology lin infusion versus . Endocrinology titple daily injection . Selection of outcomes imens in children and . Selection ng people at diagnosis of . Input on study design e I diabetes: pragmatic . Recruitment of participants

For peer review only - http://bmjopen.bmj.com/site/about/guidelines.xhtml

46

44

45

Pag	ge 41 of 41			BMJ Open	0.1136/br	
1 2 3 4 5 6 7 8 9 10 11		randomised controlled trial and economic evaluation.		 Intervention delivery Input on study conduct Results interpretation Translation Dissemination and implementation of results 	measures and study materials. Parents o children and young people with type 1 diabetes were members of the trial management committee and trial steering committee and advised on recruitment strategy. Study results and their significance to patients and their families were discussed in detail with parent contributed?	f
12 13 14 15 16 17 18 20 21 22 23 24 25 26 27 28 29 30 31 23 34 35 36 37 38 39 40 41	Roberts, 2020 Guinea-Bissau	Effects of food supplementation on cognitive function, cerebral blood flow, and nutritional status in young children at risk of undernutrition: randomized controlled trial.	Neurology	 Preparation Intervention development/refinement Execution Input on study design Intervention delivery 	"Participate and public involvement Village me tings were held to obtain community devel support for village enrollment, and additional discussions involved community members in study planning. Community health workers recommended the specific control breakfast recommended the plan for all children to receive their supplement at one of the three separate feeding centers within the villages, and also asked for five days gack week of supplementation to balance their other responsibilities. Community health workers and parents were additionally consulted about ingredients and preparation of NEWSUP at the end of the earlier pilot; based on their request we reduced the amount of moringa in the NEWSUP recipe and changed the production from a baked good to a aw cormula (to prevent burning and improve taste), and implemented gradual increase in consumption guring the first study week to allow children to become accustomed to the taste."	
43 44 45 46		Fo	r peer review only -	http://bmjopen.bmj.com/site/about/guidelines	L T A State	

	BMJ Open		0.1136/bmjop cted by copy	
Supplementary Table 3. Quality assessment of PE+ and PE- pa	ediatric RCTs in 7	The BMJ, 2011-2020	oen-2024- right, inc	
Criteria / Risk of bias	Total	PE +	P#d: - 086	<i>p</i> -value ²
n	45	10	934 Mag	
Q1. Random Sequence Generation – selection bias			on	
Low risk of bias	s 43 (95.6)	9 (90.0)	34 (@ 7. F)	
Unclea	r 1 (2.2)	0 (0.0)	1 (36.9)	0.40
High risk of bia:	s 1 (2.2)	1 (10.0)		
Q2. Allocation Concealment – selection bias			025 ;mu d to	
Low risk of bias	s 37 (82.2)	9 (90.0)	28 (3)	
Unclea	r 4 (8.9)	0 (0.0)		0.80
High risk of bias	s 4 (8.9)	1 (10.0)	3 (8 5)8	
Q3. Selective Reporting – reporting bias			ata	
Low risk of bia	s 12 (26.7)	2 (20.0)	10 (2 8.G	
Unclea	r 31 (68.9)	7 (70.0)	24 (3 8.6)	0.54
High risk of bia	s 2 (4.4)	1 (10.0)	1 (2.9)	-
Q4. Other Bias – bias not covered elsewhere			(bm trai	
Low risk of bia	s 31 (68.9)	7 (70.0)	24 (58.6	
Unclea	r 5 (11.1)	1 (10.0)	4 (b 1.4 p	1.00
High risk of bia	s 9 (20.0)	2 (20.0)	7 (20.0.	1
Q5. Blinding of Participants and Personnel – performance bias	6		imi on	
Low risk of bia	s 22 (48.9)	4 (40.0)	18 (51.45	
Unclea	r 5 (11.1)	1 (10.0)	<u>ع</u> 4 (ق اً.4	0.87
High risk of bia	s 18 (40.0)	5 (50.0)	13 (d 7. h	
Q6. Blinding of Outcome Assessment – detection bias			7, 2 9gie	
Low risk of bia	s 31 (68.9)	6 (60.0)	25 (71.4)	
Unclea	r 9 (20.0)	3 (30.0)	6 (17.1)	0.74
High risk of bia	s 5 (11.1)	1 (10.0)	4 (11.4 £	-
			artm	
			lent	
			GE	
	1		Ż-LT	

	орен		136/bmjo d by copy	
07 Incomplete Outcome Data – attrition bias			pen-20 /right,	
Low risk of bia	s 31 (68.9)	6 (60.0)	25 (21.43)	
Unclea	r 13 (28.9)	4 (40.0)	9 (\$5.78	0.5
High risk of bia	s 1 (2.2)	0 (0.0)	1 (2 .9)	-
Overall Quality Rating			r us	
GOOD or FAII	R 17 (37.8)	4 (40.0)	13 (87.13	1.0
POOL	R 28 (62.2)	6 (60.0)	22 (g 1 9]	1.0
			ded from http ool . lata mining, /	