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

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Short title: Patient engagement, quality and dissemination of paediatric randomized controlled trials

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ABSTRACT

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

family engagement in the trial, however, was associated with higher metrics for social media attention, compared with trials with no engagement.

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.

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.

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 Meta-analyses (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;

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 (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 did not report 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

'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 non-academic 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 (https://plumanalytics.com/learn/about-metrics/) and altmetric data from Web of Science (https://www.altmetric.com/research-access/) 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. 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**).

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 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	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**2020**	**2011-2020**
Published RCTs	11	10	4	6	6	0	1	3	3	1	45
PE+	*1*	*1*	*0*	*1*	*1*	*0*	*1*	*2*	*2*	*1*	*10*
PPI paragraph, yes	0	0	0	0	1	0	1	2	2	1	7
PPI paragraph, no	1	1	0	1	0	0	0	0	0	0	3
PE -	*10*	*9*	*4*	*5*	*5*	*0*	*0*	*1*	*1*	*0*	*35*
PPI paragraph, yes	0	0	0	0	1	0	0	1	1	0	3
PPI paragraph, no	10	9	4	5	4	0	0	0	0	0	32

As described in **Figure 1** and **Supplementary Table 2**, among the 10 PE+ trials, patients and families were engaged across the entire research process, and often involved in more than one aspect of the study. For example, of the 10 PE+ RCTs, 8/10 (80%) had patient and family input on the development of the intervention, and 5/10 (50%) had input on the dissemination of trial results. Only one PE+ trial engaged patients and families in developing the research question. The three PE+ RCTs that included participants 12 to 17 years of age, included engagement of youth partners.[32,48,59] Information on the number of patients and/or family engaged was provided by only one PE+ trial; in that trial parents and carers 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). 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.

Table 2. Characteristics of PE+ and PE- paediatric RCTs published in *The BMJ*, 2011-2020[1]

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)

[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.

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 PE- trials 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).

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 -**	***p*-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 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 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 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. 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. *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 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.

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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.

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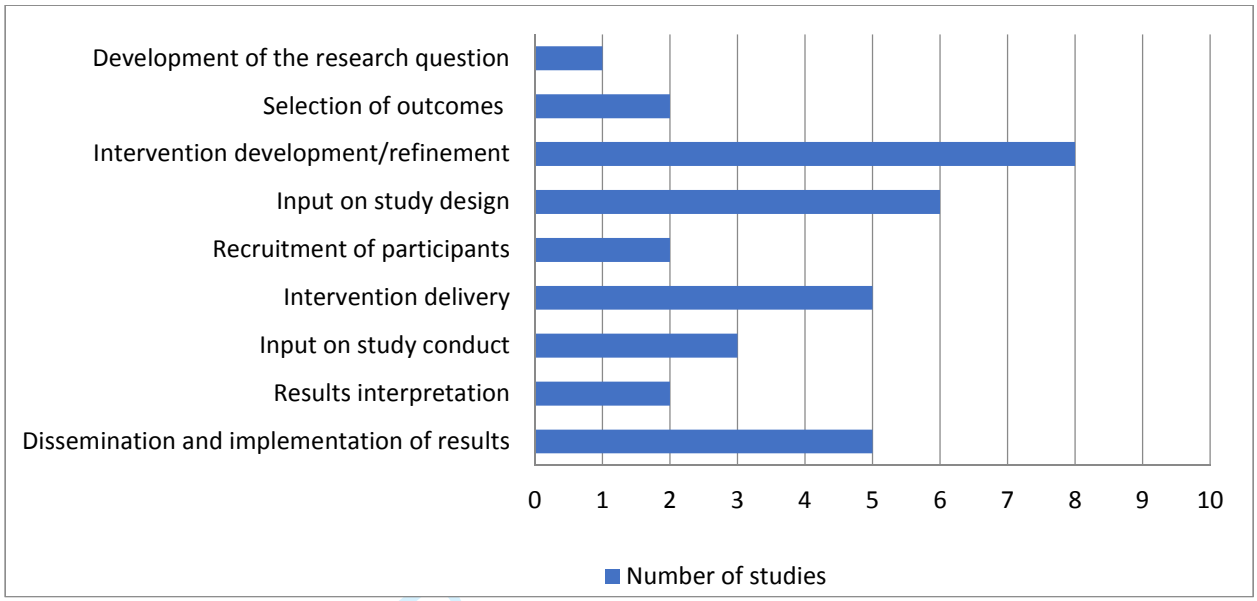
Figure Legend

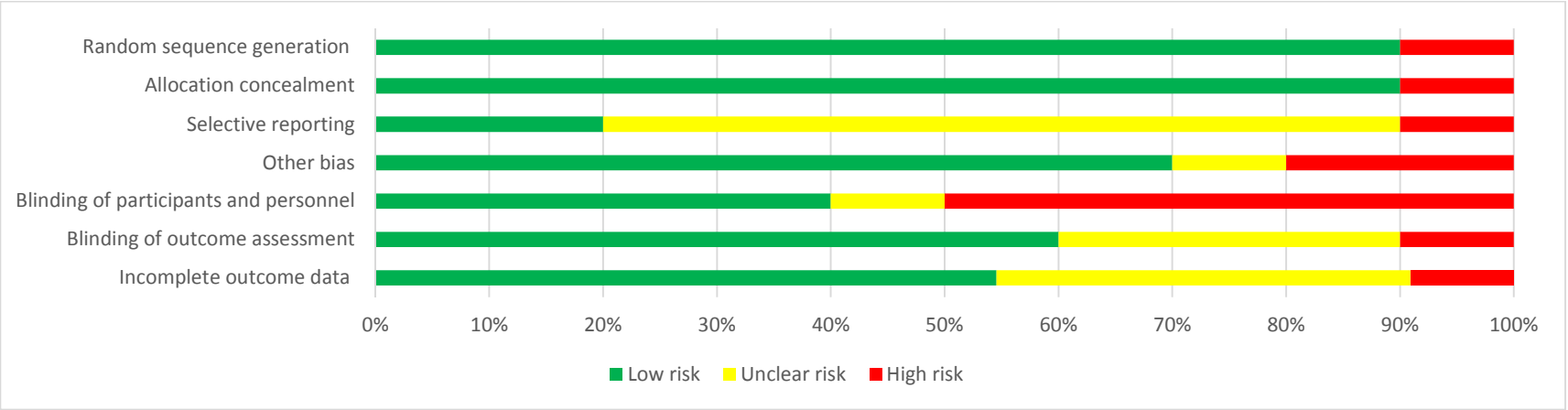
Figure 1. Areas of patient and family engagement in PE+ paediatric trials published in *The BMJ*[1]

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

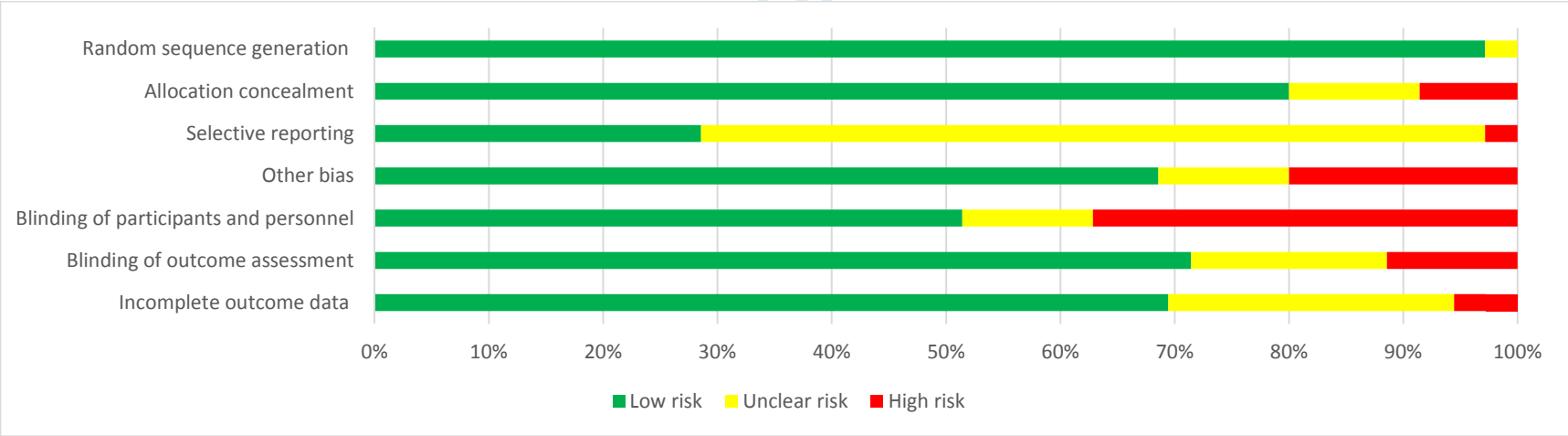


[1]Patients and families could be engaged in more than one aspect of the research process in a single study

PE+ RCTs



PE- RCTs

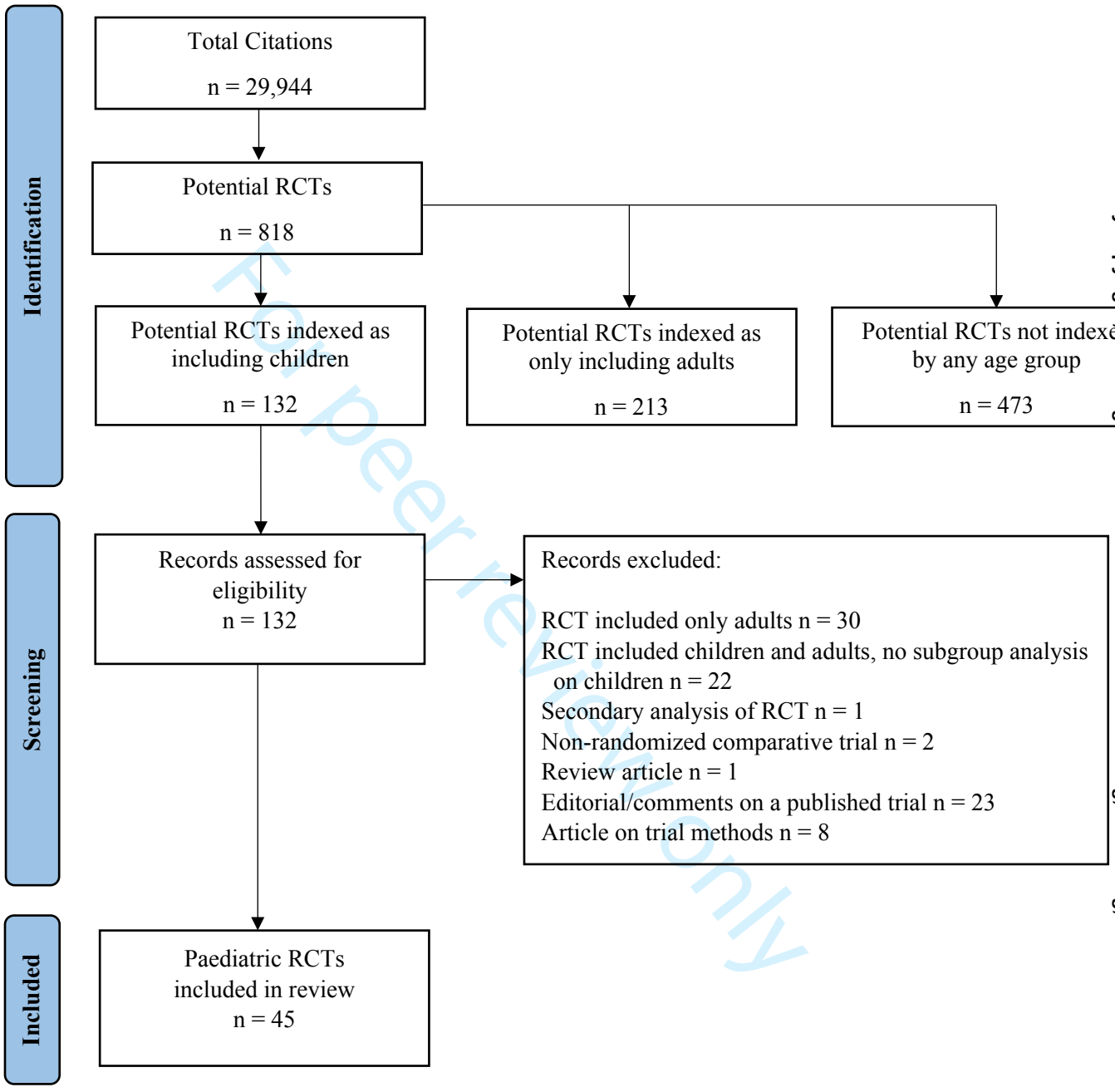


Supplementary Table 1. Ovid MEDLINE search strategy to identify indexed pediatric randomized controlled trials (RCTs) published in *The BMJ*

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 (Steps 1 to 10)	200252
6. randomly.ab.		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 Journal).jn	Limit by BMJ	182994
12. limit 11 to yr="2015"	Limit by year*	3335
13. limit 12 to (address or comment or editorial or letter or observational study or meta-analysis or review or systematic review)	Filter out addresses, comments, editorials, letters, observational studies, meta-analyses, reviews, and systematic reviews	1214
14. 12 not 13		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 adults	12

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

Supplementary Figure 1. Literature search



Supplementary Table 2. Paediatric RCTs reporting patient and family engagement published in *The BMJ* (2011-2020)

Author, year Country	Title	Clinical Category	Areas of Engagement
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
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 • Intervention development/refinement
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 development/refinement • Intervention delivery • Input on study conduct • Dissemination and implementation of results
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 design • Intervention delivery • Dissemination and implementation of results
Kaufman, 2017 Australia	Faster clean catch urine collection (Quick-Wee method) from infants: randomised controlled trial.	Urology	• Development of the research question • Selection of outcomes • Intervention development/refinement • Intervention delivery
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 design • Recruitment of participants • Dissemination and implementation of results
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

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	• Input on study design • Input on study conduct • Results interpretation • Dissemination and implementation of results
Blair, 2019 England, Wales	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 • Selection of outcomes • Input on study design • Recruitment of participants • Intervention delivery • Input on study conduct • Results interpretation • Dissemination and implementation of results
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	• Intervention development/refinement • Input on study design • Intervention delivery

Supplementary Table 3. Quality assessment of PE+ and PE- paediatric RCTs in *The BMJ*, 2011-2020[1]

Criteria / Risk of bias	Total	PE +	PE -	*p*-value[2]
n	**45**	**10**	**35**	
Q1. Random Sequence Generation – selection bias				
Low risk of bias	43 (95.6)	9 (90.0)	34 (97.1)	0.40
Unclear	1 (2.2)	0 (0.0)	1 (2.9)	
High risk of bias	1 (2.2)	1 (10.0)	0 (0.0)	
Q2. Allocation Concealment – selection bias				
Low risk of bias	37 (82.2)	9 (90.0)	28 (80.0)	0.80
Unclear	4 (8.9)	0 (0.0)	4 (11.4)	
High risk of bias	4 (8.9)	1 (10.0)	3 (8.6)	
Q3. Selective Reporting – reporting bias				
Low risk of bias	12 (26.7)	2 (20.0)	10 (28.6)	0.54
Unclear	31 (68.9)	7 (70.0)	24 (68.6)	
High risk of bias	2 (4.4)	1 (10.0)	1 (2.9)	
Q4. Other Bias – bias not covered elsewhere				
Low risk of bias	31 (68.9)	7 (70.0)	24 (68.6)	1.00
Unclear	5 (11.1)	1 (10.0)	4 (11.4)	
High risk of bias	9 (20.0)	2 (20.0)	7 (20.0)	
Q5. Blinding of Participants and Personnel – performance bias				
Low risk of bias	22 (48.9)	4 (40.0)	18 (51.4)	0.87
Unclear	5 (11.1)	1 (10.0)	4 (11.4)	
High risk of bias	18 (40.0)	5 (50.0)	13 (37.1)	
Q6. Blinding of Outcome Assessment – detection bias				
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.4)	0.74
Unclear	9 (20.0)	3 (30.0)	6 (17.1)	
High risk of bias	5 (11.1)	1 (10.0)	4 (11.4)	

Q7. Incomplete Outcome Data – attrition bias				
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.4)	0.57
Unclear	13 (28.9)	4 (40.0)	9 (25.7)	
High risk of bias	1 (2.2)	0 (0.0)	1 (2.9)	
Overall Quality Rating				
GOOD or FAIR	17 (37.8)	4 (40.0)	13 (37.1)	1.00
POOR	28 (62.2)	6 (60.0)	22 (62.9)	

[1] Values are shown as n (%).
[2] PE+ and PE- groups were compared using Fisher's Exact test.

BMJ Open

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

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

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

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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

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.

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.
- 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 research quality and dissemination has not been examined.

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 Meta-analyses (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;

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 did not report 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

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 non-academic 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 (https://plumanalytics.com/learn/about-metrics/) and altmetric data from Web of Science (https://www.altmetric.com/research-access/) 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, co-author) 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 yes	**PPI paragraph no**	**PPI paragraph yes**	**PPI paragraph 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

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.

Table 2. Characteristics of PE+ and PE- paediatric RCTs published in *The BMJ*, 2011-2020[1]

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)

[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.

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 PE- trials 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 -**	***p*-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 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.

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.

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 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.

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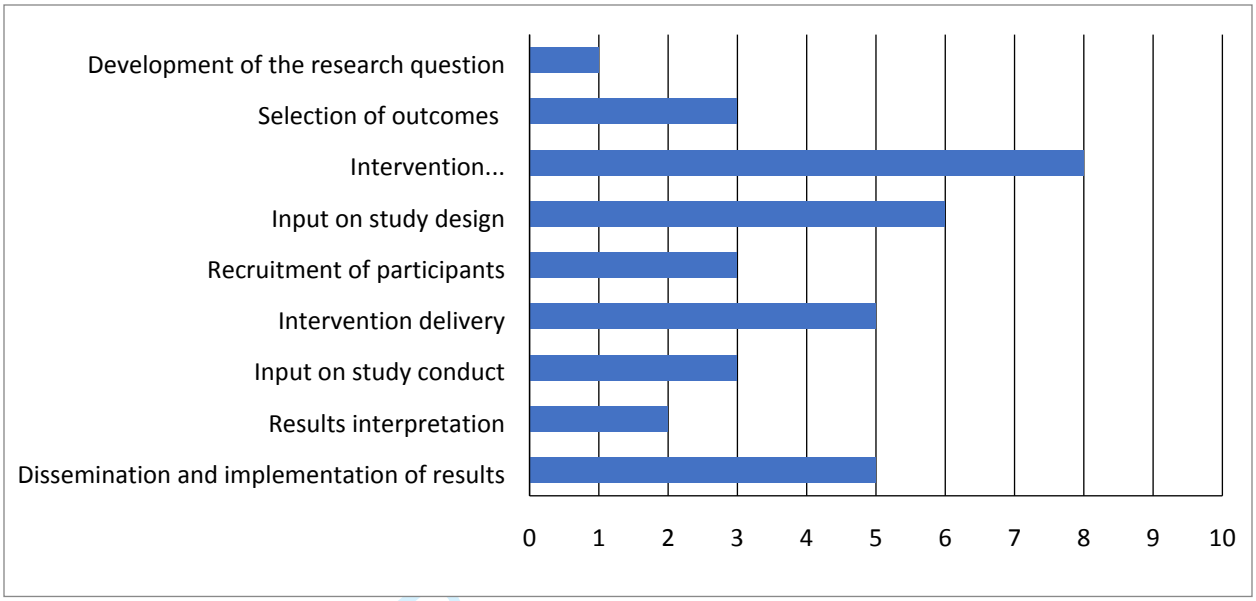
Figure Legend

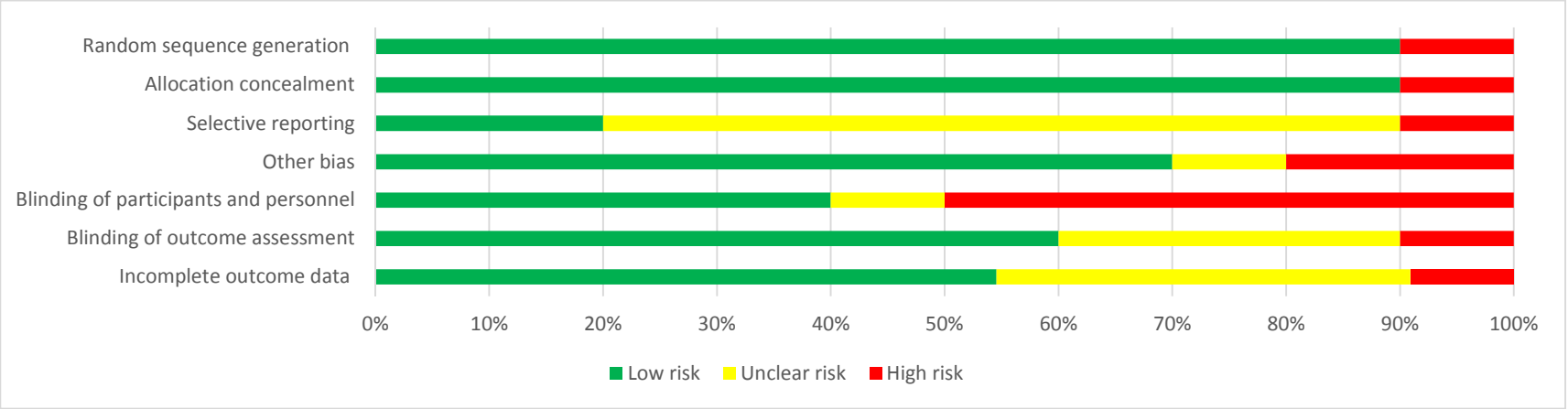
Figure 1. Areas of patient and family engagement in PE+ paediatric trials published in *The BMJ*

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

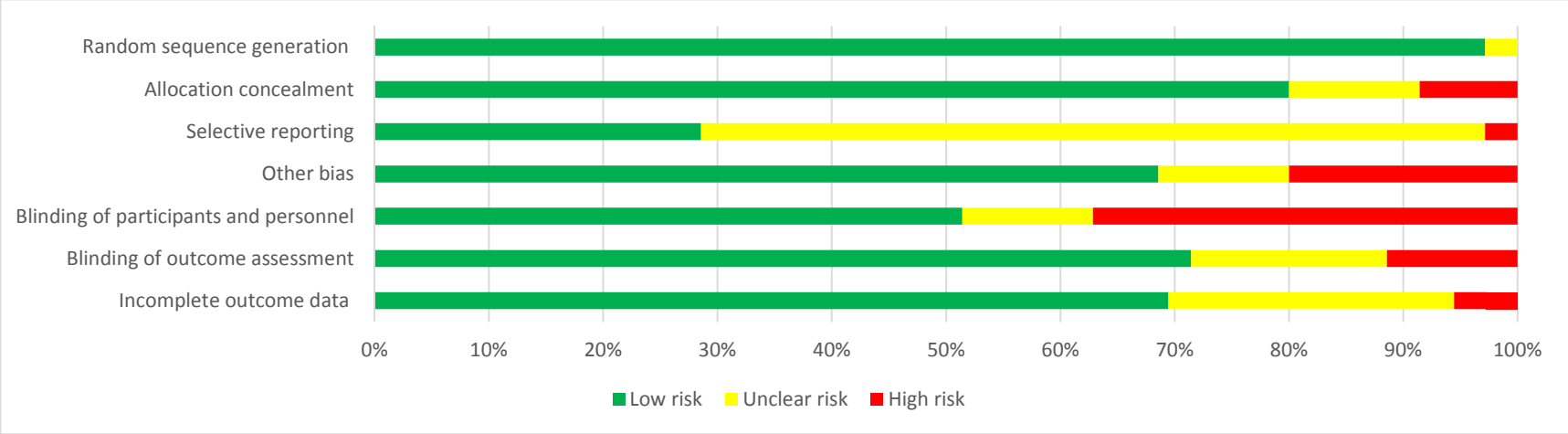


[1]Patients and families could be engaged in more than one aspect of the research process in a single study

PE+ RCTs



PE- RCTs

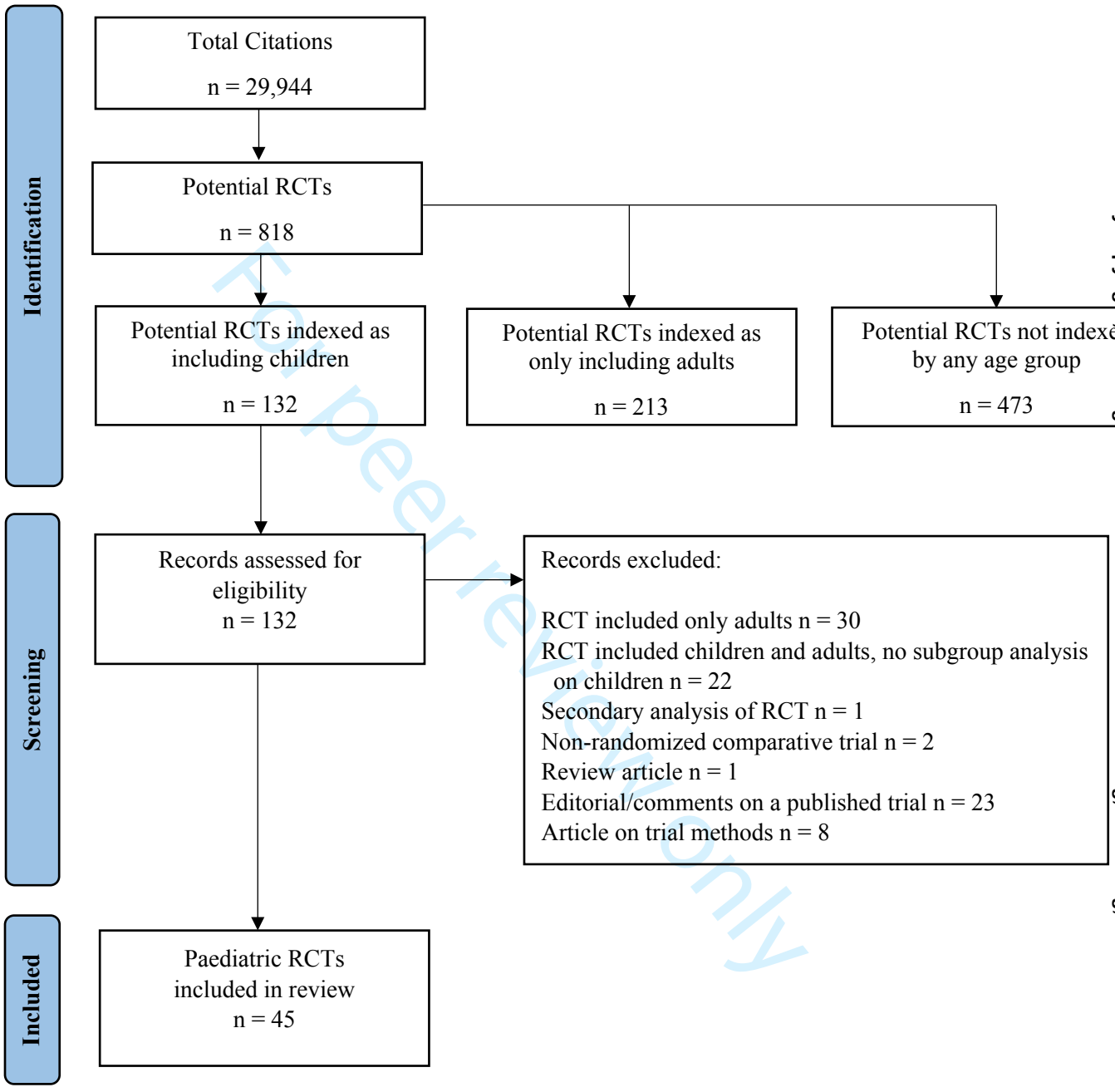


Supplementary Table 1. Ovid MEDLINE search strategy to identify indexed pediatric randomized controlled trials (RCTs) published in *The BMJ*

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 (Steps 1 to 10)	200252
6. randomly.ab.		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 Journal).jn	Limit by BMJ	182994
12. limit 11 to yr="2015"	Limit by year*	3335
13. limit 12 to (address or comment or editorial or letter or observational study or meta-analysis or review or systematic review)	Filter out addresses, comments, editorials, letters, observational studies, meta-analyses, reviews, and systematic reviews	1214
14. 12 not 13		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 adults	12

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

Supplementary Figure 1. Literature search



Supplementary Table 2. Paediatric RCTs reporting patient and family engagement published in *The BMJ* (2011-2020)

Author, year Country	Title	Clinical Category	Areas of Engagement	Descriptive Summary of Patient and Family Input
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, paediatricians, dietitians, psychologists, and various stakeholders including experts for migrant 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 Acknowledgements: "We thank local principal investigators, members of 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 Diabetes intervention. We particularly thank the dedicated input of our parent and patient representative CC (co-applicant) in particular during the development phase of the study. Other contributors to programme development: students and staff at Whitchurch High School (drama 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 health commissioners), and the local council (government) in South Gloucestershire, 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 randomised controlled trial provided

				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 in such a way that it involved parents; this showed that child-parent interactive homework would be feasible and acceptable to them. Furthermore, our work with teachers and parents clearly showed that a more intensive intervention in school …would not have been acceptable." The study protocol included: "Audits and 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, face-to-face interviews with teachers and school administrators, and telephone interviews with parents."
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** • Input on study design • Intervention delivery **Translation** • Dissemination and implementation of results	"Patients who previously had dengue and their families were intimately involved in design of the intervention. Facilitators convened and ran intervention design groups with 8-10 people, usually separately for men and women, to discuss survey results, 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 to motivate community involvement beyond the study."

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 participants 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 satisfaction with the intervention in the pilot study was high and no respondents were dissatisfied with the intervention, so demonstrating feasibility for this larger definitive trial."
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 (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 planning 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."
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 involvement 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

10.1136/bmjopen-2024-086994 on 12 March 2025. Downloaded from http://bmjopen.bmj.com/ on June 7, 2025 at Department GEZ-LTA Erasmus Hogeschool.

				process for measuring outcomes tested and adapted by asking the children, parents, and teachers about their experiences during the feasibility study. Measures of wellbeing and body dissatisfaction were included as outcomes based on their perceived importance among school staff. Our research team includes an education advisor at the Health Education Service, who has regular contact with schools and advised on school and participant recruitment."
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	"The trial protocol was reviewed by representatives of the UK Nephrotic Syndrome Trust (NeST) and the UK Renal Patient Support Group, who provided valuable input about trial design, acceptability of trial visit frequency and adverse event monitoring. A NeST representative participated on the trial steering 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 parents through their responsible clinician. The summary will also be available on the NeST website and the PREDNOS study website (www.birmingham.ac.uk/prednos)."
Blair, 2019 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	"Study design, delivery, and data interpretation were undertaken in close discussion with patients and their families. Young people were consulted on the design of the study including impact of participation, outcome

	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 of 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 contributors."
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	"Participant and public involvement Village meetings were held to obtain community level 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 each 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 raw formula (to prevent burning and improve taste), and implemented a gradual increase in consumption during the first study week to allow children to become accustomed to the taste."

Supplementary Table 3. Quality assessment of PE+ and PE- paediatric RCTs in *The BMJ*, 2011-2020[1]

Criteria / Risk of bias	Total	PE +	PE -	*p*-value[2]
n	**45**	**10**	**35**	
Q1. Random Sequence Generation – selection bias				
Low risk of bias	43 (95.6)	9 (90.0)	34 (97.1)	0.40
Unclear	1 (2.2)	0 (0.0)	1 (2.9)	
High risk of bias	1 (2.2)	1 (10.0)	0 (0.0)	
Q2. Allocation Concealment – selection bias				
Low risk of bias	37 (82.2)	9 (90.0)	28 (80.0)	0.80
Unclear	4 (8.9)	0 (0.0)	4 (11.4)	
High risk of bias	4 (8.9)	1 (10.0)	3 (8.6)	
Q3. Selective Reporting – reporting bias				
Low risk of bias	12 (26.7)	2 (20.0)	10 (28.6)	0.54
Unclear	31 (68.9)	7 (70.0)	24 (68.6)	
High risk of bias	2 (4.4)	1 (10.0)	1 (2.9)	
Q4. Other Bias – bias not covered elsewhere				
Low risk of bias	31 (68.9)	7 (70.0)	24 (68.6)	1.00
Unclear	5 (11.1)	1 (10.0)	4 (11.4)	
High risk of bias	9 (20.0)	2 (20.0)	7 (20.0)	
Q5. Blinding of Participants and Personnel – performance bias				
Low risk of bias	22 (48.9)	4 (40.0)	18 (51.4)	0.87
Unclear	5 (11.1)	1 (10.0)	4 (11.4)	
High risk of bias	18 (40.0)	5 (50.0)	13 (37.1)	
Q6. Blinding of Outcome Assessment – detection bias				
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.4)	0.74
Unclear	9 (20.0)	3 (30.0)	6 (17.1)	
High risk of bias	5 (11.1)	1 (10.0)	4 (11.4)	

Q7. Incomplete Outcome Data – attrition bias				
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.4)	0.57
Unclear	13 (28.9)	4 (40.0)	9 (25.7)	
High risk of bias	1 (2.2)	0 (0.0)	1 (2.9)	
Overall Quality Rating				
GOOD or FAIR	17 (37.8)	4 (40.0)	13 (37.1)	1.00
POOR	28 (62.2)	6 (60.0)	22 (62.9)	

[1] Values are shown as n (%).
[2] PE+ and PE- groups were compared using Fisher's Exact test.

BMJ Open

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

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Keywords:	PAEDIATRICS, Child, Social Media, Randomized Controlled Trial

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

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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

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.

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.

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 research quality and dissemination has not been examined.

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 Meta-analyses (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

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 did not report 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 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 non-academic 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 (https://plumanalytics.com/learn/about-metrics/) and altmetric data from Web of Science (https://www.altmetric.com/research-access/) 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, co-author) 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 yes	**PPI paragraph no**	**PPI paragraph yes**	**PPI paragraph 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

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.

Table 2. Characteristics of PE+ and PE- paediatric RCTs published in *The BMJ*, 2011-2020[1]

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)

[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.

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 PE- trials 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 -**	***p*-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 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. 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.

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 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.

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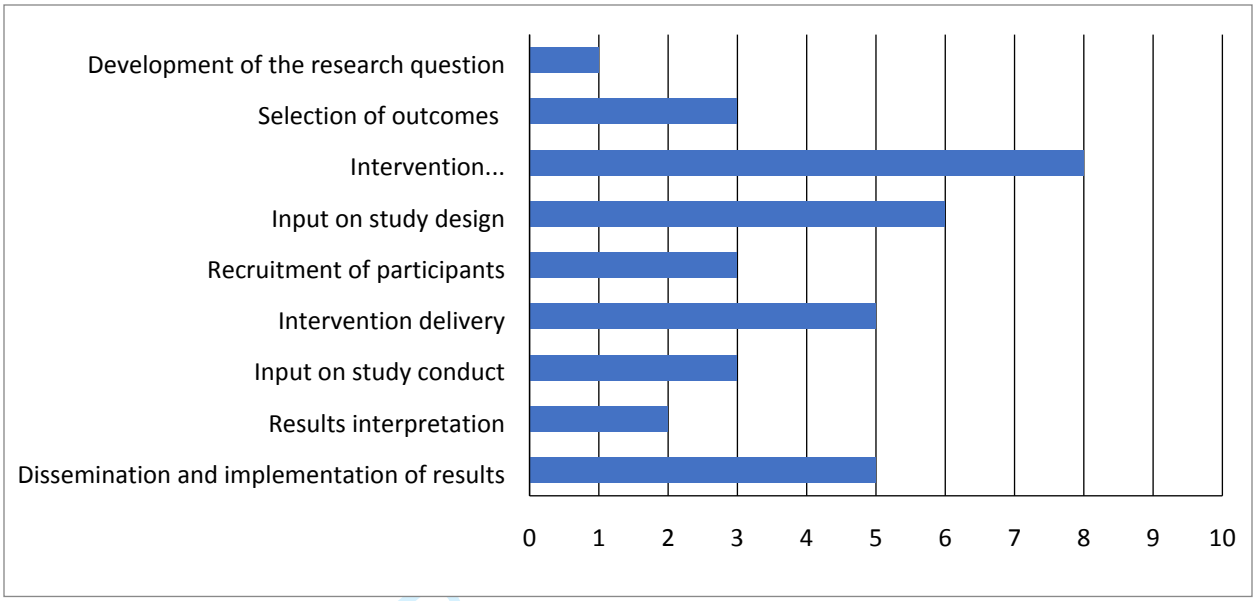
Figure Legend

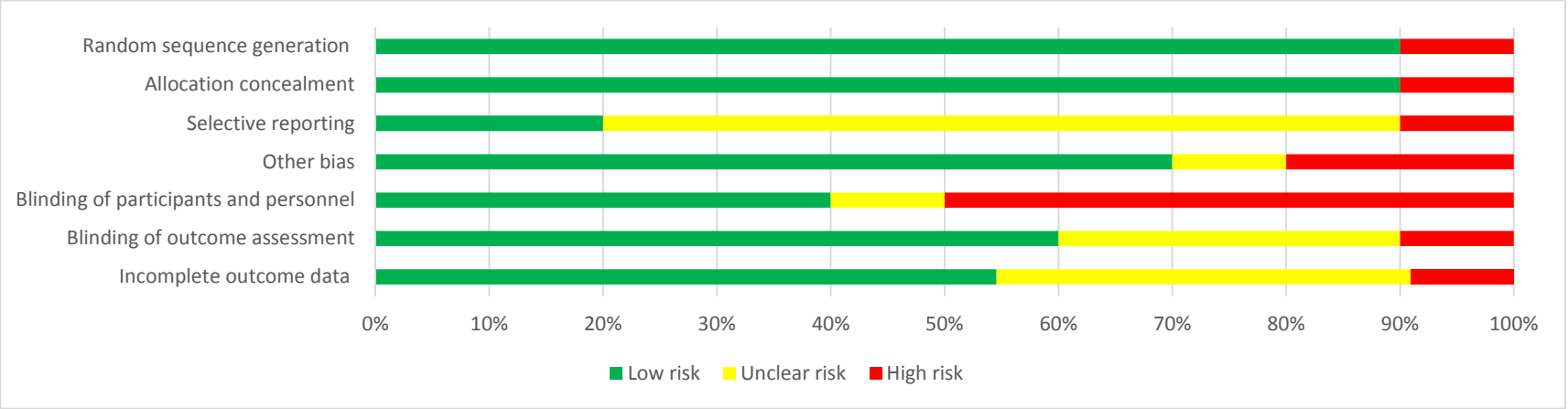
Figure 1. Areas of patient and family engagement in PE+ paediatric trials published in *The BMJ*

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

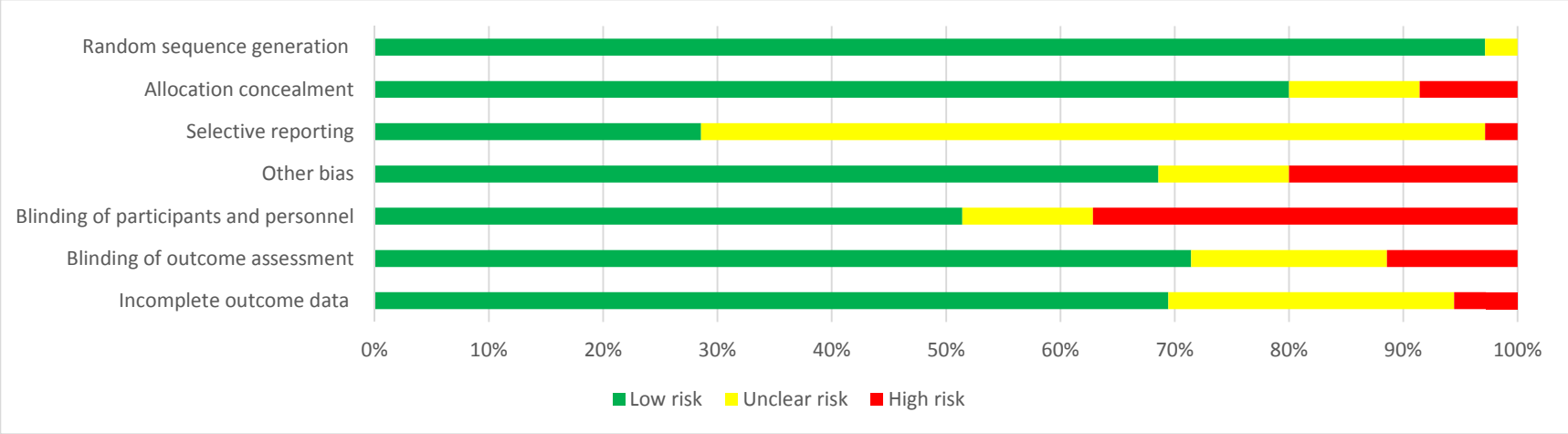


[1]Patients and families could be engaged in more than one aspect of the research process in a single study

PE+ RCTs



PE- RCTs

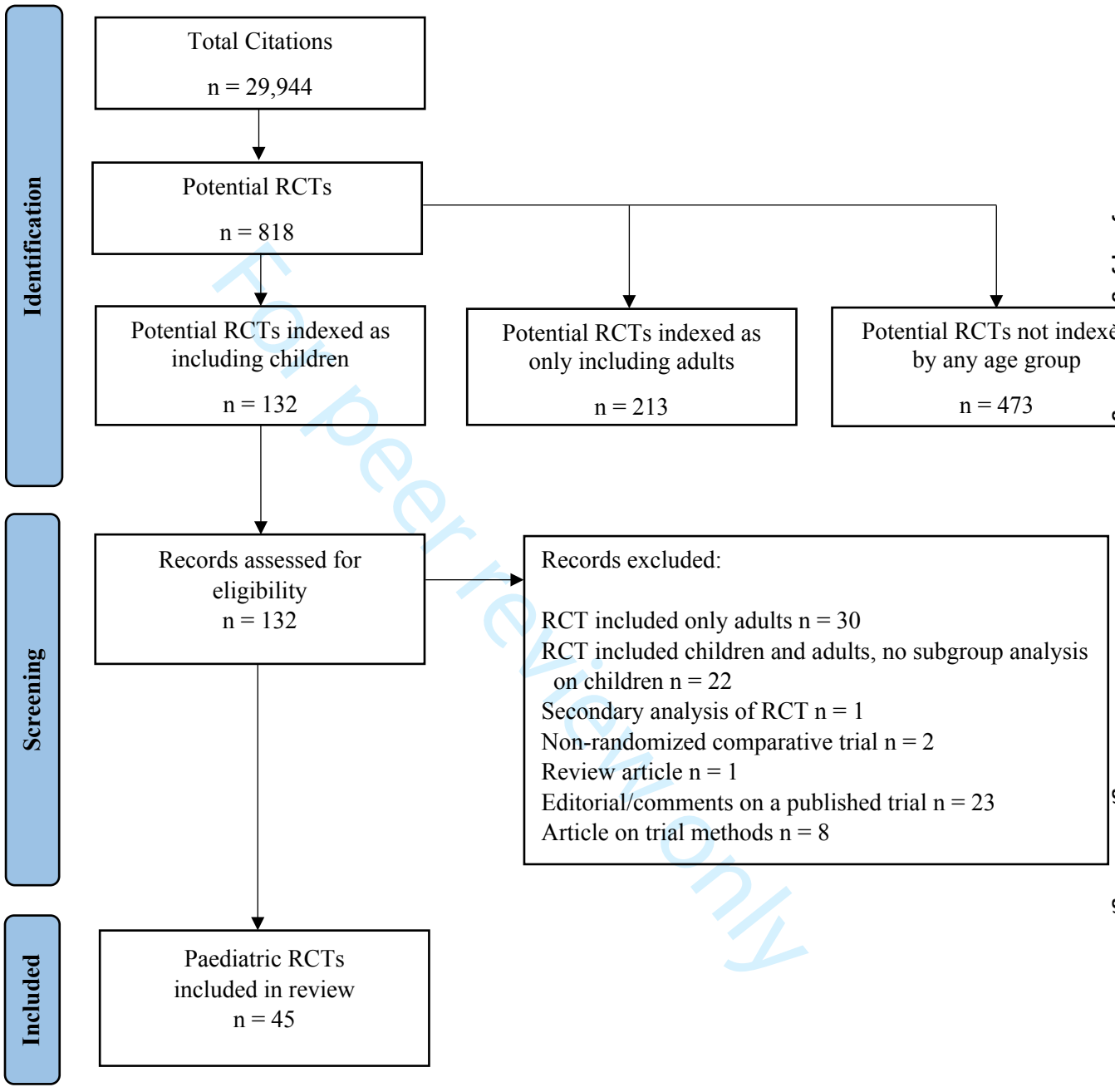


Supplementary Table 1. Ovid MEDLINE search strategy to identify indexed pediatric randomized controlled trials (RCTs) published in *The BMJ*

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 (Steps 1 to 10)	200252
6. randomly.ab.		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 Journal).jn	Limit by BMJ	182994
12. limit 11 to yr="2015"	Limit by year*	3335
13. limit 12 to (address or comment or editorial or letter or observational study or meta-analysis or review or systematic review)	Filter out addresses, comments, editorials, letters, observational studies, meta-analyses, reviews, and systematic reviews	1214
14. 12 not 13		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 adults	12

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

Supplementary Figure 1. Literature search



Supplementary Table 2. Paediatric RCTs reporting patient and family engagement published in *The BMJ* (2011-2020)

Author, year Country	Title	Clinical Category	Areas of Engagement	Descriptive Summary of Patient and Family Input
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, paediatricians, dietitians, psychologists, and various stakeholders including experts for migrant 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 Acknowledgements: "We thank local principal investigators, members of 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 Diabetes intervention. We particularly thank the dedicated input of our parent and patient representative CC (co-applicant) in particular during the development phase of the study. Other contributors to programme development: students and staff at Whitchurch High School (drama 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 health commissioners), and the local council (government) in South Gloucestershire, 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 randomised controlled trial provided

				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 in such a way that it involved parents; this showed that child-parent interactive homework would be feasible and acceptable to them. Furthermore, our work with teachers and parents clearly showed that a more intensive intervention in school …would not have been acceptable." The study protocol included: "Audits and 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, face-to-face interviews with teachers and school administrators, and telephone interviews with parents."
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** • Input on study design • Intervention delivery **Translation** • Dissemination and implementation of results	"Patients who previously had dengue and their families were intimately involved in design of the intervention. Facilitators convened and ran intervention design groups with 8–10 people, usually separately for men and women, to discuss survey results, 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 to motivate community involvement beyond the study."

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 participants 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 satisfaction with the intervention in the pilot study was high and no respondents were dissatisfied with the intervention, so demonstrating feasibility for this larger definitive trial."
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 (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 planning 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."
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 involvement 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

				process for measuring outcomes tested and adapted by asking the children, parents, and teachers about their experiences during the feasibility study. Measures of wellbeing and body dissatisfaction were included as outcomes based on their perceived importance among school staff. Our research team includes an education advisor at the Health Education Service, who has regular contact with schools and advised on school and participant recruitment."
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	"The trial protocol was reviewed by representatives of the UK Nephrotic Syndrome Trust (NeST) and the UK Renal Patient Support Group, who provided valuable input about trial design, acceptability of trial visit frequency and adverse event monitoring. A NeST representative participated on the trial steering 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 parents through their responsible clinician. The summary will also be available on the NeST website and the PREDNOS study website (www.birmingham.ac.uk/prednos)."
Blair, 2019 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	"Study design, delivery, and data interpretation were undertaken in close discussion with patients and their families. Young people were consulted on the design of the study including impact of participation, outcome

	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 of 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 contributors."
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	"Participant and public involvement Village meetings were held to obtain community level 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 each 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 raw formula (to prevent burning and improve taste), and implemented a gradual increase in consumption during the first study week to allow children to become accustomed to the taste."

Supplementary Table 3. Quality assessment of PE+ and PE- paediatric RCTs in *The BMJ*, 2011-2020[1]

Criteria / Risk of bias	Total	PE +	PE -	*p*-value[2]
n	**45**	**10**	**35**	
Q1. Random Sequence Generation – selection bias				
Low risk of bias	43 (95.6)	9 (90.0)	34 (97.1)	0.40
Unclear	1 (2.2)	0 (0.0)	1 (2.9)	
High risk of bias	1 (2.2)	1 (10.0)	0 (0.0)	
Q2. Allocation Concealment – selection bias				
Low risk of bias	37 (82.2)	9 (90.0)	28 (80.0)	0.80
Unclear	4 (8.9)	0 (0.0)	4 (11.4)	
High risk of bias	4 (8.9)	1 (10.0)	3 (8.6)	
Q3. Selective Reporting – reporting bias				
Low risk of bias	12 (26.7)	2 (20.0)	10 (28.6)	0.54
Unclear	31 (68.9)	7 (70.0)	24 (68.6)	
High risk of bias	2 (4.4)	1 (10.0)	1 (2.9)	
Q4. Other Bias – bias not covered elsewhere				
Low risk of bias	31 (68.9)	7 (70.0)	24 (68.6)	1.00
Unclear	5 (11.1)	1 (10.0)	4 (11.4)	
High risk of bias	9 (20.0)	2 (20.0)	7 (20.0)	
Q5. Blinding of Participants and Personnel – performance bias				
Low risk of bias	22 (48.9)	4 (40.0)	18 (51.4)	0.87
Unclear	5 (11.1)	1 (10.0)	4 (11.4)	
High risk of bias	18 (40.0)	5 (50.0)	13 (37.1)	
Q6. Blinding of Outcome Assessment – detection bias				
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.4)	0.74
Unclear	9 (20.0)	3 (30.0)	6 (17.1)	
High risk of bias	5 (11.1)	1 (10.0)	4 (11.4)	

Q7. Incomplete Outcome Data – attrition bias				
Low risk of bias	31 (68.9)	6 (60.0)	25 (71.4)	0.57
Unclear	13 (28.9)	4 (40.0)	9 (25.7)	
High risk of bias	1 (2.2)	0 (0.0)	1 (2.9)	
Overall Quality Rating				
GOOD or FAIR	17 (37.8)	4 (40.0)	13 (37.1)	1.00
POOR	28 (62.2)	6 (60.0)	22 (62.9)	

[1] Values are shown as n (%).
[2] PE+ and PE- groups were compared using Fisher's Exact test.