QUALITY OF REPORTING FOR PILOT RANDOMIZED CONTROLLED TRIALS IN THE PEDIATRIC UROLOGY LITERATURE – A SYSTEMATIC REVIEW

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*Authors have no conflict of interest to disclose*
BACKGROUND

• Purpose of a pilot study is to examine the feasibility of conducting a larger definitive randomized controlled trial (RCT)

• The Consolidated Standards of Reporting Trials (CONSORT) statement extension (2016) to pilot/feasibility studies was developed in response to the growing number of publications in the literature described as pilot studies, but that did not have real feasibility outcomes or missed important reporting items.

(ref)
WHAT IS A PILOT STUDY?

• Pilot studies are: “...investigation designed to test the feasibility of methods and procedures for later use on a large scale or to search for possible effects and associations that may be worth following up in a subsequent larger study.” (Everett, 2006)
  – Means of testing the water prior to full scale trial
  – Important step to determining if definitive RCT plan is feasible

• Unlike definitive RCTs, pilot studies aim to determine feasibility, not treatment, diagnostic, or policy outcomes
  – Will not provide meaningful effect size estimates
The aim of this systematic review was to **assess the quality of reporting in pilot RCTs in the Pediatric Urology literature** based on their adherence to the **CONSORT extension for pilot/feasibility studies**.
METHODS

Study Design:

• A comprehensive search was conducted through MEDLINE® and EMBASE®

• Pilot RCTs from 2010-2019 (n=1347)

• Two reviewers independently performed title and abstract screening as well as full text review, with discrepancies resolved by consensus (n=36)

• Quality appraisal, which was also done in duplicate, was performed using the 17 criteria CONSORT extension checklist
METHODS

• An overall quality of reporting score (OQS) was calculated by dividing the number of checklist items present in each study by the maximum possible score (17) and expressed as a percentage.

• Studies were then classified as:
  • low (<40%)
  • moderate (40–70%)
  • high OQS (>70%)
Mean OQS was compared with the presence or absence of four a priori key methodological factors:

- Year
- Biostatistician
- Method of randomization
- Sample size justification

- Data were analyzed using SPSS version 22.0.
RESULTS -

The diagram shows the number of articles for various CONSORT extension criteria, with the following criteria and their respective bars indicating the frequency:

- Title identified as pilot study
- Contact details for corresponding author
- Pilot trial design
- Eligibility criteria and trial setting
- Interventions
- Specific objectives
- Pre-specified outcomes
- Randomization method
- Blinding
- Numbers of participants randomized
- Recruitment status
- Numbers of participants analyzed
- Outcomes and uncertainties
- Adverse effects
- Conclusions and implications for future research
- Trial registration
- Funding disclosure

The length of each bar corresponds to the number of articles, with the Y-axis representing the criteria and the X-axis representing the number of articles from 0 to 40.
### RESULTS – MEAN OQS COMPARED WITH THE PRESENCE OR ABSENCE OF FOUR A PRIORI KEY METHODOLOGICAL FACTORS

<table>
<thead>
<tr>
<th>Methodological Factor</th>
<th>Mean OQS</th>
<th>p-value</th>
</tr>
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<tbody>
<tr>
<td><strong>Year</strong></td>
<td></td>
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</tr>
<tr>
<td>2016-19 (n=30)</td>
<td>45 ± 18.5%</td>
<td></td>
</tr>
<tr>
<td>2010-15 (n=6)</td>
<td>52 ± 14%</td>
<td></td>
</tr>
<tr>
<td><strong>Biostatistician support</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes (n=4)</td>
<td>69 ± 10%</td>
<td>0.01</td>
</tr>
<tr>
<td>No (n=32)</td>
<td>49 ± 14%</td>
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</tr>
<tr>
<td><strong>Method of randomization described</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes (n=11)</td>
<td>63 ± 11%</td>
<td>0.01</td>
</tr>
<tr>
<td>No (n=25)</td>
<td>45 ± 13%</td>
<td></td>
</tr>
<tr>
<td><strong>Sample size justification</strong></td>
<td></td>
<td></td>
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<tr>
<td>Yes (n=6)</td>
<td>69 ± 11%</td>
<td>0.01</td>
</tr>
<tr>
<td>No (n=30)</td>
<td>47 ± 13%</td>
<td></td>
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</tbody>
</table>
CONCLUSIONS

• The mean OQS of pilot studies in pediatric urology was suboptimal (51%)

• Key variables that were significantly associated with a higher OQS were biostatistician support, sample size calculation and method of randomization

• Therefore, adopting the CONSORT extension checklist as a prerequisite for submission of studies identified as ‘pilot’ may improve the reporting and transparency of pilot studies, leading ultimately to improved implementation of future RCTs.
THANK YOU