Assessing and Reporting HR-QOL in Pediatrics with Cystic Fibrosis: A Systematic Literature Review of Clinical Trials

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Divya Patel, Vatsal Chhaya*, Kapil Khambholja
Catalyst Clinical Research, Wilmington, NC, USA

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INTRODUCTION

- Globally, nearly 1,000 new pediatric CF cases occur annually, with over 75% of children being tested before the age of two.[1]
- Pediatric assessments of CF is crucial, as early complications can significantly impact growth, development, and long-term health.
- CF's wide-ranging symptoms significantly impact quality of life, underscoring the need for focused humanistic outcomes research.

HR-QoL lower in children with CF than healthy peer.

Worse pulmonary exacerbations, nutritional status, and FEV1, contributes to significant humanistic consequences.[2]

- **Research gap:** Despite enough evidence of humanistic consequences of CF and regulatory guidelines recommending the inclusion of PROs in trials, the current clinical research on CF has a limited focus on assessing the HR-QOL outcomes, reflecting in sparse clinical trials on CF children having HR-QOL endpoints.
- **Objective:** To synthesize available evidence and provide comprehensive characterization of HR-QoL parameters utilised among paediatric CF patients in clinical trial settings.

METHODS

Study retrieval and selection based on **PICO framework**:

- **Participants:** Pediatric patients (0-18 years) with CF;
- **Intervention/exposure:** Studies with various interventions for managing CF, discussing QoL measures;
- **Comparator/control:** No comparator;
- **Outcome:** HR-QoL measures reported in CTs, tools used for HR-QoL assessment

Inclusion Criteria:

- Studies involving pediatric patients, aged 0-18 years, diagnosed with CF.
- Study types included clinical trials such as RCT, cross-over, open-label trials.
- Studies including HR-QoL as primary or secondary outcome measure.
- Articles published in English between Jan 2014 and Jun 2024.

Exclusion Criteria:

- Studies including adults, or both adults and children.
- Study types such as observational studies, case reports, reviews.
- Studies evaluating only clinical outcomes and no data on HR-QoL.

Study selection

Titles and abstracts screening followed by full-text articles retrieval

Data extraction

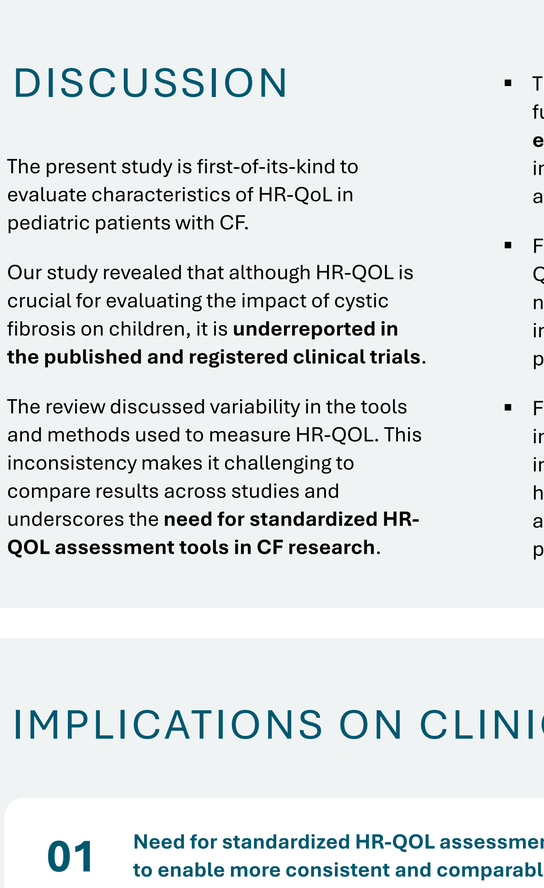
Standardized form including study characteristics, demographics, HR-QOL tools, and its domains

Data synthesis & Presentation

Narrative synthesis, tabular and graphical presentation for frequency of HR-QoL tools used, domains studied

RESULTS

- A total of **24** articles were included for analysis.



- **Study characteristics:** Majority of studies (17/24; 70.8%) were RCTs, six (25%) were open-label and one (4.2%) uncontrolled trial.
- **HR-QoL tools:** The most used tool for assessing HR-QoL was **Cystic Fibrosis Questionnaire-Revised (CFQ-R) (79%.1)** followed by **Pediatric Quality of Life Inventory (PedsQL) (16.7%).**
- Five studies included questionnaire for parents (CFQ-R parents, parent PedsQL); comprising similar domains under evaluation.
- Clinical parameters such as **FEV1**, **BMI**, and **nutritional status** were associated with HR-QoL.
- **Reliability:** Three studies evaluated reliability using Cronbach alpha; the findings were in range of 0.6-0.9.
- **Registry-findings:**
  - 25 of 91 CTs (27.5%) reported HR-QoL as study endpoint in the registered clinical trials.
  - 76% (16/25) of CTs used CFQ/CFQ-R for assessing HR-QoL (Figure 2).
  - SN-5 and VAS were used in two registered trials, the use of these scales were not observed in published literature (database findings).

HR-QoL Variables

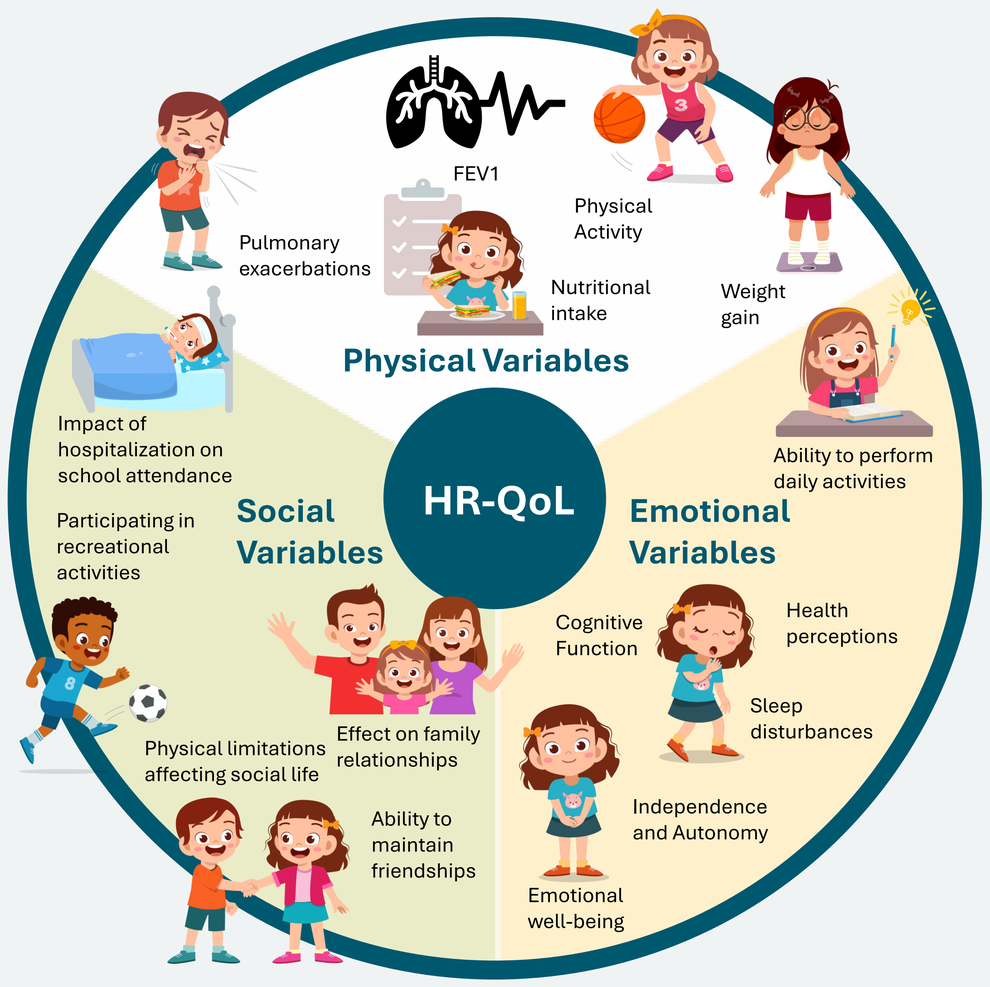


Figure 1:

Frequency of HR-QoL tool used in clinical trials (Database findings)

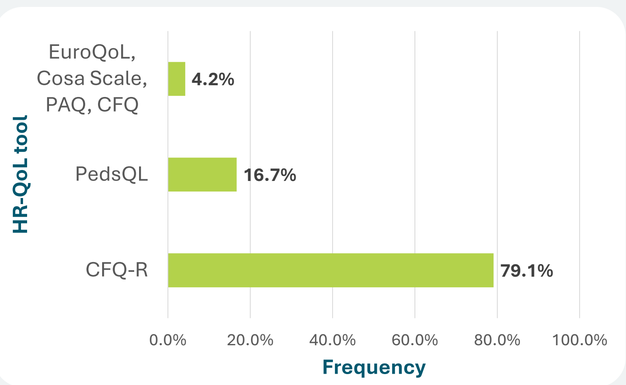
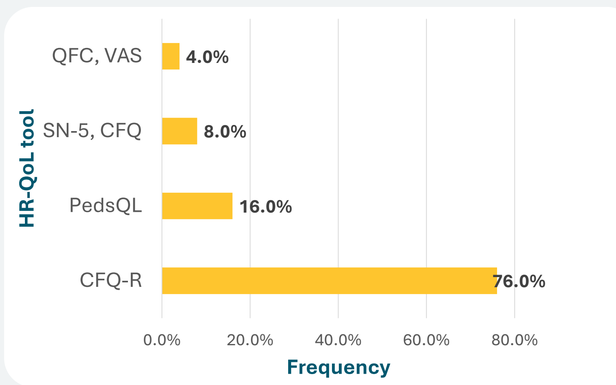


Figure 2:

Frequency of HR-QoL tool used in clinical trials (Registry findings)



DISCUSSION

- The present study is first-of-its-kind to evaluate characteristics of HR-QoL in pediatric patients with CF.
- Our study revealed that although HR-QOL is crucial for evaluating the impact of cystic fibrosis on children, it is **underreported in the published and registered clinical trials**.
- The review discussed variability in the tools and methods used to measure HR-QOL. This inconsistency makes it challenging to compare results across studies and underscores the **need for standardized HR-QOL assessment tools in CF research**.
- The predictors of HR-QoL included pulmonary function (FEV1), frequency of **pulmonary exacerbations, and nutritional status** indicating importance to address both physical and emotional aspects of care.[3]
- Further, variability in different aspects of HR-QoL for adult and pediatric patients, necessitates integrating HR-QoL assessments into routine clinical practice for both adult and pediatric patients.
- Few challenges anticipated in this process include absence of standardized measurement instruments, limited awareness among healthcare professionals regarding HR-QoL, and primary focus on clinical outcomes over patient experiences.

IMPLICATIONS ON CLINICAL TRIAL

01 Need for standardized HR-QOL assessment tools specific to pediatric CF patients, to enable more consistent and comparable measurements across clinical settings.

02 Healthcare providers to consider incorporating HR-QOL assessments into routine practice to better understand and address the comprehensive needs of CF patients.

03 Self-assessment scales (VAS) should be used in routine assessments to evaluate symptoms of anxiety or sleep disturbances.

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CONTACT INFORMATION

Kapil Khambholja, Ph.D.
Executive Director, Head of Medical Writing and Product Strategy Lead
Catalyst Clinical Research
Phone: +91-77029 49998 | Email: kapil.khambholja@catalystcr.com
www.CatalystCR.com

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