

## INTRODUCTION



Tremendous advances are being made in treatments that improve survival rates and health-related quality of life (HRQoL) in childhood diseases.



A number of generic patient-reported outcome measures (PROMs) have been developed to measure HRQoL in children and adolescents.



ClinicalTrials.gov database can provide contemporary insights into PROM utilization trends in clinical studies, unlike published results, which are usually available several years after studies start.

## OBJECTIVE

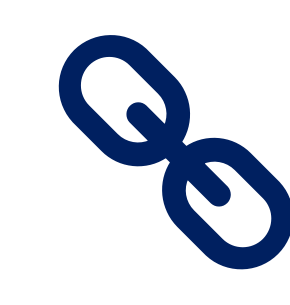


To examine trends in the use of generic PROMs in registered clinical trials in children, using the ClinicalTrials.gov database.

## METHODS



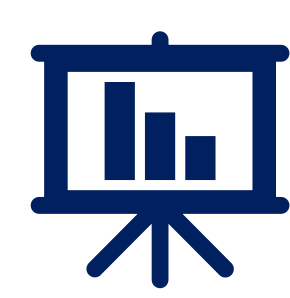
ClinicalTrials.gov database was queried to identify interventional (clinical) trials in pediatric population, registered between January 2008 and December 2021.



Studies that reported using generic pediatric PROMs (identified in a recent systematic review<sup>1</sup>) were identified using instruments' full names, abbreviations, and relevant existing version names strung together using Boolean operators.



Trends in the use of generic PROMs (n = 110) were divided into preference (n = 17) and non-preference (n = 93) based and assessed by year, region, disease area and trial phase.

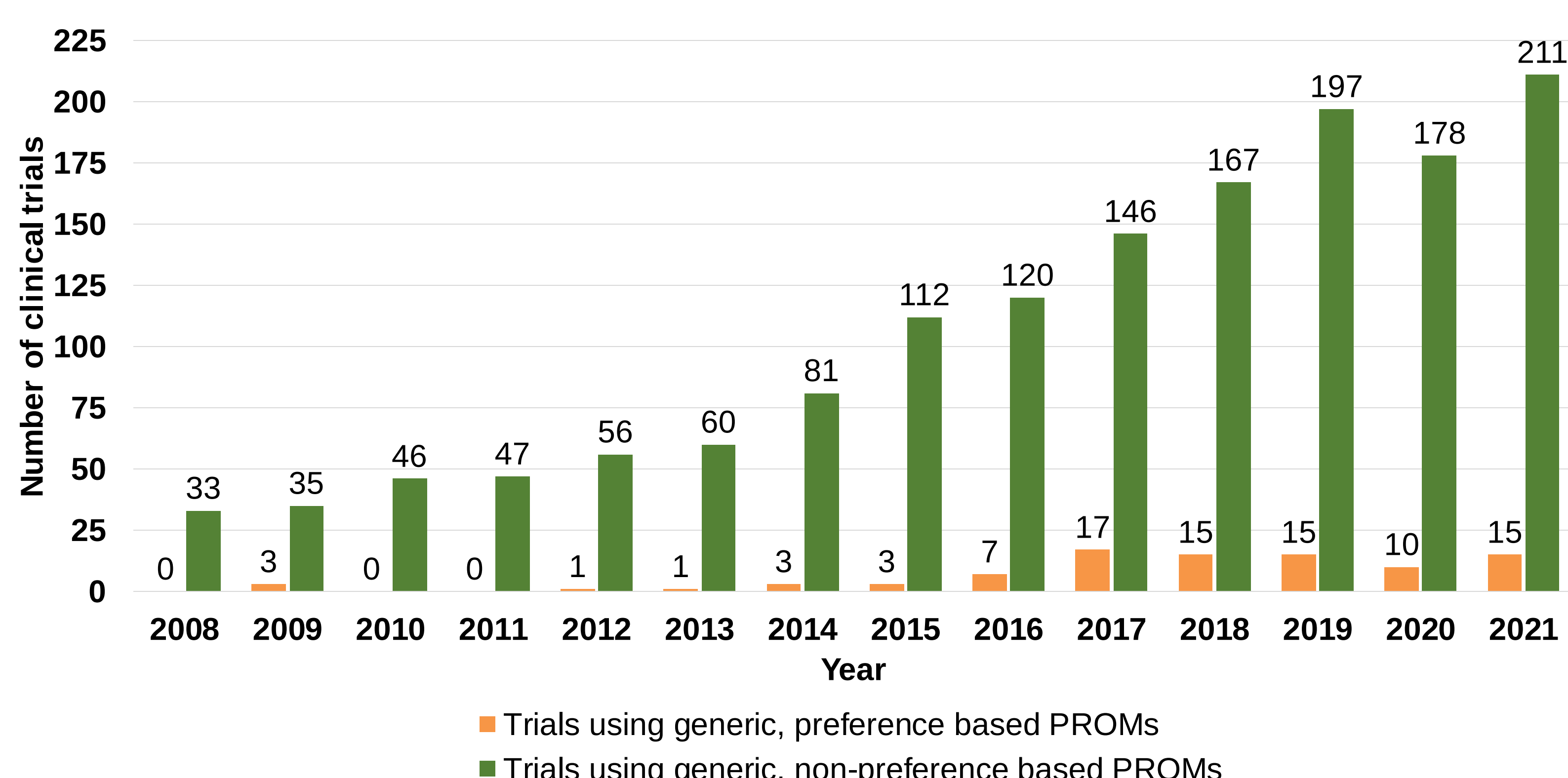


Trends in the use of the five commonly used generic PROMs were further assessed.

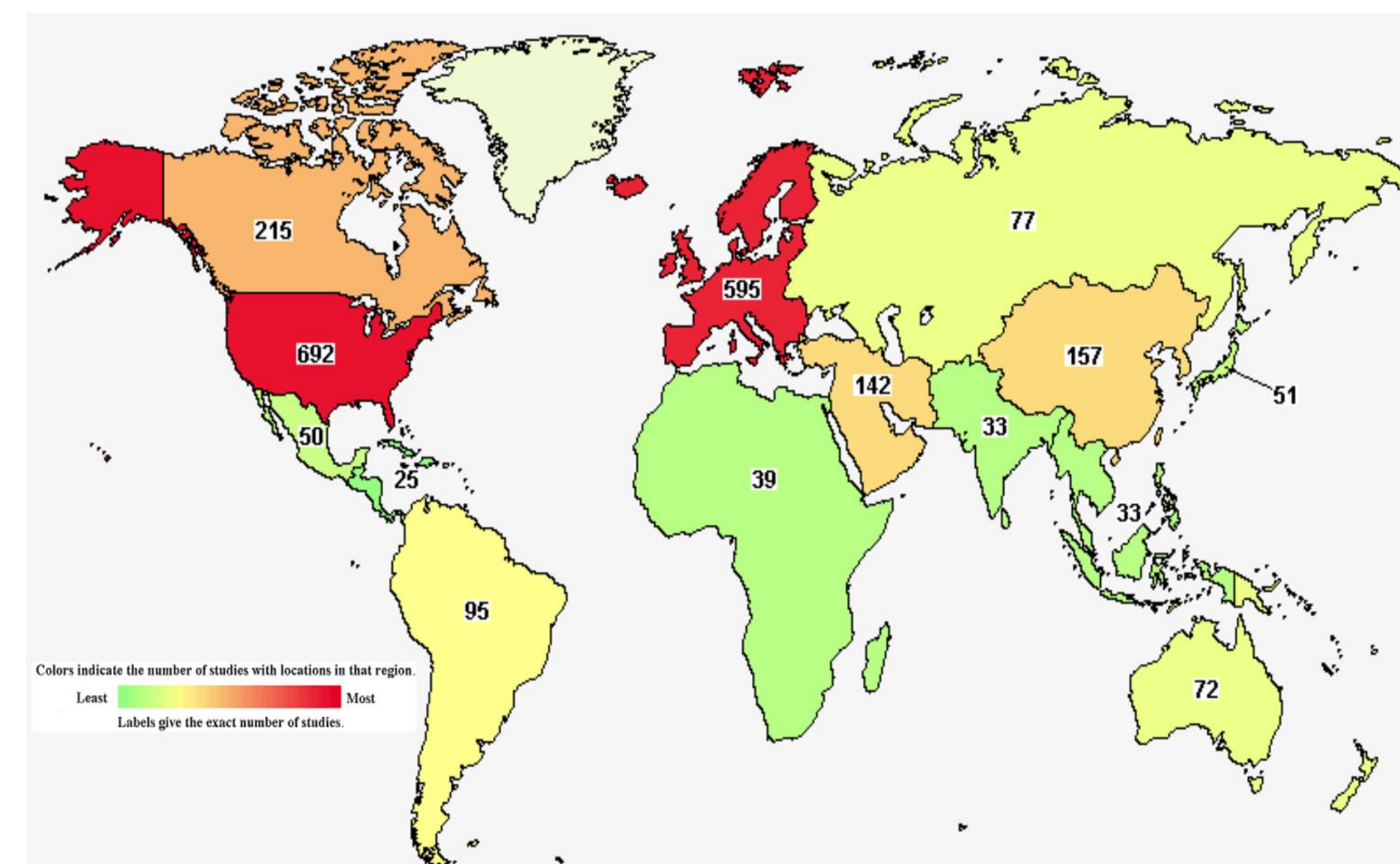
## RESULTS

- A total of 41,635 pediatric clinical trials registered between 2008 to 2021 were identified. Of these, 8,846 (21.2%) used any type of PROM; with an increase from 256 (13%) in 2008 to 1,068 (28%) in 2021
- The proportion of trials that used generic PROMs of interest was 1,551 (3.7%), with an increase from 33 (1.68%) in 2008 to 219 (5.7%) in 2021.
- Among clinical trials using generic PROMs, 1,489 (96%) trials used non-preference-based PROMs, and 90 (6%) used preference-based PROMs.

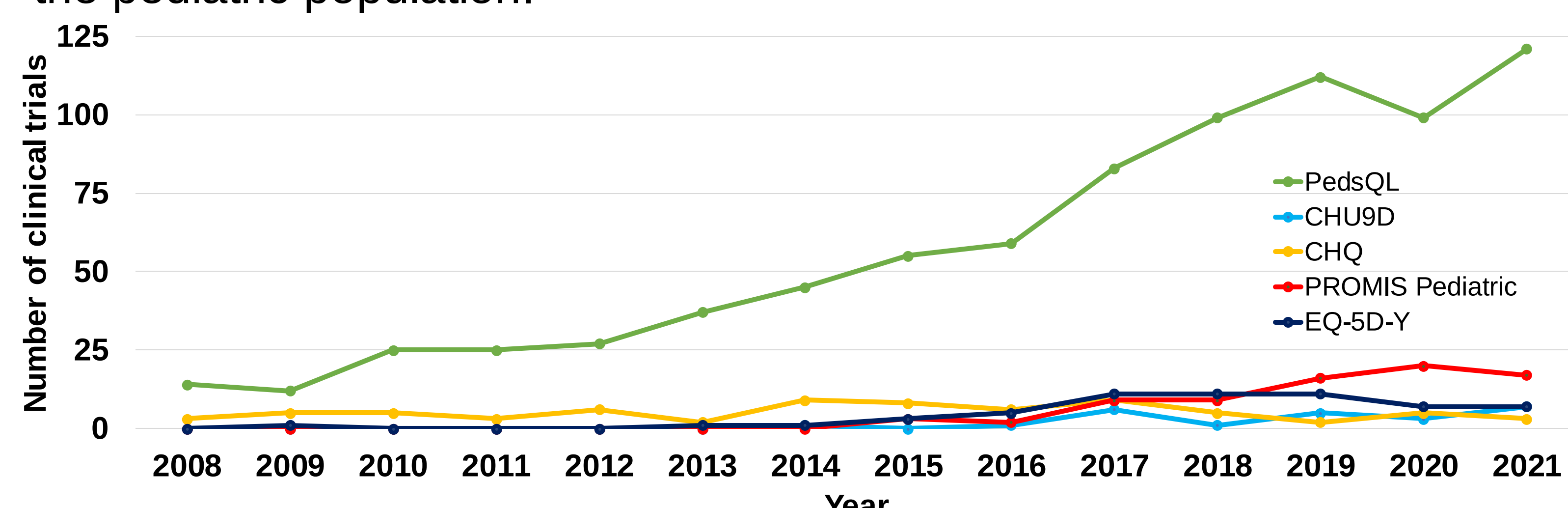
**FIGURE I:** Annual trends in use of generic PROMs for children/adolescents in clinical trials



**Figure II:** Regional distribution of pediatric clinical trials with generic PROMs.

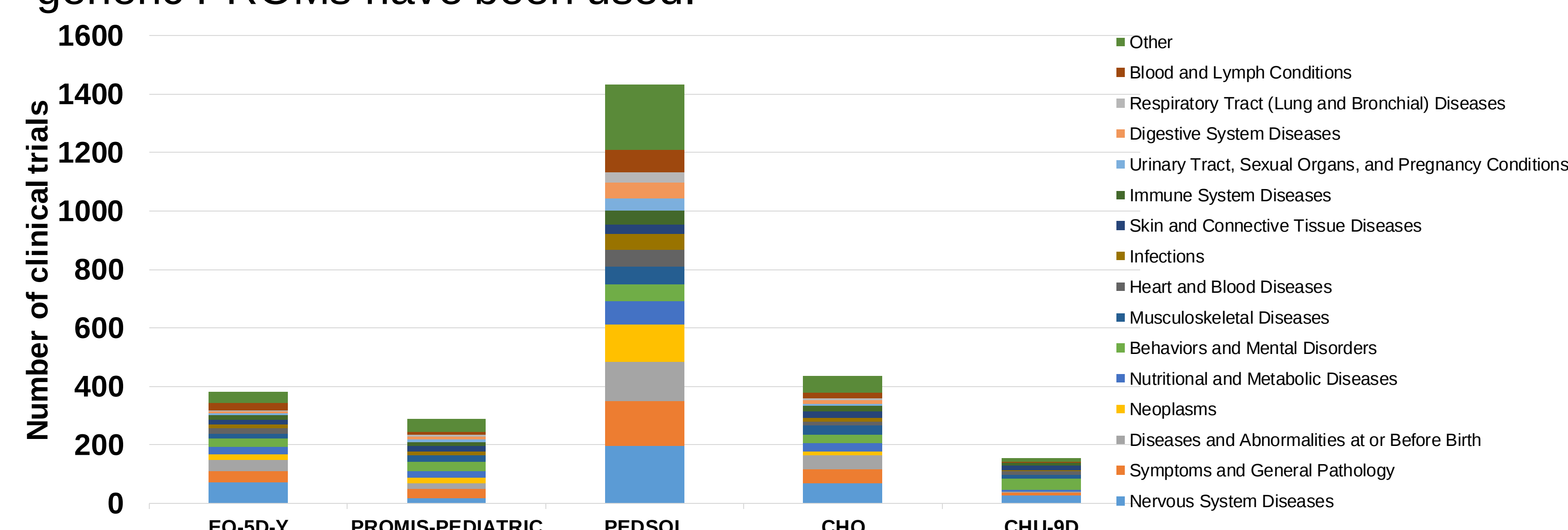


**Figure III:** Annual trends in five most commonly used generic PROMs in the pediatric population.

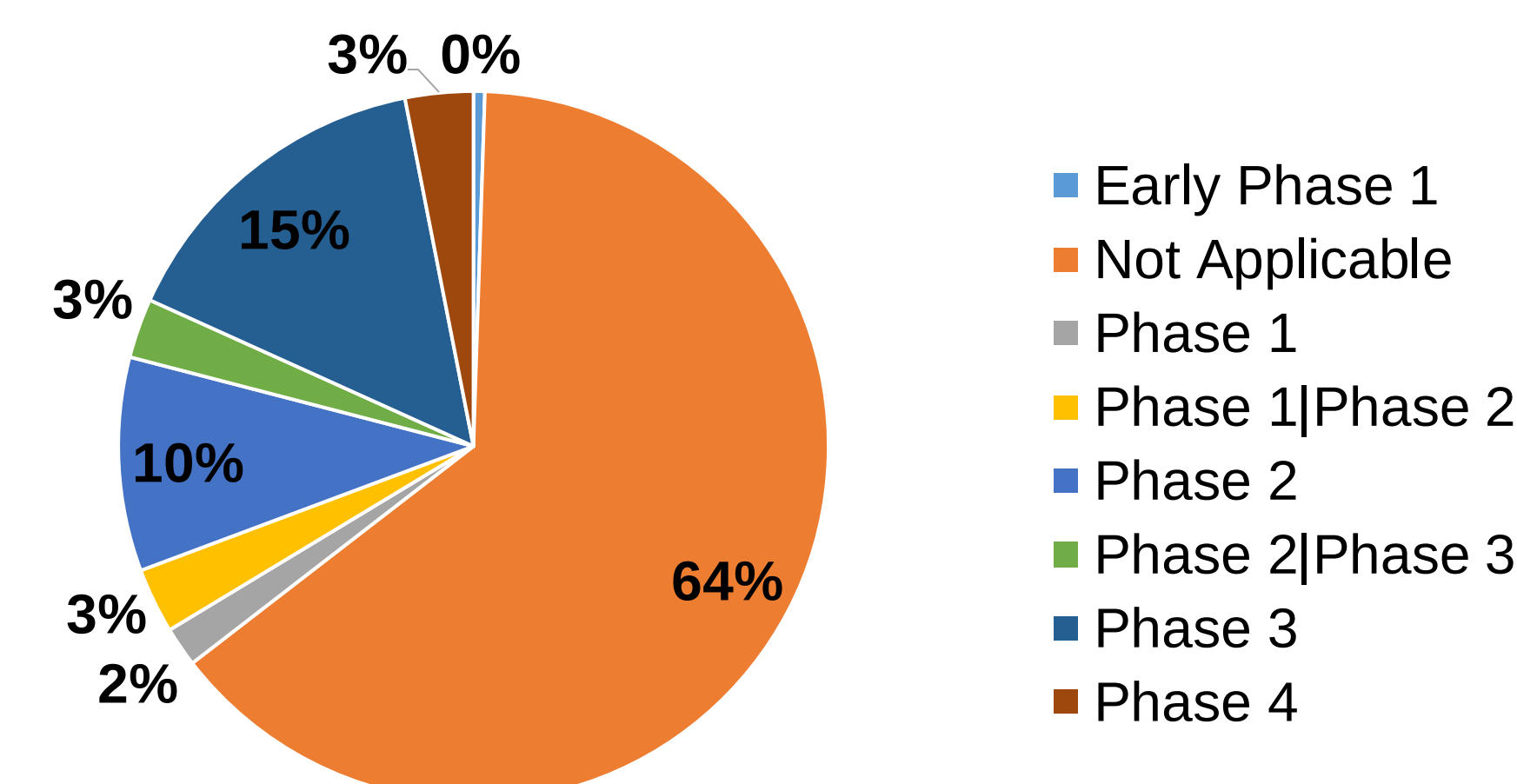


PedsQL: Pediatric quality of life inventory<sup>2</sup>; CHU-9D: Child Health Utility 9D<sup>3</sup>; CHQ: Child Health Questionnaire<sup>4</sup>; PROMIS Pediatric: Patient Reported Outcomes Measurement Information System Pediatric<sup>5</sup>; EQ-5D-Y: EuroQoL-5D-Youth<sup>6</sup>

**Figure IV:** Distribution of disease areas in which five most common generic PROMs have been used.



**Figure IV:** Distribution of clinical trials using generic PROMs by study phase (n = 1,551)



## DISCUSSION

- Since 2008, there has been an increase in the use of generic PROMs in clinical trials. PedsQL is the most used measure, with the highest increase in utilization from 2008 to 2021.
- Non-preference based generic PROMs are much more widely used than preference based. It is possible that preference-based scoring algorithms may exist or be forthcoming for some non-preference based PROMs.

## CONCLUSIONS

- Despite recommendations by regulatory agencies, the inclusion of generic PROMs remains uncommon in pediatric clinical trials.
- Efforts should be undertaken to include PROMs in pediatric clinical trials as they represent an underused opportunity to capture HRQoL treatment outcomes in pediatric populations.

### REFERENCES:

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**Disclosures:** Pickard AS is a member of the EuroQol group. No other disclosures to report.

Presented at the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Conference, Vienna, Austria, November 2022.

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