Inclusion of Patient-Reported Outcome Measures in Pediatric versus Adult Populations in FDA Biological and Novel Drug Therapy Approvals from 2021–2025

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INTRODUCTION

- Food and Drug Administration (FDA) guidance recommends administering patient-reported outcome (PRO) measures to pediatric populations[1]
  - Observer-reported outcome measures do not directly capture the patient experience
  - Proxy measures completed by caregivers do not necessarily reflect the patient's perspective
- The value of collecting direct patient experience data from pediatric populations, including assessment using PRO measures, has received increasing attention and was the focus of a recent International Society for Quality of Life Research workshop[2]
- Disparities in the administration of PRO measures to pediatric populations have been documented in several studies, especially cancer trials[3], as well as a recent study that examined implementation of commonly administered measures (e.g. PROMIS measures, PedsQL)[4]

OBJECTIVES

- The objective of this research was to examine the current landscape of PRO measure implementation in pediatric versus adult populations for biologics and novel drug therapies (i.e. therapies never before approved or marketed in the US) approved by the FDA from 2021–2025.

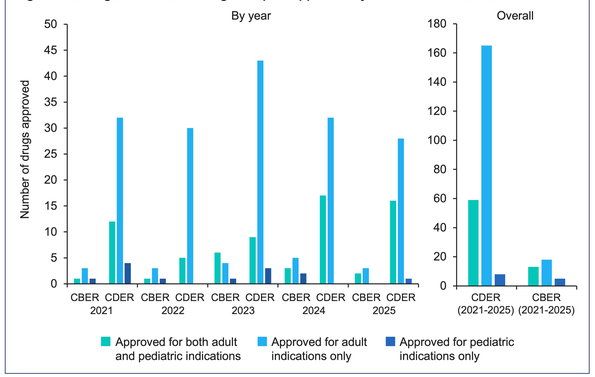
METHODS

- Searches were conducted in the FDA Center for Drug Evaluation and Research (CDER) Novel Drug Approvals and Center for Biologics Evaluation and Research (CBER) Biological Approvals listings for products that were initially approved between 2021–2025
- Products that would not typically include PRO assessments (e.g. vaccines, plasma) and non-therapeutic products (e.g. diagnostic tests) were excluded
- For each eligible product identified, the corresponding product label was retrieved. ClinicalTrials.gov and MEDLINE® were searched for registrational trial records, trial protocols and publications
- PRO-related information from all sources was extracted. Detailed information was systematically compiled and aggregated into summary tables in Microsoft Excel® for each product identified, including its initial approval date, database source (CDER versus CBER), therapeutic area, treatment population age group(s), and PRO measures included in the label, registrational trial and/or publications
- Results were analyzed and summarized using descriptive statistics

RESULTS

- A total of 270 products were identified from the CDER and CBER listings (**Figure 1**)
  - 183 (67.8%) were approved for adult indications only
  - 72 (26.7%) were approved for both adult and pediatric indications
  - 13 (4.8%) were approved for pediatric indications only
  - Two (0.7%) were not evaluable at the time of analysis (lifecycle-driven product and late-2025 approval with label not yet available)

Figure 1. Biological and novel drug therapies approved by the FDA from 2021-2025



PRO label claims

- A total of 44 products (16.3%) received a PRO label claim
  - Thirty-three (75.0%) were for products approved for adult indications and 11 (25.0%) were for products approved for both adult and pediatric populations
  - Similar proportions were observed for PRO labeling statements for products, with 18.0% indicated solely for adults and 15.2% indicated for both adult and pediatric populations; most statements on pediatric PRO endpoints were limited to adolescents
  - No products approved solely for pediatric indications received a PRO label claim
- Of the 11 products approved for both adult and pediatric indications that received a PRO label claim:
  - Seven (63.6%) were for products approved for adult and adolescent populations and the label included PRO data for both populations
  - Two (18.2%) were for drugs approved for adults, adolescents and children under age 12 and included PRO data for all of these age groups in the label
  - Two (18.2%) were for products that were indicated for adults, adolescents and children under age 12, but the label only included PRO data for adults and adolescents

Registrational trials

- A total of 175 products (64.8%) included at least one PRO measure in the registrational trials, with PRO measures most commonly administered in trials of products indicated for adults (**Figure 2**)
- Nearly two thirds of products indicated solely for adults (64.5%), three quarters of products indicated for both adult and pediatric populations (72.2%) and two fifths of products indicated solely for a pediatric population (38.5%) included at least one PRO measure in their registrational trials (**Figure 3**)
- Of those products indicated for both adult and pediatric populations:
  - 98.1% administered PRO measures to adult participants
  - 86.5% administered PRO measures to adolescent participants (ages 12–17)
  - 23.9% exclusively administered PRO measures to adult participants
  - 68.0% of products indicated for adults, adolescents and children administered PRO measures to child participants (under age 12)

Figure 2. Registrational trials that included at least one PRO measure by population (n=175)

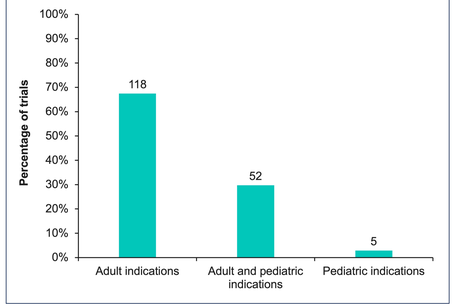


Figure 3. Percentage of registrational trials including at least one PRO measure by population

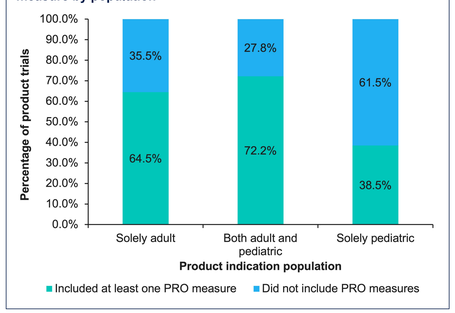
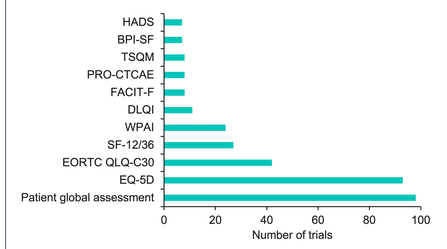
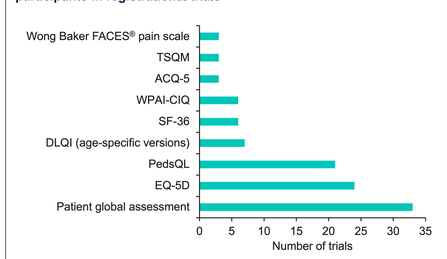


Figure 4. PRO measures most commonly administered to adult participants in registrational trials



Key: BPI – SF, Brief Pain Inventory – Short Form; DLQI, Dermatology Life Quality Index; EORTC QLQ-C30, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire Core 30; EQ-5D, EuroQol- 5 Dimension; FACIT-F, Functional Assessment of Chronic Illness Therapy – Fatigue; HADS, Hospital Anxiety and Depression Scale; PRO-CTCAE, Patient-Reported Outcomes version of the Common Terminology Criteria for Adverse Events; SF12/36, 12/36-Item Short Form Survey; TSQM, Treatment Satisfaction Questionnaire for Medication; WPAI, Work Productivity and Activity Impairment Questionnaire

Figure 5. PRO measures most commonly administered to pediatric participants in registrational trials



Key: ACQ-5, Asthma Control Questionnaire; DLQI, Dermatology Life Quality Index; EQ-5D, EuroQol- 5 Dimension; PedsQL, Pediatric Quality of Life Inventory; SF36, 36-Item Short Form Survey; TSQM, Treatment Satisfaction Questionnaire for Medication; WPAI-CIQ, Work Productivity and Activity Impairment Questionnaire plus Classroom Impairment Questionnaire

Characteristics of PRO measures identified

Generic versus disease-specific

- Across all products with a PRO label claim, generic (49.2%) versus disease-specific (50.8%) measures appeared with approximately the same frequency
  - Similar breakdowns were observed for label claims in adult indications (48.0% generic versus 52.0% disease-specific) and indications for both adult and pediatric populations (50.0% each generic versus disease-specific)
- In registrational trials, the proportion of generic versus disease-specific measures administered to participants was greater for pediatric populations (69.3% versus 30.7%) compared with adults (56.7% versus 43.3%)

Most commonly used measures

- The measures most commonly administered to adult participants (**Figure 4**) included a patient global assessment, followed by the EuroQol- 5 Dimension (EQ-5D), European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire Core 30 (EORTC QLQ-C30), 12/36-Item Short Form Survey (SF-12/SF-36), Work Productivity and Activity Impairment Questionnaire (WPAI), and the Dermatology Quality of Life Index (DLQI)
- The measures most commonly administered to pediatric participants (**Figure 5**) included a patient global assessment, followed by the EQ-5D, the Pediatric Quality of Life Index (PedsQL), DLQI (age-specific versions), SF-36, and WPAI

Publication of PRO-related findings

- Published PRO-related results from registrational trials were identified for 124 (70.9%) products that included at least one PRO measure, representing:
  - 69.5% of products indicated solely for adults that included at least one PRO measure
  - 73.1% of products indicated for both adult and pediatric populations that included at least one PRO measure
  - 80.0% of products indicated for solely for pediatric populations that included at least one PRO measure
- However, nearly one fifth of trials (19.4%) that administered PRO assessments to both adult and pediatric populations only published PRO-related findings for adult trial participants

KEY TAKEAWAYS

- Across all age groups, very few biological and novel drug therapies received a PRO label claim within the past 5 years; label claims for pediatric populations were mostly limited to adolescents and no products indicated solely for a pediatric population received a label claim
- Inclusion of PRO measures in registrational trials was slightly more frequent in products indicated for both adult and pediatric populations than for products indicated solely for adults; however, nearly one quarter of these trials only administered PRO assessments to adult participants
- Most products indicated solely for a pediatric population did not include PRO measures in their registrational trials
- In registrational trials, the proportion of generic versus disease-specific measures administered to participants was greater for pediatric populations compared with adult populations

CONCLUSIONS

- Despite FDA guidance and calls for inclusion of PRO assessments in pediatric populations in the literature and symposia, PRO measures remain underutilized for pediatric populations in registrational trials of biological and novel drug therapies, particularly for children under age 12 and for indications limited to pediatric populations
- Expanding the incorporation of pediatric PRO measures into clinical development programs could help improve patient-centered treatment outcomes in this population
- Increasing the use of fit-for-purpose, disease-specific pediatric PRO measures has the potential to ensure assessment of the most relevant concepts for this patient population and better support evaluation, interpretation and documentation of meaningful treatment benefit

REFERENCES

1. US Food and Drug Administration, 2025. **2.** ISOQOL, 2024. **3.** Murugappan et al. *J Natl Cancer Inst*. 2022; 114(1):12-19. **4.** Kuharic et al. *Value Health*. 2026; 29(3):449-456.

DISCLOSURES

LTW, **KV**, **MB** and **MG** are employees of Lumanity, Inc.

An electronic version of the poster can be viewed by scanning the QR code.

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