Landscape of Patient-Reported Outcomes Measures (PROms) in Oncology Trials: Key Insights Into Patient Voice and Value

Trupti Dhumal, Ph.D.; Jinal Chheda, M.S.; Sahil Bhave, M.S.; Ambarish Ambegaonkar, Ph.D.
APPERTURE LLC
(Corresponding Author: ambi@apperturehealth.com)

PCR 255

APPERTURE
FOCUSED VALUE

Background

- In the past few decades, the treatment philosophy in oncology has evolved from being disease-centered to more patient-centered.
- Patient-reported outcome measures (PRO) are integral to oncology clinical research since they provide a window into the patient's perception of the disease, and the surrogate endpoints may help understand treatment benefits.[1,2]
- Strengthening the integration of PROms in clinical trials can enhance the overall patient experience and ensure that clinical outcomes align with patient priorities.[3]
- Analyzing the landscape of PROms in trials may help assess their role in improving patient care, guiding regulatory decisions, and shaping future research.

Objective

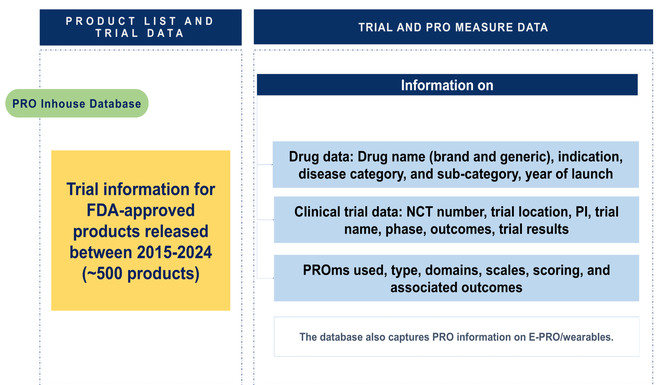
This study aimed to characterize the use of PROms in US-FDA approved oncology therapies in the past decade by:

- Assessing the landscape of clinical trials of approved FDA drugs in oncology.
- Assessing the prevalence of PROms used in each clinical trial.
- Providing an overview of the PROm by its type and associated domains.

Methods

- Data on oncology clinical trials and PROms were extracted from an internal database of 500 FDA approved and launched since 2015.
- Using the database, we analyzed clinical trials incorporating PROs in US FDA-approved drugs from 2015 to 2025.
- The analysis focused on actively recruiting/completed and industry-sponsored clinical trials.
- We examined the distribution of trials across different cancer types and assessed PRO usage by trial phase and cancer type.
- Further analysis included the frequency of use of PROs as a primary vs secondary outcome.
- We also analyzed the distribution of PROms based on type, domains, and adoption before and after the release of the US FDA's 2021 guidance on measuring PROms in oncology trials.

PRO Database Structure



References

1. Bellino S, La Salvia A. The Importance of Patient Reported Outcomes in Oncology Clinical Trials and Clinical Practice to Inform Regulatory and Healthcare Decision-Making. Drugs R D. 2024;24(2):123-127. doi:10.1007/s40268-024-00478-2
2. Basch E. Toward patient-centered drug development in oncology. N Engl J Med. 2013;369(5):397-400. doi:10.1056/NEJMp1114649
3. de Ligt KM, Hommes S, Vromans RD, Boomstra E, van de Poll LV, Krahmer EJ. Improving the Implementation of Patient-Reported Outcome Measure in Clinical Practice: Tackling Current Challenges With Innovative Digital Communication Technologies. J Med Internet Res. 2025;27:e60777. Published 2025 Feb 5. doi:10.2196/60777

Results

Table 1: Overview of PROs used in Oncology Clinical Trials

CATEGORY	PROm/INSTRUMENT	DOMAINS
CORE-GENERIC	European Organization for Research and Treatment of Cancer quality of life questionnaire-C30 (EORTC QLQ-C30)	Functional (Physical, Role, Emotional, Cognitive, and Social Functioning); Global Health Status; Symptoms; Additional symptoms (Dyspnea, Insomnia, Appetite Loss, Constipation, Diarrhea), and Perceived Financial Impact
	EuroQol 5-Dimension (EQ-5D-3L)	Mobility; Self-Care; Usual Activities; Pain/Discomfort; Anxiety/Depression
	EQ-5D-5L	Same five domains as EQ-5D-3L, with five levels of Severity to Enhance Sensitivity
	EuroQol 5-Dimension Visual Analog Scale (EQ-5D-5L VAS)	Mobility; Self-Care; Usual Activities; Pain/Discomfort; Anxiety/Depression
	Functional Assessment of Cancer Therapy – General (FACT-G)	Physical; Social; Emotional; and Functional Well-Being
	Patient-Reported Outcomes Measurement Information System Global-10 (PROMIS-10)	Physical Functioning; General Mental Health; Emotional Distress; Satisfaction with Social Activities and Relationships; Ability to Carry Out Usual Social Activities; Pain; Fatigue, and Overall Quality of Life
	Short Form-12 (SF-12)	Physical Functioning; Role Physical; Bodily Pain; General Health; Vitality; Social Functioning; Role Emotional; Mental Health
	Short Form-36 (SF-36)	Same domains as SF-12, with additional items for a more comprehensive assessment
COGNITIVE/NEUROLOGICAL	Montreal Cognitive Assessment (MoCA)	Memory; Executive Functioning; Attention; Language; Visuospatial, and Orientation
	M.D. Anderson Symptom Inventory – Brain Tumor (MDASI-BT)	13 Core Symptoms (e.g., Pain; Fatigue; Nausea) and 6 interference items (e.g., Activity; Mood)
	Happiness and activity scale	Subjective experiences of happiness; Potentially including areas like Physical Health; Psychological Well-Being; Social Relationships; and the Environment
SYMPTOM-SPECIFIC	Patient Global Impression of Severity (PGIS)	Disease Severity and Response to Treatment
	Patient Global Impression of Change (PGIC)	Activity Limitations; Symptoms; Emotional; and Overall Quality of Life
	Myeloproliferative Neoplasm Symptom Assessment Form Total Symptom Score (MPN-SAF TSS)	10 Symptoms (e.g., Fatigue; Early Satiety; Abdominal Discomfort; Inactivity; Problems with Concentration; Night Sweats; Itching; Bone Pain; Fever; Weight Loss)
	Advanced Systemic Mastocytosis Symptom Assessment Form (AdvSM-SAF)	8 Symptoms (e.g., Fatigue; Pain; Nausea; Vomiting; Diarrhea; Spots; Itching; Flushing)
	Rotterdam Symptom Checklist (RSCL) Activity Level Scale	Physical Symptom Distress; Psychological Distress; Activity Level, and Overall Global Life Quality
	Work Productivity and Activity Impairment (WPAI) Questionnaire WPAI	Absenteeism (missing work); Presenteeism (reduced productivity while at work); Overall Work Productivity, and Non-Work Activity Impairment
	Non–Small Cell Lung Cancer Symptom Assessment Questionnaire (NCSLC-SAQ)	Cough; Pain; Dyspnea (Shortness of Breath); Fatigue; and Appetite
	Myelofibrosis Symptom Assessment Form (MFSAF)	Severe Constitutional Symptoms (i.e. Fatigue, Night Sweats, Fever, Weight Loss), Pruritus, and Symptoms
	Western Ontario McMaster Arthritis Index adapted for PVNS and GCTTS (WOMAC PVNS-GCTTS)	Pain; Stiffness; Physical Function
	PROMIS Physical Function (PF) items	NA
	Brief Pain Inventory (BPI)	Pain Intensity and Pain Interference
CANCER-SPECIFIC	EORTC QLQ-BR23 (Breast Cancer Module)	Body Image; Sexual Functioning; Sexual Enjoyment; Future Perspective; Systemic Therapy Side Effects; Breast Symptoms; Arm Symptoms; and Upset by Hair Loss
	EORTC QLQ-BR45 (Breast Cancer Module)	Body Image; Sexual Functioning; Sexual Enjoyment; Future Perspective; Systemic Therapy Side Effects; Breast Symptoms; Arm Symptoms; and Upset by Hair Loss
	EORTC QLQ-H&N35 (Head and Neck Cancer Module)	Functional (Swallowing; Speech; Taste; Opening Mouth; Social Eating); Symptom (Pain; Dry Mouth; Sticky Saliva; Taste); Other (Social Contact, Illness; Sexuality)
	EORTC QLQ-OES18 (Esophageal Cancer Module)	Dysphagia; Eating; Reflux; and Pain
	EORTC QLQ-OV28 (Ovarian Cancer Module)	Symptoms (Abdominal; Hormonal; Peripheral; Chemotherapy Side Effects); Functional (Body Image; Attitudes; Sexual Functioning; Sexual Enjoyment)
	EORTC QLQ-CX24 (Cervical Cancer Module)	Symptom Experience; Body Image; and Sexual Function, Six Single-Item Scales (Lymphoedema, Peripheral Neuropathy, Menopausal Symptoms, Sexual Worry, Activity, and Enjoyment)
	EORTC QLQ-HCC18 (Hepatocellular Carcinoma)	Fatigue; Body Image; Jaundice; Nutrition; Pain; and Fever, along with Single Items addressing Abdominal Swelling and Sex Life
	EORTC QLQ-PR25 (Prostate Cancer Module)	Urinary Symptoms; Bowel Symptoms, Treatment-related Symptoms; and Sexual Activity and Functioning
	EORTC QLQ-LC13 (Lung Cancer Module)	13 Core Symptoms (Coughing; Pain; Dyspnea; Sore Mouth; Peripheral Neuropath; and Hair Loss)
	TTD in EORTC QLQ-C30 (Derived time-to-deterioration metrics)	NA
	MCL-Specific Symptoms (EORTC Item Library-Mantle Cell Lymphoma)	Pain; Instability; Swelling; Bruising; Popping Soun; Tenderness; Stiffness; Locking or Catching
CANCER-SPECIFIC (FUNCTIONAL ASSESSMENT OF CANCER THERAPY)	FACT-B (Breast)	Physical Well-Being, Social/Family Well-Being, Emotional Well-Being, Functional Well-Being, Breast Cancer Subscale
	FACT-M (Melanoma)	Melanoma-specific concerns alongside general quality of life domains
	FACT-C (Colon)	Colorectal cancer-specific concerns in addition to general quality of life domains
	FACT-MM (Multiple Myeloma)	Multiple Myeloma-specific concerns in addition to general quality of life domains
	FACT-LYM (Lymphoma)	Physical Well-Being, Social/Family Well-Being, Emotional Well-Being, Functional Well-Being, Lymphoma
	FACT-P (Prostate)	Prostate cancer-specific concerns alongside general quality of life domains
	FACT-Taxane	Physical Well-Being, Social/Family Well-Being, Emotional Well-Being, Functional Well-Being, Taxane Therapy
	FACT/GOG-Ntx4 (Neurotoxicity)	Physical Well-Being, Social/Family Well-Being, Emotional Well-Being, Functional Well-Being, Neurotoxicity
	FLymSI-18 (Lymphoma Symptom Index-18)	Disease-Related Symptoms – Physical, Disease-Related Symptoms – Emotional, Treatment Side Effects, Function/Well-Being
	FACT-B TOI-PFB (Trial Outcome Index – Physical/Functional/Breast subscale)	NA
SATISFACTION, PREFERENCES, & UTILITY	Cancer Treatment Satisfaction Questionnaire (CTSQ)	Expectations of cancer therapy, Feelings about side effects, Oral cancer therapy adherence, Convenience, Satisfaction with cancer therapy, Stopping cancer therapy, and Reasons for nonadherence
	Oncology Opportunity Cost Assessment Tool (OOCAT Survey)	Opportunity Cost of Seeking Cancer Care, encompassing Time Requirements for Appointments, Financial Implications of Travel, and Logistical/Quality-of-Life Challenges
ADVERSE EVENTS	Patient-Reported Outcomes version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE)	Presence; Severity, and Interference of Symptoms
ANXIETY, DEPRESSION, AND SUICIDE	Hospital Anxiety and Depression Scale (HADS)	14 items focused on Anxiety and Depression
	Beck Depression Inventory (BDI)	Emotional; Cognitive; Motivational; and Physiological
	Patient Health Questionnaire (PHQ-9)	Severity and Frequency of Depressive Symptoms
	Memorial Anxiety Scale for Prostate Cancer (MAX-PC)	Prostate Cancer Anxiety; PSA Anxiety; and Fear of Recurrence
	Columbia Suicide Severity Rating Scale (C-SSRS)	Severity of Suicidal Ideation and Behavior

Figure 1: Presence of PROs across Cancer Types

PRO	Breast Cancer	Skin Cancer	GI Cancer	Gynecological Cancer	Hematology	Lung Cancer/ NSCLC	Head and Neck Cancer	Colon Cancer	Solid Tumor	Soft Tissue Sarcoma	Urologic Cancer	Renal Cancer
EORTC QLQ-C30	X	X	X	X	X	X	X	X			X	
EQ-5D-3L	X	X	X								X	
EQ-5D-5L	X		X	X				X		X	X	
EQ-5D-5L VAS	X											
FACT-G			X				X					
PROMIS-10					X					X		
SF-12												
SF-36												
MoCA			X									
MDASI	X									X		
Happiness and activity scale									X			
PGIS					X							
PGIC	X				X							
MPN-SAF TSS					X							
AdvSM-SAF			X									
RSCL Activity Level Scale	X											
WPAI	X											
NCSLC-SAQ						X						
MFSAF					X				X			X
WOMAC PVNS-GCTTS									X	X		
PROMIS Physical Function (PF) items												
EORTC QLQ-BR23	X											
EORTC QLQ-BR45	X											
EORTC QLQ-H&N35					X		X					
EORTC QLQ-OES18			X									
EORTC QLQ-OV28				X								
EORTC QLQ-CX24				X								
EORTC QLQ-HCC18			X									
EORTC QLQ-PR25											X	
EORTC QLQ-LC13						X						
TTD in EORTC QLQ-C30	X											
MCL-Specific Symptoms (EORTC Item Library)												X
FACT-B	X											
FACT-M		X							X			
FACT-C								X				
FACT-MM					X							
FACT-LYM					X							
FACT-P											X	
FACT-Taxane	X											
FACT/GOG-Ntx4	X		X									
FLymSI-18												
FACT-B TOI-PFB	X											
CTSQ					X							
OOCAT Survey					X							
PRO-CTCAE	X				X	X						
HADS	X					X					X	
BDI	X					X					X	
PHQ-9												
MAX-PC												
C-SSRS						X						

- US-FDA approved 145 oncology therapies which were studied in 3106 trials.
- Over the past decade, n=558 (17%) of the total clinical trials included a PRO.
- Of the total clinical trials that included a PRO, 2.5% clinical trials used it as a primary outcome, whereas 97.4% used it as a secondary outcome.
- PRO use was the highest in breast cancer (16%), hematological malignancies (18%), lung cancer (20%), urological cancers (21%), and gastrointestinal cancers (20%).
- PROs assessing HRQOL and functional status were the most commonly used in clinical trials.
- EORTC-QLQ-C30 was the most frequently used measure across hematological , lung and breast cancers.
- FACT-measures were predominantly utilized in hematological malignancies and urological/prostate cancers.

Figure 2: Clinical Trials With and Without the Inclusion of PRO

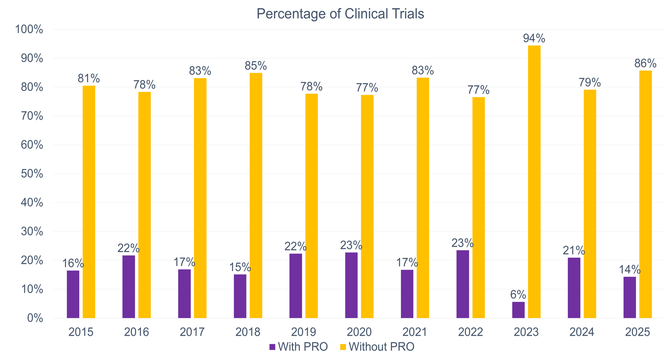
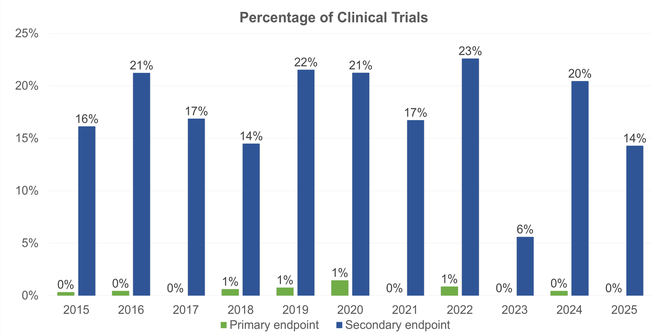


Figure 3: Clinical Trials with PRO as a Primary vs Secondary Outcome



Conclusion

- In the past decade, breast, lung, and hematological cancers have seen the highest number of trials with PRO use.
- While PROs were included in only 17% of clinical trials supporting FDA-approved oncology therapies, their use was largely limited to secondary outcomes.
- Only 2.5% of trials that included PROs used them as a primary outcome, highlighting a missed opportunity to center patient-reported experiences in evaluating treatment efficacy.