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PATIENT AND FAMILY PROMS: KEY TO UNLOCK PATIENT AND FAMILY CENTERED-CARE

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www.acuteleuk.org

Disclosure

- No conflict of interest

Pathway to person-centered care

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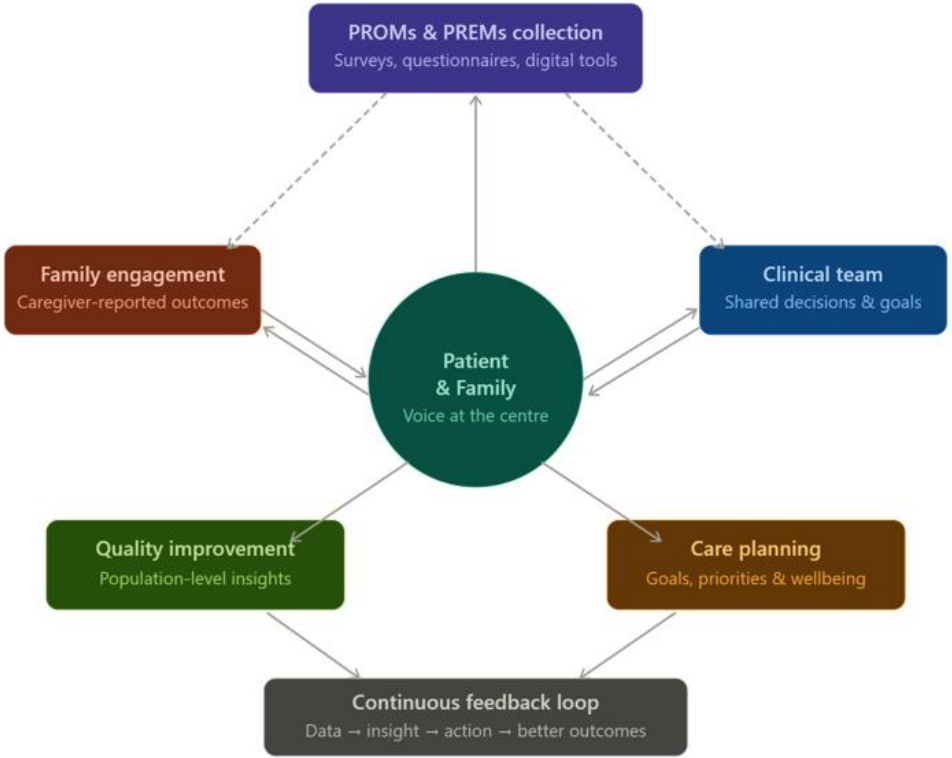

Definition

- **PROMs** (Patient-Reported Outcome Measures) and **FROMS** (Family-Reported Outcome Measures) — and their companion **PREMs** (Patient-Reported Experience Measures) are structured tools that capture health outcomes, quality of life, symptoms, and care experiences directly from patients and families, without clinical interpretation acting as a filter in both clinical trials and clinical practice. They complete the picture of care quality.

- They capture what matters most to patients and their families.

Why PROMs and FROMS unlock patient and family-centered care

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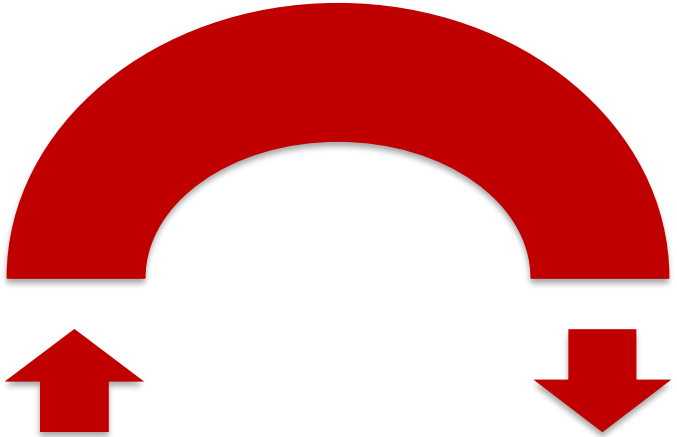
- **They shift the question from "what's the matter with you?" to "what matters to you?"** Traditional clinical metrics measure what clinicians can observe or test. PROMs capture what patients actually feel — pain, fatigue, emotional wellbeing, functional ability — making the invisible visible.
- **They enable shared decision-making.** When a clinician reviews a patient's PROMs before a consultation, the conversation begins from the patient's lived experience rather than from test results alone. Goals and treatment priorities become collaborative.
- **FROMs** (Family Reported Outcome Measures) are especially important in pediatrics, elderly care, and disability services, where family members serve as essential proxies and partners. Their perspective on burden, coping, and home-life impact is often invisible without a structured instrument.
- **At the population level**, aggregated PROMs data can reveal which interventions actually improve quality of life, drive quality improvement cycles, and support commissioning decisions grounded in what patients value — not just what is clinically measurable.
- **The feedback loop is what makes it transformative.** PROMs only unlock their potential when the data flows back: to the individual (their own trajectory over time), to the care team (prompting timely action), and to the system (driving improvement). Without that loop, they remain a survey rather than a lever.

FROM CLINICAL TRIAL OUTCOMES TO CLINICAL PRACTICE

The translation gap is real – and PROMs are the bridge

Data Capture
HM-PRO,FROM-16, disease-specific measures

Real-World Validation
Evidence Based Advocacy



Clinical Trial
PROs as secondary endpoints

Clinical Practice
Routine PROM / FROM in outpatient

Clinical Trials

Role of PROs in Trial Design

- **PROs can be included** as primary or secondary endpoints in clinical trials
- **Increasingly recognized** by regulators, clinicians, and patients as essential patient-centered tools
- **Capture critical data** on symptoms, financial toxicity, and health-related quality of life
- **Several trial design considerations are unique to PROs** — instrument selection, recall periods, missing data, and clinically meaningful thresholds
- **Historically more common in later-phase** (II/III) trials for assessing tolerability and symptom benefit
- **Growing momentum to integrate PROs** into early-phase dose-finding and tolerability studies
- **PROs are evolving** from supplementary tools into core components of trial architecture across the full development continuum

Key Challenges

- **Missing data and compliance** — especially over long follow-up periods
- **Instrument selection** — validated tools must be used; not all PROs are equally fit-for-purpose
- **Burden minimization** — over-assessment can reduce patient adherence
- **Early-phase integration** — PROs remain underutilized in Phase I/II trials
- **Standardization across trials** — heterogeneity in PRO strategies has historically limited regulatory utility

Data capture – How PROs are collected

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Key Moments of Capture

- **At clinic visits** (in-person, on a device)
- **Between visits** (remote/daily diaries via app)
- **At specific time points** in a trial protocol (e.g., baseline, week 4, week 12)
- **Event-triggered** (e.g., after a symptom episode)

Collection methods

- **Paper-Based Questionnaires** The traditional method — patients fill out standardized forms (e.g., SF-36, EQ-5D, PROMIS) on paper at clinic visits. Simple but prone to data entry errors and delays.
- **Electronic PRO (ePRO)** The modern standard. Data is captured digitally via:
  - **Tablets or kiosks** at clinical sites
  - **Web-based portals** accessed from home
  - **Mobile apps** on smartphones (most common today)
  - **Interactive Voice Response (IVR)** — phone-based for less tech-savvy patients
- **Wearables & Passive Capture** Emerging approach where devices (smartwatches, biosensors) passively collect patient data like activity, sleep, and heart rate to complement self-reported data.

Important Principles in PRO Data Capture

Principle	Why It Matters
Standardized instruments	Enables comparison across studies
Ecological Momentary Assessment	Captures data in real time, reducing recall bias
Compliance monitoring	Tracks whether patients are completing forms on time
Data integrity & audit trails	Required for regulatory submissions (FDA, EMA)
Multilingual support	Ensures accessibility across populations

Regulatory considerations

- The **FDA** and **EMA** have issued guidance requiring that PRO instruments used as clinical trial endpoints be validated, culturally adapted, and collected in a way that minimizes missing data and bias.
- ePRO systems must also comply with **21 CFR Part 11** (electronic records standards).

Evidence based advocacy

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Qualitative and Quantitative

- Patient interviews
- Online bulletin boards
- Discreet choice experiment
- Surveys

Some work conducted

- Global QoL survey
- Global patient and carers experience survey
- Patient preference study
- Carer preference study

> 2500 leukemia patients

> 600 family members/partners of patients affected by leukemia

From >80 countries

Source: ALAN publication www.acuteleuk.org/publications

Quality of Life in Acute Leukemia: Global Findings

90%

of patients did not recognise their symptoms as leukemia before diagnosis

ALL

patients reported the worst QoL of all leukemia subtypes; women had significantly higher burden than men

Choice of PRO

Pain, cognitive impairment and tiredness scored highest in patient preference studies — yet cognitive impairment and tiredness are not captured by EQ-5D.

≥50%

of caregivers reported a very large or extremely large impact on their own quality of life

56%

Of patients only partially understood — or did not understand — the information given by their clinician at diagnosis

Fatigue

was the main side effect across all leukemia types, but AL patients were more likely to experience loss of appetite, nausea, and sleeping problems

#1

factors worsening QoL: feeling isolated and reduced ability to carry out meaningful activities

Burden on patients

50%+ of AL patients felt isolated
70%+ had to stop working due to disease
Younger age, female gender, and lower income were the strongest predictors of poor QoL.

Shared decision-making deficit

Only 43% of patients and 42% of carers felt sufficiently involved in treatment decisions.

Source: ALAN / JPCRR 2023; Picker 2024

The missing holistic approach

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What Trials Measure	What's Often Missing	What ALAN Data Shows
• Complete remission rate • Overall survival • Progression-free survival • Adverse event grading (CTCAE)	• Fatigue & cognitive impact • Emotional distress / depression • Social isolation & relationships • Caregiver burden & family QoL • Eating habits & daily function	• Isolation is a key predictor of poor QoL • Younger patients & acute leukemia report highest burden • Family members carry significant invisible burden • Clear communication improves patient-reported outcomes

Source: ALAN Global QoL Survey (552 patients, 42 countries, 2019); Nier et al. Cancer Control 2026; Oliva et al. Front Hematol 2025

Clinical practice : why systematic routine assessment is essential ?

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Current Reality

- PROMs remain a research tool, not a clinical standard
- QoL data collected in trials rarely reaches routine practice
- Many PRO instruments are outdated & not leukemia-specific
- Clinician-patient communication gaps are common and unmeasured
- Family/carer needs are almost systematically overlooked

What Routine Assessment Enables

- Early identification of distress, isolation, and functional decline
- Tailored support services informed by patient-generated evidence
- Improved clinical communication and shared decision-making
- Comparable data across clinical trials and real-world practice
- Validation of caregiver needs alongside patient outcomes

Mechanisms to unlock patient and family-centered care

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1. Embed PROMs and FROMs in Routine Clinic

Mandatory HM-PRO, FROM-16 or equivalent at every haematology outpatient visit — not just in trials. Capture data at multiple timepoints in the patient journey.

2. Include Family/Carer FROMs

Extend assessment beyond the patient. ALAN's global data shows 40% of family members experience QoL impact — this must be systematically measured and addressed.

3. Standardise Instruments

Adopt disease-specific validated tools (e.g. HM-PRO, FROM-16) that allow comparison across trials and settings. Ensure translation into multiple languages.

4. Improve Clinical Communication

Train clinicians to use PROMs and FROMs data during consultations. ALAN evidence shows clear communication with patients is significantly associated with better QoL outcomes.

5. Inform Support Services

Use PROMs and FROMs data to design targeted psychosocial, financial, and practical support interventions — particularly for younger patients and carers of acute leukemia patients.

6. Integrate into HTA & Policy

PROMs and FROMs data must feed into HTA decisions. Advocate for tools to be codified with orphan codes and integrated into EU Joint Clinical Assessments and national HTA frameworks.

Source: ALAN Publications 2019–2026 | acuteleuk.org/publications

THE UNCOMFORTABLE TRUTH

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*"We should move from **reported** to **relevant** in patient reported outcomes as many PROs are outdated and not specific to leukemia. We end up not measuring what matters to patients. Patient involvement in quality of life data collection is key" – Zack Pemberton-Whiteley, EHA 2023*

"Survivorship is not just survival. Patient preferences are key to understanding what patients want and how they want to survive" — Jan Geissler, EHA Patient Joint Symposium 2023

QoL should be a co-primary endpoint and studies should be designed to identify what's most important to patients – EHA Patient Joint Symposium 2025

"Survival at any price is not everyone's priority: subgroups of acute leukemia patients are willing to trade treatment response against quality of life." - ALAN patient preference research · EHA 2025

"Unaddressed adverse effects, such as fatigue, significantly reduce patient confidence in care plans and negatively impact quality of life" for people with lymphoma and CLL – Lorna Warwick, EHA 2025

The evidence is clear — PROMs and FROMs work.

Evidence based advocacy work that validated PROMs and FROMs captures what survival statistics miss: isolation, depression, family burden, unmet communication needs.
These are not soft outcomes — they are predictors of adherence, wellbeing, and long-term health.

The time for pilot projects and “we should” is over.

We are not delivering for our patients — or for those who care for them.
We need systematic, routine, validated PROMs and FROMs — for patients AND their families — embedded in every clinical encounter, every trial, and every HTA submission.

Key ALAN Publications: Nier et al. Cancer Control 2026 · Oliva et al. Front Hematol 2025 · Salek et al. Front Hematol 2025 · Pemberton-Whiteley et al. J Patient Cent Res Rev 2023

The change we need

FROM		To
• Survival as the gold standard	→	• Survival + QoL as co-equal endpoints
• Patients as recipients of care	→	• Patients as partners in care design
• Family as bystanders	→	• Family as active stakeholders with their own FROMS
• PROMs and FROMs as research tools	→	• PROMs and FROMs as standard clinical practice
• One-size PRO/FRO instruments	→	• Disease-specific, validated, multilingual measures

An agenda for change – we need to act now !

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01	**Immediate: Systematic PROMs /FROMs**	All hematology centres adopt routine validated PROMs/FROMs at diagnosis, during treatment, post-treatment, and at relapse. Implement family FROMs alongside patient PROMs.
02	**Short-term: Trial Design Reform**	Make QoL a co-primary endpoint in all new AL clinical trials. Agree on shared validated instruments. Ensure patient involvement in trial design.
03	**Medium-term: Data Translation**	Build real-world registries that link trial PRO/FRO data to clinical outcomes. Use evidence-based advocacy work to validate and extend trial findings to underrepresented populations.
04	**Medium-term: Support Services**	Use PROMs and FROMs data to commission psychosocial support, caregiver respite, financial counselling, and communication skills training — particularly for younger patients and acute leukemia.
05	**Long-term: Policy Embedding**	Integrate FROMs and PROMs into EU Joint Clinical Assessments and national HTA frameworks. Advocate for orphan code status for PRO/FRO-driven evidence.

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