

Understanding the Patient Experience in Early Breast Cancer: A Review of Patient-reported Outcome Measures used in Real-world Evidence

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PCR144

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Objectives

- There is need to better understand patient-reported outcome measures (PROMs) in the context of observational studies in the real world to capture what is important to the early breast cancer (eBC) population and patient experience.
- This review aimed to evaluate PROMs used in the eBC patient population and understand the relevant concepts of patients with eBC that assess important aspects of health and impacts on daily life.

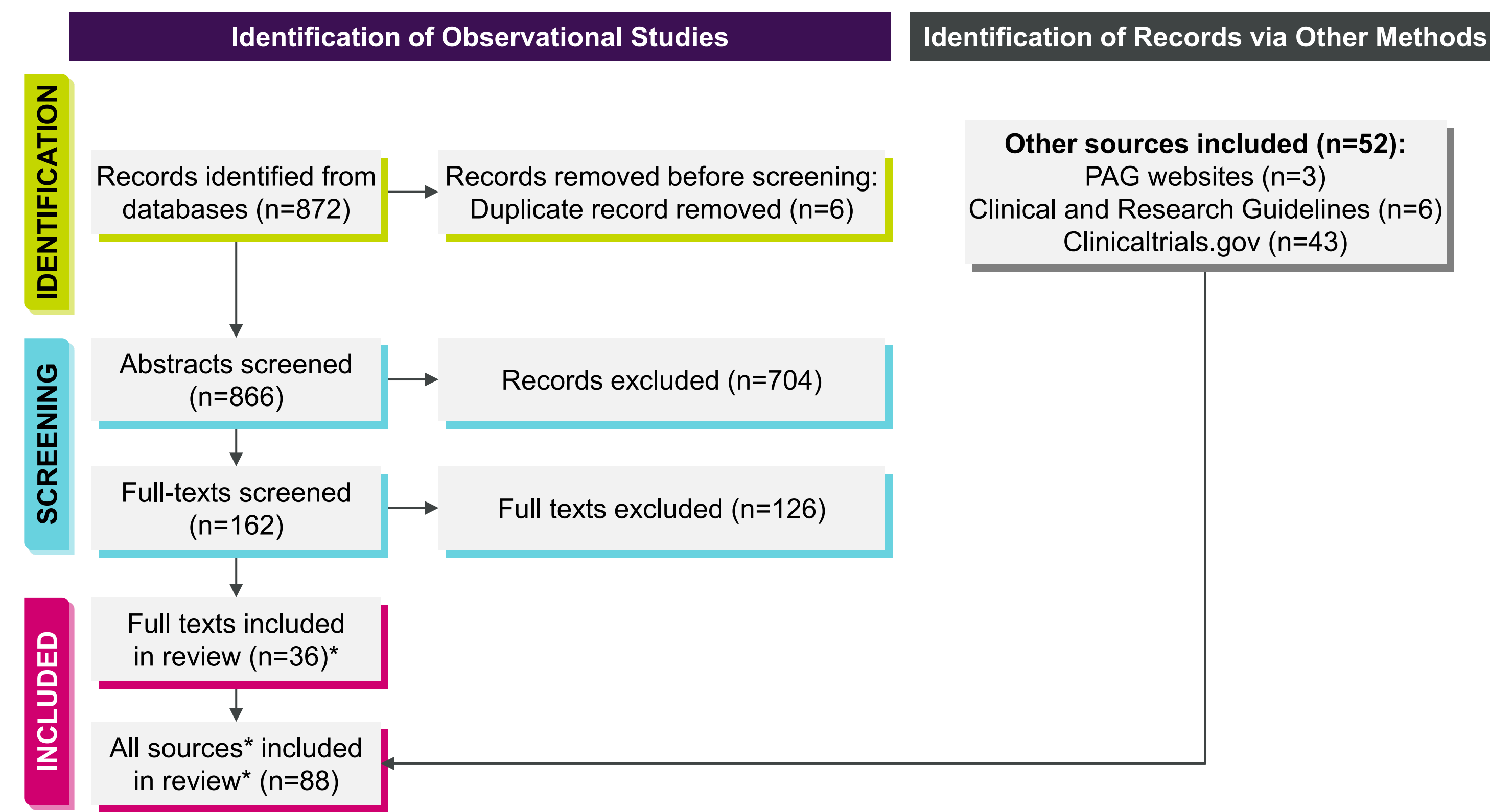
Plain Language Summary

- **Why did we perform this research?** To understand how eBC experiences are captured in observational studies. Observational studies gather real-life data on peoples' experiences, unlike clinical trials which test new treatments in controlled environments.
- **What was done?** We summarized relevant PROMs and health concepts identified from relevant studies in eBC. PROMs are tools to measure health and well-being from the perspective of people with lived experience.
- **What are findings of this research?** A wide range of symptoms, impacts, as well as unmet needs were identified, highlighting the challenges of individuals living with eBC. A total of 65 different PROMs were identified, however, these need to align with what matters most for the eBC community.
- **What are implications of this research?** A shortlist of PROMs capture important aspects of living with breast cancer, however, experience from individuals living with earlier stages of breast cancer is still needed to identify the most critical aspects. This will help ensure that future research in real world settings focus on what is most important to people throughout their treatment pathway.

Methods

- A targeted literature review was conducted via MEDLINE/PubMed and Embase databases to identify articles published from 2016–2022 based on pre-defined inclusion/exclusion criteria.
- Patient organization websites, clinical trials.gov, and clinical/research guidelines were also reviewed for eBC health concepts and PROMs. PROMs were extracted from selected articles, followed by concept mapping to align PROMs with patient concepts.

Figure 1. PRISMA Diagram and Summary of Resources



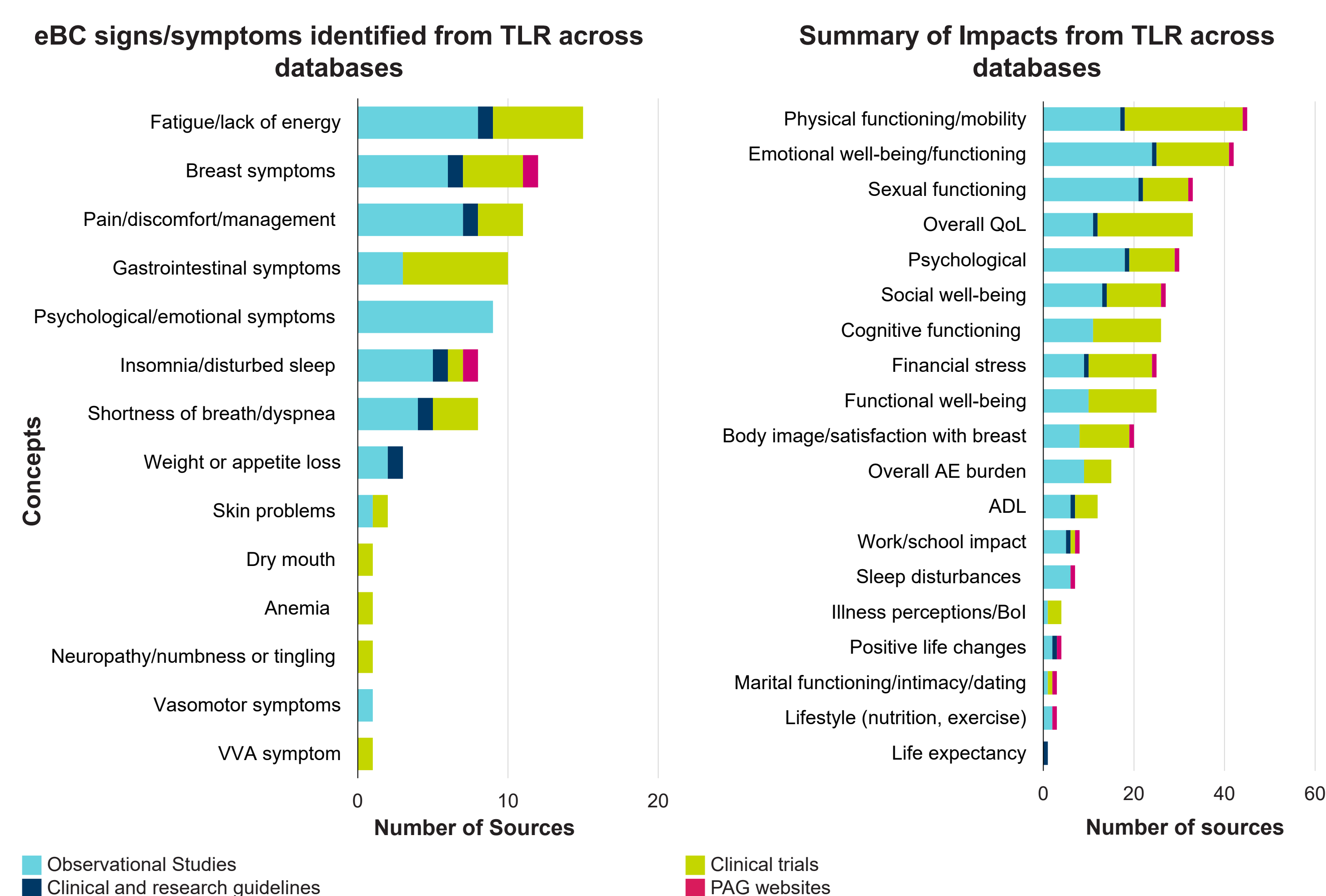
The review focused on observational studies, which were mostly quantitative prospective cohort and cross-sectional survey studies; only four qualitative or mixed-methods studies were found, identifying the need for additional patient input.

Results

Summary of Health Concepts

- Important health concepts included breast symptoms, fatigue, pain/discomfort, as well as impacts relating to physical functioning, activities of daily living, sexual functioning, social and family impacts, financial concerns, and work. Emotional impacts of *anxiety*, *depressive symptoms*, *irritability*, *mood changes*, *post-traumatic stress* were critical themes across the treatment journey.
- Several concepts related to unmet needs in psychological support, financial assistance, symptom management communication, and treatment options were underrepresented in the literature.

Figure 2. eBC concepts in Observational Studies and other Databases



Gastrointestinal symptoms included nausea/vomiting; Functioning well-being included role functioning; Overall QoL included well-being/self-care/global health; Overall AE burden included treatment-related side effect bother/burden of therapy; Social well-being included functioning/family support; Sexual functioning included well-being/enjoyment/distress

Abbreviations: ADL = activity of daily living; AE = adverse event; CT = clinical trials; eBC = early breast cancer; EORTC QLQ-BR23 = European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire Breast Cancer; EORTC QLQ-C30 = European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire Core 30; FACT-B = Functional Assessment of Cancer Therapy–Breast; FACT-G = Functional Assessment of Cancer Therapy–General; HADS = Hospital Anxiety and Depression Scale; OS = observational studies; PAG = patient advocacy group; PRISMA = Preferred Reporting Items for Systematic Reviews and Meta-Analyses; PROMs = patient-reported outcome measures; PROMIS = Patient-reported Outcomes Measurement Information System; QoL = quality of life; SF-36 = 36-item Short Form Health Survey; TLR = targeted literature review; VVA = vulvovaginal atrophy

Results (cont.)

- **A variety of side effects were frequently reported from the literature review:** *Energy-related side effects (e.g., fatigue)*, *gastrointestinal symptoms*, *pain*, *sleep disorders*, *arm and breast symptoms*, and *organ-related side effects*.
- These six side effects were reported in relation to surgery followed by adjuvant or neoadjuvant treatments, radiotherapy, chemotherapy, and other systemic therapies.

Summary of PROMs

- In total, 25 disease-specific and 40 generic PROMs were identified.
- PROMs most frequently used in observational studies (OS) and clinical trials (CT) included:
 - European Organization for Research and Treatment of Cancer Quality of Life Questionnaire Core 30 (EORTC-QLQ-30)¹ (n=12 OS/25 CT)
 - European Organization for Research and Treatment of Cancer Quality of Life Questionnaire Breast Cancer (EORTC-QLQ-BR23)² (n=8 OS/11 CT)
 - Functional Assessment of Cancer Therapy–General (FACT-G)³ (n=3 OS)
 - Functional Assessment of Cancer Therapy–General (FACT-B)⁴ (n=3 OS/3 CT), Breast Q (n=3 OS)
 - Functional Assessment of Cancer Therapy–Endocrine Subscale (FACT-ES)⁵ (n=2 OS/2 CT)
 - EQ-5D-5L⁶ (n=2 OS/8 CT), 36-item Short Form Health Survey (SF-36)⁷ (n=2 OS/1 CT)
 - Functional Assessment of Chronic Illness Therapy–Fatigue (FACIT-F)⁸ (n=2 OS/2 CT)
 - Hospital Anxiety and Depression Scale (HADS)⁹ (n=3 OS), and Impact of Event Scale (IES)¹⁰ (n=3 OS).
- Concept mapping was conducted on a selection of PROMs that were frequently reported and/or potentially addressed patient concerns highlighted in the literature.

Figure 3. PROM Mapping: Impacts (Concept Coverage of Selected PROMs)

CONCEPTS	EORTC QLQ-C30 ¹	EORTC QLQ-BR23 ²	FACT-G ³	FACT-B ⁴	EQ-5D-5L ⁶	SF-36 ⁷	HADS ⁹	PROMIS-29 ¹¹
Cognitive functioning	■							
Physical functioning	■	■	●		■	■		■
Basic ADLs (dressing, brushing teeth, eating, etc.)	●				■	●		
Instrumental ADLs: Role, other daily activities/functioning	■		●		■	■		■
Emotional/psychological Impacts	■	▲	■	▲	■	■	■	■
Social Impacts/relationships	■		■			■		■
Sexual functioning/enjoyment		■	●					
Body image		■		■			▲	
Impact on work/school	▲		●					●
Financial Impact	■							

■ = Concept directly addressed in instrument; ▲ = Concept indirectly addressed in instrument; ● = Concept coverage but discrepancy with scoring. Only concepts for impacts from each instrument were assessed; symptom concepts are NOT included in this figure; psychometric properties were not evaluated. FACT-B concepts cover the breast cancer subscale only.

Conclusions

- This review identified PROMs that address a range of health concepts that can be applied towards individuals living with eBC in the real-world setting.
 - The PROMs identified in this review are in line with other literature, which highlights the EORTC-QLQ-30 and FACT-B as common measures in breast cancer for clinical research and clinical settings.¹²⁻¹⁴
 - Many of these measures, however, were developed for patients later in the disease stage and assume individuals with eBC will be able to notice the signs and symptoms of cancer, however, eBC patients may be asymptomatic.
- Given the complexity of eBC, the next phase of this work will review these results with patient experts. Qualitative interviews with eBC patients will also be undertaken to further understand patient perceptions and barriers of current PROMs in the real-world setting. This will help ensure future prospective research adequately reflects the variety of patient experiences, diagnostic differences, and treatment pathways, ultimately supporting real-world decisions for patients.

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Additional references available upon request.

Acknowledgements

Editorial and graphic design support were provided by Michael Grossi and Kawthar Nakayima of Evidera Inc., a business unit of PPD, part of Thermo Fisher Scientific, which was contracted by AstraZeneca to conduct this study. We also acknowledge the contribution of Kimmie McLaurin (AstraZeneca), Clara Lam (AstraZeneca) and Dorsa Khazaei (previously Evidera).