

# Assessment of Patient Reported Outcomes in Chronic Versus Acute Disease: A Comparison of Study Design and Analytic Approaches

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## Background

- The assessment of patient reported outcomes (PRO) in new drug development has been systematically aligned to study visits and PRO assessment schedules using recall periods such as daily diary, weekly, or monthly reports.
- Through qualitative patient inquiry, researchers query patients on their day-to-day symptoms and impact on daily living and function that they experience with a condition to ensure that the recall period best aligns to that experience.
- For chronic and acute conditions, variability in symptoms, function, and other impacts should be considered in the schedule of assessment, derivation of endpoints, and methods for analyzing the data.
- Specifically, in chronic conditions, scoring as a weekly endpoint has little implication in the evaluation of change. In this case, an average score may be derived as the patient is often in a continuous health state with minimal daily variability.
- However, in acute conditions, which is generally event driven, systematic collection of PROs may pose a challenge as the event will likely contribute to immediate changes in the patient’s health state. In this case, averaging the score over a period risks the loss of information on the patient’s day experience.
- In acute conditions, it is important to consider both the general state of patient functioning as well as the impact of unexpected events such as disease exacerbations or flares, adverse events, treatment side effects, and potential use of rescue medication to get a better understanding of a patient’s experience during a clinical trial
- The assessment schedule for patient reported outcomes in chronic versus acute conditions must consider the daily variability in disease.
  - Is the condition episodic where peak days of symptom may occur (e.g., endometriosis, dysphagia)?
  - Is the condition constant with little symptom variability over the course of a week (e.g., treatment resistant depression)?
  - Are the symptoms unidimensional and homogenous representing various levels of severity on a concept (e.g., fatigue)?
  - Do the symptoms vary in terms of concepts, e.g., heterogenous yet scored as a single symptom domain (e.g., gastrointestinal symptoms)?

## Objective

- This research presents common PRO assessment schedules as well as considerations for a variety of chronic and acute diseases to provide guidance for collecting and analyzing patient outcomes in chronic and acute conditions.
- This includes several published examples of analytic methodologies in depression and pain using the PROMIS item banks to demonstrate variability in chronic and acute conditions.

## Schedule

- The following suggested strategies for developing the schedule of assessments begins with the context of use for each PRO as well as consideration of the type of condition, chronic versus acute.

**Table 1** Schedule of Assessments: Chronic Condition

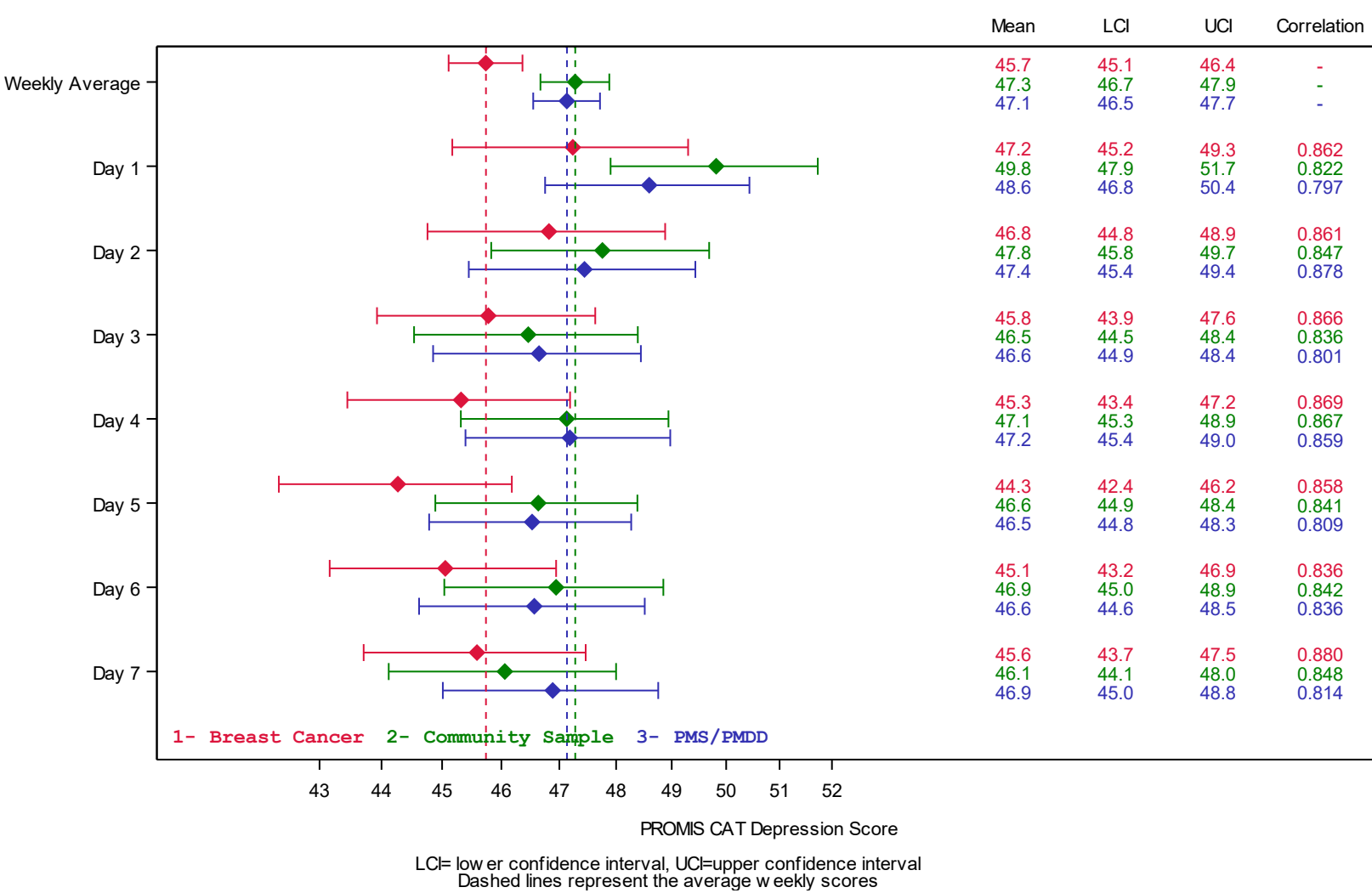
Condition	Week 1	Week <i>j</i>	Weekly Average	Biweekly Average
Chronic Condition Symptom Measure	Average or sum score over the course of each week		Weekly Average	Biweekly Average
Anchor Items	Last 7 days			
Functional Outcome	Last 7 days			

**Table 2** Schedule of Assessments: Acute Condition

Condition	Day 1	Day <i>j</i>	Daily Average	Weekly Average
Acute Condition Symptom Measure	Daily score and weekly average score over the course of each week		Daily Average	Weekly Average
Anchor Items	Last 7 days			
Functional Outcome	Last 7 days			

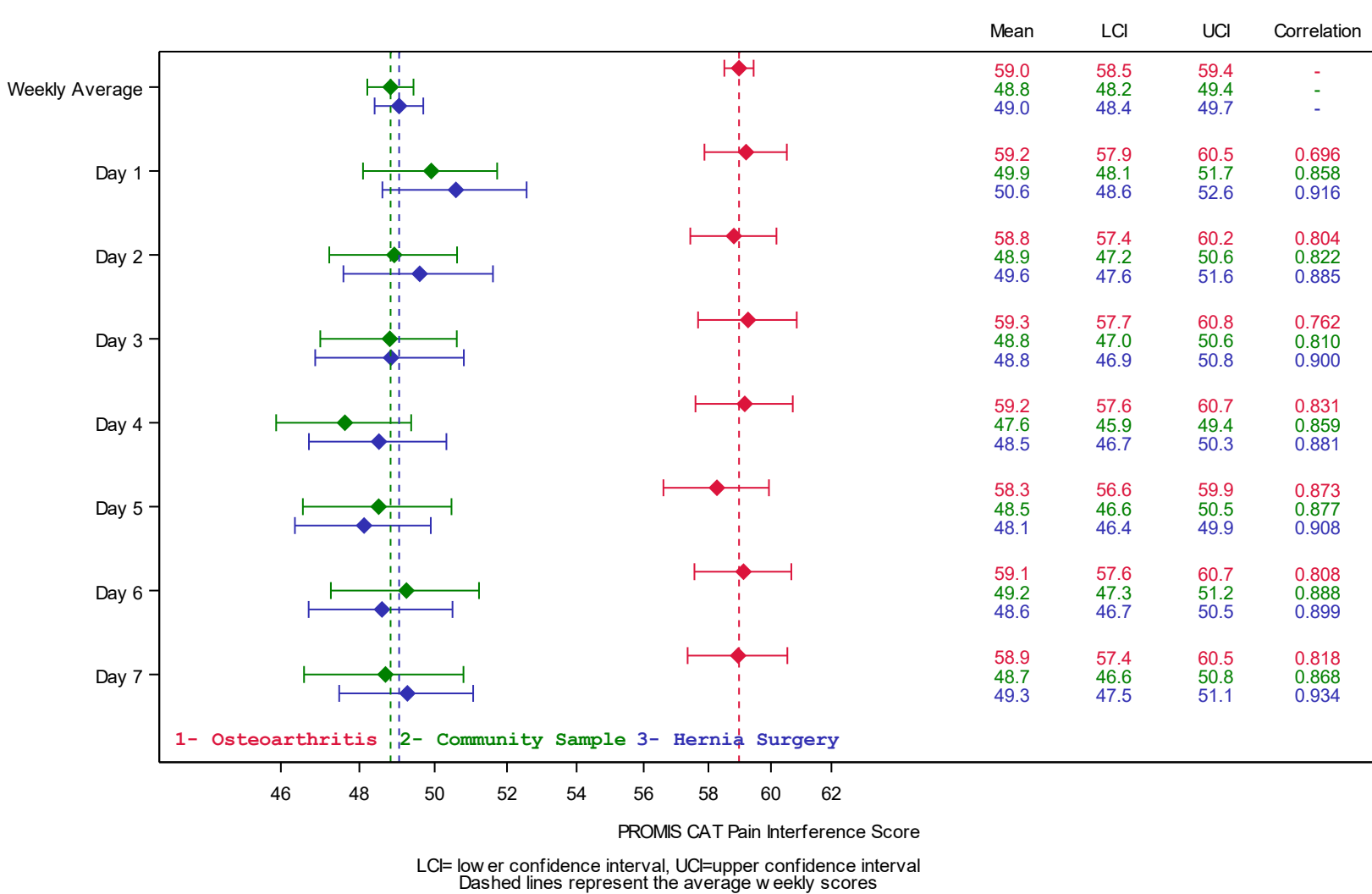
## Example 1: Depression

- Depression symptoms may vary over the course of the week due to the underlying condition.
- Analysis of daily and weekly scores assist in the interpretation of patient symptom experience.



## Example 2: Pain

- Pain is an acute condition which, at the symptom level, varies daily and should be assessed using a daily diary.
- Pain interference, as shown here, has minimal daily variability.



Purnajo, I., Kapadia H., Floden, L. and Hudgens, S., 2022, November. Methods comparing daily diary and weekly assessments in various acute and chronic populations using PROMIS CAT questionnaires. In *QUALITY OF LIFE RESEARCH* (Vol. 31, pp. S22-S23). VAN GODEWUICKSTRAAT 30, 3311 GZ DORDRECHT, NETHERLANDS: SPRINGER.

## Conclusions

- In chronic and acute conditions, it is important to consider the assessment schedule and the related endpoint strategy to properly analyze and communicate interpretable patient outcomes.

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