

INTRODUCTION

- There is a paucity of evidence regarding a well-defined, reliable, and feasible method for measuring efficacy outcomes from a patient perspective in Community-Acquired Bacterial Pneumonia (CABP) trials.
- Most CABP studies evaluate treatment efficacy on the basis of clinical outcomes; however, there is no patient-reported outcome (PRO) measure to capture additional symptoms of how patients feel, function, or survive. The Foundation for the National Institutes of Health (FNIH) Biomarkers Consortium and ICON plc are developing these clinically relevant endpoints that measure tangible benefits for patients in clinical trials of antibacterial drugs.

PURPOSE

- The goal of this study was to explore CABP symptoms as reported by patients, and to develop a draft PRO instrument designed to comprehensively assess these symptoms.
- The information gathered in concept elicitation interviews, expert reviews and cognitive debriefing interviews will be used to modify and expand the conceptual framework which forms the basis of a new CABP-specific PRO instrument.

METHODS

- Concept elicitation (CE) was conducted by telephone interviews with patients within 10 days of CABP diagnosis. Data were analyzed using an iterative process to identify themes and concepts and was recorded in a saturation grid. Saturation was monitored according to the FDA PRO guidance. Using this qualitative data, a draft PRO instrument was prepared. Cognitive debriefing interviews were conducted to assess item readability, relevance, comprehensiveness, and content validity.
- Twenty patients participated in concept elicitation interviews and the demographics and patient characteristics as reported below in Table 1.

TABLE 1. CE Patient Characteristics and Demographics

Characteristic	Distribution (N=20)
Age	
Mean (SD)	59.5 (18.8)
Range	29-90
Sex	
Female	9 (45%)
Male	11 (55%)
Race/Ethnicity	
Caucasian	12 (60%)
Hispanic	-
Black/ African American	6 (30%)
Asian	1 (5%)
American Indian/Alaskan Native	1 (5%)
Education	
High School/GED	6 (30%)
Some college	5 (25%)
Associate's degree	1 (5%)
Did not complete high school	3 (15%)
Bachelor's degree	5 (25%)
Employment Status	
Student	-
Employed full-time	4 (20%)
Employed part-time	1 (5%)
Retired	5 (25%)
Semi-Retired	1 (5%)
Unemployed/seeking work	1 (5%)
Temporarily unable to work	2 (10%)
Permanently unable to work	4 (20%)
Other (e.g. self-employed)	2 (10%)
Other Characteristics	
Patient was hospitalized	20 (100%)
Mean days in hospital (SD) ¹	3.5 (1.9)
Recent trauma ²	1 (5%)
Comorbidity ³	14 (70%)
Previous CABP	7 (35%)
Smoking (past or current) ⁴	12 (60%)
History of alcoholism	1 (5%)
Positive microbiological culture ⁵	2 (10%)

¹Data only available for 11 patients; ²⁻³Data only available for 17 patients; ⁴Data only available for 19 patients; ⁵Culture not taken for 7 patients; unknown result for 1 patient

RESULTS

- The most common symptoms reported included a lack of energy or tiredness (N=18), cough (N=16), and shortness of breath (N=16). Nearly half the patients also reported fever, chest pain and general aches/pain as well as significant impacts on their social (N=10) and physical functioning (N=17) related to CABP. Figure 1 below shows the frequency of all spontaneously reported symptoms. Report of symptoms reached saturation at interview sixteen.
- In addition to identifying symptoms associated with CABP, the CE interviews resulted in the spontaneous report of how these symptoms impacted functioning. Symptoms negatively affected physical functioning, social functioning/relationships, activities of daily living, emotions, and sleep (difficulty sleeping and increased sleep). Figure 1 also shows the frequency of all spontaneously reported impacts. Reports of the impacts on functioning reached saturation at interview four.
- Subsequent cognitive debriefing in 9 patients and 3 clinical experts demonstrated that the items in the draft PRO were understandable, relevant, and interpreted as intended. Following cognitive debriefing, the conceptual framework was revised to represent the item numbers, specific domains, and total symptom score (Figure 2).

FIGURE 1: Frequency of Symptoms and Impacts in CABP (N=20)

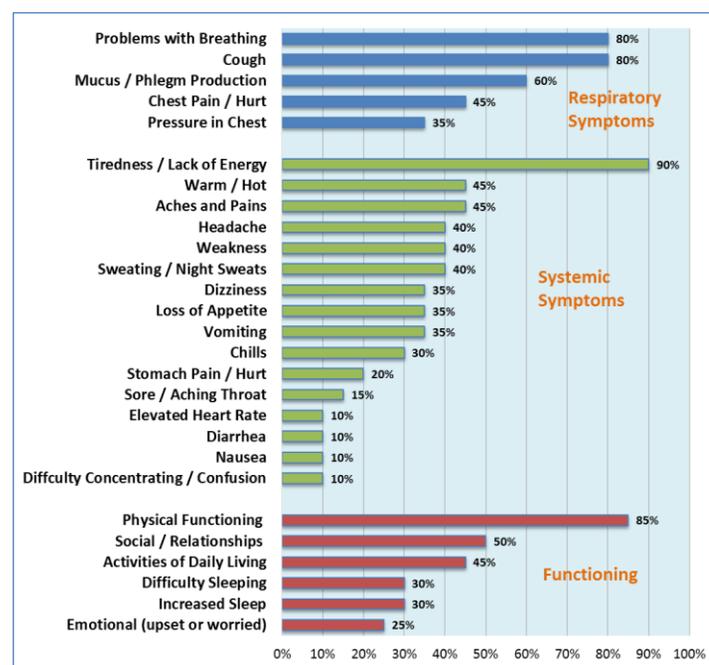
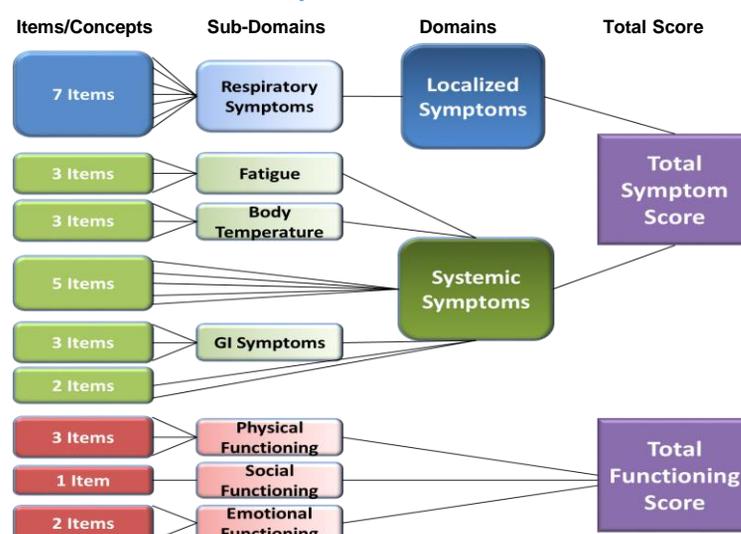


FIGURE 2: Final Conceptual Framework Model



CONCLUSIONS

- Qualitative data from patients and input from experts formed the basis of the CABP PRO structure and item pool. These patient-reported CABP symptoms were shown to demonstrate content saturation and concept validity and provide unique information important for both comprehensive evaluation of individuals with CABP and evaluation of new antibacterial treatments.
- With completion of the PRO instrument development work described above, a daily symptom diary with established content validity is now ready for psychometric validation.

REFERENCES

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See Poster PIN83 for ABSSSI PRO measure development related to this project

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