

# Dementia Methods Pre-Summit Materials

## Session 3

### Measuring Disease Impact: Collecting Information from People with Dementia and their Caregivers

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## I. Introduction

Patient self-report enables researchers to capture information only accessible to patients and may contribute to the measurement of therapeutic intervention effects (Frank et al., 2011). However, concerns about insight and the ability to complete standardized questions limit acceptance of patient self-report from people with dementia in intervention trials.

Based on eight key informant interviews supplemented by a targeted review of the published literature, this report provides background information for the five research questions to be discussed during Session 3 of the Dementia Methods Pre-Summit.

1. What is the value of direct self-report by people with dementia?
2. What is known about the strengths and limitations of self-report by people with dementia?
3. For what domains are caregiver or clinician reports preferable and why?
4. How can technology expand collection of information directly from people with dementia?
5. What are best practices in obtaining consent for participation of people with dementia as research subjects?

The interviewed key informants hold a variety of leadership positions in such diverse organizations as regulatory agencies, academic medical centers, and service, research, and advocacy non-profits, and represent the stakeholder communities of researchers, clinicians, quality measurement experts, and PCORnet affiliates.

## II. Findings from Key Informant Interviews

### Value of Direct Self-Report by People with Dementia

When asked generally about the value of self-report by people with dementia, four of eight key

informants spontaneously sought to clarify the interviewer use of the term “people with dementia” noting that there are multiple forms of dementia and people are at different stages of disease progression. The key informants identified three factors critical to determining value of self-report by people with dementia: (1) individual differences before diagnosis or illness; (2) heterogeneity in dementia types; and (3) temporal changes due to disease progression. These factors should be considered as context across the individual sections below.

**Unique and essential information.** Key informants emphasized that only the individual can report on his or her subjective and lived experience. As explained by one researcher, “...self-reports [from people with dementia] are absolutely the same as self-reports from anyone with any type of disease and condition. When you want to understand the experience of having that disease or condition (whatever it might be), it is essential to really understand the perspective of the person with the illness.”

**Respect.** One researcher key informant called out that “an organizing principle for all research” should be “respect for persons as participants, including persons with dementia who have the ability to communicate and share.” Similarly, when asked about the value of self-report by people with dementia, another researcher explained that “it helps engage the person in the research process which is pretty important. It personifies instead of objectifying the person, which is a major hazard in dementia research.”

**Patient input on self-report measures.** Many key informants found that people with dementia can inform:

- the research questions that should be asked based on patient-expressed interests and concerns
- how the questions can be asked in the language patients use and understand when reporting on their experiences in clinical settings

**Partners rather than subjects.** Some key informants suggested including people with mild-to-moderate cognitive impairment as research advisory group members or part of focus group discussions to inform the development of research questions. This topic is addressed specifically in Session 1 of the Pre-Summit.

### **Limitations of Self-Report and Challenges with Collecting Data**

Overall, key informants most often mentioned the inability to speak and lack of insight as limiting factors for collection and use of self-report.

**Heterogeneity in capacity.** Key informants pointed to two main areas in which people with dementia

change in their capacity to self-report as their disease progresses: their ability to understand the questions being asked, and their ability to communicate their responses to questions. While increased severity of disease can affect one or both of these, key informants continued to emphasize the individual variability in people with dementia and their change in capacity for self-report.

**How to ask.** Key informants reflected on the challenge of asking people who have cognitive impairment how they experience that impairment.

**No guideposts for accepting self-report.** All key informants noted that disease progression (severity of disease), and expression of disease (e.g. changes in behavior, memory, and insight) affect a person with dementia's ability to participate in self-reported assessment. These factors, among others, make it difficult to generalize about the stage at which people with dementia can provide valid and reliable self-report. Several key informants commented generally that they encountered difficulties obtaining self-report from people with dementia whose disease progression had reached a stage that affected their expressive communication ability.

**Disease expression, individual characteristic, or comorbidity?** Key informants identified a need to distinguish between symptoms of the disease and premorbid characteristics of the individual (e.g., has the person always avoided health care? Always been non-communicative about health issues?).

**Ad hoc rather than systematic assessment.** One researcher informant perceived that clinical care may offer more opportunity to incorporate viewpoints of people with dementia than is found in research because clinical practice does not demand generalizing to a broader population. Research requires a more systematic approach to identifying who has adequate mental capacity to provide reliable self-report and domains to which they can contribute.

**Overcoming challenges to acceptance of self-report.** Of three key informants asked about their experience with clinician and researcher resistance to self-report, two noted that resistance has diminished over the past several years and the third reported not having experienced the issue. One suggested that to manage resistance "[we could] start from a commitment that we're going to incorporate the viewpoints of the person who is affected by the condition...designing research procedures [to accommodate them...and developing] a systematic way of making a decision, criteria to screen [that would give] people some level of comfort, with structure, support and clarity."

**Efficiency and insight.** Key informants identified other factors influencing whether self-report is sought from people with dementia, such as the additional time required to communicate with a person with dementia when a caregiver is available for more "efficient" information gathering. They also shared concerns about how a person with dementia's lack of insight can affect the accuracy of their self-report in ways that could lead to negative outcomes (e.g., reporting that they are preparing all of their meals when they are not eating and their refrigerator is empty).

## Domains for Self-Report

**Subjective experiences and outcomes.** Most key informants emphasized the importance of obtaining people with dementia's self-report on subjective outcomes, indicating other ways data on more objective outcomes might be gathered (i.e., observation, assessment instruments). One key informant identified the specific need to correct the error of applying what is known about the early impairment of factual reporting that occurs for people with Alzheimer's to other forms of dementia. This key informant emphasized the importance of recognizing that some people with dementia are able to provide accurate self-reports on objective outcomes.

**Functional status and ADL.** One key informant noted that people with dementia should respond about their abilities to help understand cognitive decline. Some key informants stated that informant [proxy] report was considered appropriate, valid, and reliable for domains like functional status.

**Ability tests for self-report.** Several key informants deemed cognitive tests inadequate for assessing a person with dementia's ability to self-report—especially on experiences that are not related to their memory but instead concern their subjective experience. One researcher specified the need for systematic research to test tools for determining if a person can self-report.

**Augmenting data collection.** Given concerns raised about the accuracy of both self-report and informant report, and the complex array of factors that influence them, several key informants discussed the importance of employing or developing a variety of strategies and tools to either triangulate data points, or facilitate and improve reporting quality. Among the examples provided were training informants about how to report, clarifying that their role is not to provide their own perspective, but rather to answer on behalf of the individual; ensuring the informant's capacity to report is not impaired; investigating relationships between data from people with dementia, informants and biomarkers, MRI scans and other approaches; training researchers and clinicians in best practices for communicating with people with dementia; conducting more research into more objective ways to measure outcomes, including the development of more user-friendly (e.g., actigraphy) and more accurate (e.g., pedometers) technology; and researching non-verbally communicated self-reports (e.g., facial expressions and behaviors) from people with dementia.

## Best Practices in Obtaining Informed Consent

Several key informants opposed cognitive screening tests being used to exclude any person with dementia from participation in a research study, and yet capacity to understand was important to informed consent. One respondent suggested an article—in which the author noted that half of 434 research articles reviewed used the Mini Mental State Examination (MMSE) to determine capacity to participate in studies, and more importantly, the review suggests a pattern of frequent, unexplained and sometimes not acknowledged exclusion of people with dementia (Taylor et al., 2012).

Three key informants spontaneously suggested not using the standard IRB form or process. Their suggested solutions to IRB challenges and to consenting people with dementia included:

- Modifying the language in a step-wise fashion while reviewing it with people with dementia (adaptive consent). Researchers would essentially apply best practices in communication to the consent process, asking for example, “Tell me, what are we going to do?”.
- Asking a series of questions: “Are you here for a birthday party?” “Are you here for research?” – to demonstrate they understand they are participating in research.
- Designating an informant for a time when the person with dementia is no longer able to consent (e.g., applying an advanced directive model/approach to research consent). This would be especially important for longitudinal research as the disease progresses and ability to consent changes.
- Requiring participation of a legal representative of the person with dementia in the consent process for higher risk research and for patients in later stages of disease progression.
- Re-consenting people with dementia throughout the study when it comprises longitudinal research—although one person noted that this approach would be too time consuming and suggested evaluating capacity instead.

### **Use of Technology for Data Collection**

Across the interviews, key informants expressed interest in this topic area but had limited direct experience. One key informant has used ActiGraph to study sleep activity level, and pedometers—finding the latter more useful as a motivational tool than an outcome measure, due to measurement variability. Also this key informant indicated one important challenge with the use of technology can be the need for a caregiver to oversee its use—especially if it is complicated or confusing (including some pedometers).

## **III. Key Points from the Literature**

**Lack of congruity between patient and caregiver report on subjective measures.** Studies have found low correlation between self-report and caregiver ratings (Beer et al., 2010; Andrieu et al., 2016). Beer found that family and staff rated QOL scores seven points lower than self-ratings, and supported the validity of the self-ratings by the association of pain and restraint use with lower scores. Andrieu reported “For total QOL-AD scores, 50% of caregivers underestimated patient QOL compared with self-ratings, whereas 12% of caregivers overestimated patient QOL, and 38% agreed with patient ratings (Andrieu et al., 2016, p429).



**Caregiver depression, burden and disease progression influence proxy reporting.** Informant report accuracy may also be influenced by factors such as caregiver (or other informant) experience of depression (Bowling et al., 2015; Frank et al., 2011); caregiver burden or health; and disease progression in the person with dementia—all of which increase the likelihood of an inverse relationship between patient and caregiver report (Frank et al., 2011).

**Conceptual framework for patient self-report is lacking.** Bowling and colleagues examined multiple measures of quality of life for dementia patients, finding that few were based on rigorous conceptual frameworks and most were based on expert opinion rather than with input from the dementia population.

**Tools such as memory aids may help.** Rubright and colleagues studied the use of MacArthur Competence Assessment Tool for Clinical Research (MacCAT-CR) alone or with a memory and organizational aid, a one page summary of the informed consent information. Those that used the memory and organizational aid were more likely to be judged competent and had higher MacCAT-CR scores. In other words, it addressed patients' deficits in memory and attention.

## **IV. Priority Issues in Methods**

**Conceptual frameworks.** Some key informants called for better conceptual frameworks for measures, as one noted, "We don't have an appropriate conceptual framework for QOL [quality of life] and we need the conceptual framework to help organize future research."

**Longitudinal research.** Several key informants had conducted--or emphasized the high value of conducting--longitudinal studies with people with dementia. Key reasons given for prioritizing longitudinal research were to: (1) answer the research question, "What is the normal course of aging" by evaluating a large population cohort over time; and (2) overcome the limitation of cross-sectional study designs, which do not capture individual variability in insight and other individual characteristics. For example, longitudinal studies would allow investigators to distinguish between impaired insight that is a function of disease progression from individuals with limited insight before disease onset, or at the earliest stages.

**Informant vs self-report.** Many key informants identified the need to compare and balance informant reports and what people with dementia report. Key informants expressed reservations and concerns about how the impact of dementia on caregivers' experiences affects their ability to accurately report on behalf of people with dementia. In addition, the nature, length, and intensity of the relationship between the informant and the person with dementia should be considered when assessing the value of informant report.

Multiple key informants called for research on ways to objectively measure outcomes without relying



on informants as the primary source of data. To meet this need, key informants suggested advancing research on assessing insight in people with dementia, especially as disease progresses through later stages of illness. One key informant suggested future work could build on her previous research comparing patient and caregiver ratings of decline in memory over time based on the Informant Questionnaire on Cognitive Decline in the Elderly (IQ-CODE). This key informant also suggested some approaches to real-time self-assessment of the insight of people with dementia into their own memory loss. This involves a series of brief memory tests. Before administering the tests, individuals are asked how well they think they are going to perform on the test. After the test is completed, they are asked how well they think they performed on the test. This awareness of their own deficits in real-time could be of use, and would build on the key informants past work, as well as more current studies in this area.

**Enhancing self-report by obtaining non-verbal information.** Some key informants suggested investigating whether post-verbal people with dementia can provide valid and reliable (elicited v. spontaneous) self-report using nonverbal methods (facial expressions and behaviors). If so, it would allow them to report on more domains and at later stages of disease progression. Similarly, there was strong interest in developing methodological strategies to facilitate self-report by people with dementia. Some suggested visual materials (e.g., modeled after the pain scale) as possible facilitators to study.

**Understand the need from the patient perspective.** Throughout the interview, one clinician key informant emphasized the importance of addressing QOL concepts and measurement issues to help with developing a coherent conceptual framework for discussing, understanding and developing programs of research that better align with people with dementia's ability to answer different questions about their experience.

## **Appendix I: Methods**

### *Key Informant Interviews*

The Dementia Methods Pre-Summit Steering Committee prospectively identified a set of eight key informants representing a variety of stakeholder communities and organization or institution types and initiated contact. The group of informants interviewed represented the communities of: clinical practice (2); academia/research (4); regulatory authority (1) and quality measurement (1).

AIR contacted all eight identified key informants and all agreed to participate. Using a protocol developed collaboratively between PCORI and AIR, AIR conducted thirty-minute, semi-structured interviews with each informant.

Prior to scheduled interviews, AIR developed profiles of key informants' research backgrounds and areas of expertise to focus the brief interviews on those topics that would leverage their knowledge and research experience. Following each interview, the interviewer and note taker discussed the individual's unique comments and perspectives and added these comments to the set of themes emerging across interviews.

Interview questions were pre-coded to the five research questions identified for Session 3 of the Pre-summit as shown in Table 1. Two questions were coded as a single item given content overlap (question 1 around value and question 2 about strengths and limitations). After reviewing notes from each interview and categorizing content by the research question, two investigators open-coded the data, independently reading and developing themes and patterns for each question.

**Table 1. Research Questions**

Agenda Questions	Structured Interview Guide Questions
<p>1. What is the value of direct self-report by people with dementia?</p> <p>2. What is known about the strengths and limitations of self-report by people with dementia?</p>	<p>Have you conducted or reviewed research involving individuals with dementia who self-report on experiences or outcomes?</p> <p>What do you view as the value of self-report by people with dementia, in research studies?</p> <p>What are the challenges or limitations of self-report by people with dementia?</p> <p>Overall, how would you summarize the value of direct self-report by people with dementia in dementia research?</p> <p>What are the different considerations for research relative to clinical care, in terms of collecting information directly from people with dementia?</p> <p>In your research, have you experienced resistance by researchers, clinicians, or care facilities, to asking people with dementia to self-report about their experiences?</p> <p>What kind of resistance? How have you overcome that?</p> <p>As you know “engagement” of non-scientists in research is increasing, and such engagement in research is a requirement for research funding from some funders. How can people with dementia be directly involved, as research partners rather than research subjects, in research on interventions intended to benefit them?</p> <p>What are some facilitators of this involvement, as a research partner or consultant, not as research subject?</p> <p>What are some barriers to this direct involvement as a partner or consultant?</p> <p>What role might caregivers and clinicians have in facilitating engagement in research by people with dementia?</p>
<p>3. For what domains are caregiver or clinician reports preferable and why?</p>	<p>In your view what are the critical areas for which additional research is needed in order to accept any self-report from people with dementia?</p>

Agenda Questions	Structured Interview Guide Questions
4. How can technology expand collection of information directly from people with dementia?	<p>What are your experiences with different technologies for data collection?</p> <p>How can technology expand collection of information directly from people with dementia?</p> <p>Are there specific types of technology you would recommend for specific circumstances? Which ones and when?</p>
5. What are best practices in obtaining consent for participation of people with dementia as research subjects?	<p>We are interested in best practices in obtaining informed consent with people with dementia. What are your experiences with obtaining consent in this population?</p> <p>What would you identify as best practices in obtaining consent for participation as research subjects in this population?</p> <p>What are the best ways to assess a person’s capacity to self-report?</p> <p>How should changes in capacity to provide informed consent be assessed in longitudinal studies? (How frequently should capacity be assessed?)</p>
6. Concluding Questions	<p>When you think about research on care and services for people with dementia and their caregivers what are the top areas to focus on?</p> <p>How should funding agencies prioritize among the potential research needs?</p> <p>For example, is further research needed on obtaining informed consent from people with dementia?</p> <p>What additional research is needed to understand incorporation of new technology in patient self-report in dementia?</p> <p>What are research needs for understanding the capacity to self-report?</p> <p>What information is needed on effective and meaningful engagement of people with dementia in research, as research partners? How would you prioritize the top research needs?</p>

In qualitative research, the goal is to present the depth and breadth of findings based on themes.<sup>1</sup> Quantification of respondents is provided here as general guidance for extent to which a theme was evident in this small sample.

<sup>1</sup> Denzin, N., & Lincoln, Y. (Eds.). (2011). Handbook of qualitative research (4th ed.). Thousand Oaks, CA: Sage.

## Literature Review

The AIR team conducted an abbreviated review of literature related to the five research questions. We used key terms<sup>2</sup> to identify an initial set of peer-reviewed articles. From the initial set we identified additional articles by scanning the references (snowballing approach). Given the breadth and depth dementia research, we limited our articles to those published in the past 15 years and used two additional filtering criteria to prioritize our article section. The first is the number of citations to date<sup>3</sup> and the second is the journal impact factor. We scanned abstracts of candidate articles to further assess relevance and conducted in-depth review of the full texts of 17 articles. Below we provide an annotated bibliography of ten articles that influenced the report.

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<sup>2</sup> These key terms are: (“dementia” or “Alzheimer”), (“patient report” or patient-reported or self-report” or “proxy report” or PRO or PROM)/“consent”/“technology”.

<sup>3</sup> Note, the number of citations is affected by how recently the article has been published.

## Appendix II: Bibliography

Andrieu, S., Coley, N., Rolland, Y., Cantet, C., Arnaud, C., Guyonnet, S. ... & Vellas, B. (2016). Assessing Alzheimer's disease patients' quality of life: Discrepancies between patient and caregiver perspectives. *Alzheimer's & Dementia*, 12(4), 427-437. The objectives for this study were to explore discrepancies between self and caregiver reports of patient QOL in a large (N=1131) population of community-dwelling persons with Alzheimer's Disease at baseline and during 2 years of follow-up and to determine factors associated with disagreement at baseline.

Beer, C., Flicker, L., Horner, B., Bretland, N., Scherer, S., Lautenschlager, N. T., ... & Almeida, O. P. (2010). Factors associated with self and informant ratings of the quality of life of people with dementia living in care facilities: A cross sectional study. *PLoS One*, 5(12), e15621. Researchers recruited 351 people with dementia living in residential care facilities and compared QOL as reported by patients, family caregivers and staff informants. QOL was measured using self and informant The authors concluded that "the majority of people with dementia living in residential care facilities can rate their own QOL. Informant ratings underestimate self ratings of QOL of people with dementia, and appear to be associated with factors which are not associated with self ratings." (p1)

Bowling, A., Rowe, G., Adams, S., Sands, P., Samsi, K., Crane, M. ... & Manthorpe, J. (2015). Quality of life in dementia: A systematically conducted narrative review of dementia-specific measurement scales. *Aging & Mental Health*, 19(1), 13-31. The researchers conducted a systematic narrative review of studies using QOL measures for people with dementia. They assessed the scope and domains included in the QOL measures, theoretical and conceptual frameworks, and the extent of user or patient involvement in their development. The authors concluded most measures were only loosely based on conceptual frameworks, usually referring to the work of Lawton. The authors found that "the items and scales [were] mainly...developed based upon researchers' ideas rather than upon patient/client conceptualizations, and many measures [were] based upon proxy measurement by caregivers--which, though perhaps apt in cases of severe dementia, are of questionable validity for people with mild to moderate dementia." (p28)

Darlak, L., Bass, D. M., Judge, K. S., Wilson, N., Looman, W., McCarthy, C. & Kunik, M. E. (2017). Engagement of veterans with dementia in partners in Dementia care: An evidence-based care coordination program. *Journal of Applied Gerontology*, 36(5), 570-591. This paper describes the engagement of veterans with dementia in a community and medical care coordination intervention model that encourages participation by people with dementia despite their cognitive impairment. Results provide an example of the extent and limits of these veterans' engagement in psychosocial interventions.

Dupuis, S. L., Gillies, J., Carson, J., Whyte, C., Genoe, R., Loiselle, L., & Sadler, L. (2012). Moving beyond

patient and client approaches: Mobilizing 'authentic partnerships' in dementia care, support and services. *Dementia*, 11(4), 427-452. This paper argues against "well-intended but often paternalistic approaches that place patients or clients at the centre of care, but rarely, if ever, actively involve them in decision-making" and that the stigma and misunderstanding surrounding dementia lead to an erroneous assumptions about the capacity of people with dementia to be involved in care for themselves and others.

Frank, L., Lenderking, W. R., Howard, K., & Cantillon, M. (2011). Patient self-report for evaluating mild cognitive impairment and prodromal Alzheimer's disease. *Alzheimer's research & therapy*, 3(6), 35. This paper describes the potential value of self-report by persons with early stage Alzheimer's Disease or Mild Cognitive Impairment and the paucity of measures for self-report in domains that are particularly appropriate to self-report, including health-related quality of life, and every day and executive functioning.

Karlawish, J., Cary, M., Moelter, S. T., Siderowf, A., Sullo, E., Xie, S., & Weintraub, D. (2013). Cognitive impairment and PD patients' capacity to consent to research. *Neurology*, 81(9), 801-807. Researchers used a cognitive testing instrument (DS-2) to sort 90 patients with Parkinson's disease into three groups: normal, on the borderline, and impaired, and compared them with a normal cohort of 30 people in the same age range. Then the study examined these patients' decision making capability for two clinical trials: a drug trail and a surgical trail, using two sets of instruments (MacCAT-CR, and MoCA) and also compared the results with three expert judgments.

Lichtner, V., Dowding, D., Esterhuizen, P., Closs, S. J., Long, A. F., Corbett, A., & Briggs, M. (2014). Pain assessment for people with dementia: A systematic review of systematic reviews of pain assessment tools. *BMC geriatrics*, 14(1), 138. This study evaluated 28 observational pain assessment tools. None could be recommended for use in any clinical setting due to lack of comprehensive evidence in reliability, validity, feasibility or clinical utility.

Rubright, J., Casarett, D. J., Gur, R., Xie, S. X., Karlawish, J., & Sankar, P. (2010). A memory and organizational aid improves Alzheimer disease research consent capacity: results of a randomized, controlled trial. *The American Journal of Geriatric Psychiatry*, 18(12), 1124-1132. The authors tested whether a memory and organizational aid improves AD patient performance on measures of capacity and competency to give informed consent. Results showed that a consent process that addresses an AD patient's deficits in memory and attention can improve capacity to give informed consent for early phase AD research.





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Taylor, J. S., DeMers, S. M., Vig, E. K., Borson, S. (2012). The disappearing subject: exclusion of people with cognitive impairment and dementia from geriatrics research. *Journal of the American Geriatrics Society*, 60(3), 413-9. The authors examined 434 articles examined and found that 16% used recruitment methods likely to reduce participation by persons with cognitive impairment. Approximately 29% of the studies specified the criteria of exclusion; half of these used the Folstein Mini-Mental State Examination (MMSE), while approximately a fifth excluded individuals based on a non-specified dementia. Slightly less than half provided a justification for exclusion and 14% discussed exclusion as a possible limitation.