Dementia Methods Pre-Summit Materials

Session 4

Study Design and Implementation: Testing Interventions for People with Dementia and their Families

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I. Introduction

A well-established gap in the research evidence base surrounding non-pharmacological therapies (i.e., care programs and supportive services) aimed at directly assisting people with dementia persists (Maslow 2012; Kelly 2016). Obtaining evidence on outcomes of care and services for people with dementia continues to be a high priority among researchers, patients and the public (Kelly 2016). Relatively few non-pharmacologic therapies have been tested in 'gold standard' large randomized controlled studies (RCT) or have shown consistent results (Alzheimer's Association, 2016). Reviews of nonpharmacological interventions have repeatedly discussed the need for stronger methodology and evaluation criteria. (Gaugler 2011; Minnesota Evidence-based Practice Center, 2016).

Challenges of RCT Research in Dementia

Though the RCT remains the gold standard in evaluation of outcomes, given randomized allocation, manipulation of the treatment arms and a use of a control group, several challenges to the conduct of RCTs exist in non-pharmacological dementia research (Richie 2015; Cohen-Mansfield 2012):

Obtaining consent: Understanding the complexity of research questions and study methods in order to make an informed decision on participation can be difficult in the general population but is especially fraught in a cognitively impaired population.

Failure to assess heterogeneity of treatment effect: RCTs assess the average effect of an intervention over all enrolled subjects and gives little-to-no insight about the components, and tell us little about what determines positive outcomes at the individual level, where interventions may work differentially for each individual.

Lack of representativeness: Inclusion and exclusion criteria and care settings specifically designed for research studies are not representative of a real-world population of people with dementia.

Sample size and power: Recruitment and retention of research subjects with dementia is a challenge for multiple reasons and can hinder the ability to meet sample size targets in RCTs, limiting inferences that can be made about results.

Statistical significance: RCT outcomes are considered successful based on assumptions involving statistical significance. Improvements in outcomes that fall short of this level of significance can still be valuable to people with dementia. Clinically meaningful improvements may differ from statistically significant improvements.

Lack of blinded group assignment: The intervention is observable to participants (with cognitive function intact) and can be divulged to researchers, limiting the effectiveness of intervention assessment

Use of Non-RCT research

Given the challenges of RCTs, use of non-RCT designs in dementia research has value (Cohen-Mansfield 2012). The authors identify three reasons for inclusion of non-RCTs in the evidence base on non-pharmacologic interventions:

1. Nonpharmacologic interventions are easily identifiable by the researcher or observer which complicates blind randomization
2. Applicability of non-RCT results to people with dementia, their caregivers and policy/ decision-makers tested in usual care settings rather than strict standardized settings
3. Can be used when budgetary restrictions limit possible study designs, or when expensive RCTs are unjustifiable

Challenges in the use of non-RCT research in patients with dementia mirror the issues of non-RCT research in general patient populations (Weuve 2015), including:

As well as challenges specific to research with with older patients, name determining with-person change given aging and cognitive decline.

1. Selection bias
2. Measurement imprecision
3. Confounding
4. Determining within-person change given aging and cognitive decline

II. Case Studies in Non-Pharmacological Non-RCT Methodologies

A short description of studies using high-quality non-RCT methodology in the evaluation of non-pharmacologic interventions follows. Additional studies are included in the Study Inventory:

van de Ploeg et al. (2012): A randomized crossover trial to study the effect of personalized, one-to-one interaction using Montessori-based activities on agitation, affect and engagement in nursing home residents:

Methods: A repeated measures cross-over design (N=44) with random allocation of treatment and control order.

Inclusion criteria: chart diagnosis of dementia, agitation occurring at least several times a day outside nursing interventions that was not due to pain or untreated illness; residence in specialist dementia unit or nursing home for at least three months and consent by next of kin or guardian.

Intervention: Montessori-based activities or control blocks for 30 minutes twice weekly for two weeks and then each group switched to the other block.

Assessment: a single target behavior was selected in discussion with staff based on nurses' ratings of residents' behaviors in the previous two weeks using the Cohen Mansfield Agitation Inventory (CMAI). Agitation based on a 1-minute interval at baseline, during and after Montessori or control period.

Outcome: Montessori-activities reduced agitated behavior by 50% and control by 42% compared to baseline (0.93 rate ratio; p=0.527) but increases in positive affect (2.89; p=0.001) and constructive engagement (2.29; p<0.001).

Strengths: This is one of the first studies to use both a baseline and treatment control condition to test the effectiveness of personalized activities (van de Ploeg 2012).

Limitations: Found similar reduction in agitation with the non-personalized control condition in primary outcome, but may show that even a simple social contact intervention may be beneficial to persons with dementia (van de Ploeg 2012).

Low et al. (2013): The Sydney Multisite Intervention of LaughterBosses and ElderClowns (SMILE) study: cluster randomised trial of humour therapy in nursing homes

Methods: A single-blind two-group longitudinal cluster randomized controlled design (n=398) witin 36 nursing homes.

Inclusion criteria: Over 50 years of age, admitted to full-time care in nursing home less than 12 weeks prior, exhibiting risky behavior identified by nursing home staff.

Intervention: Humor therapy intervention: LaughterBoss training for staff members and between 9-12 therapy sessions with ElderClown, a trained performer who provides residents with humor sessions.

Assessment: Cornell Scale for Depression in Dementia (CSDD) collected at baseline, post-intervention (13 weeks; N=371) and follow-up (26 weeks post-intervention; N=343). Secondary measures included the Chen-Mansfield Agitation Inventory (CMAI) and the Neuropsychiatric Inventory Nursing Home.

Outcome: The humor therapy group showed no significant differences on CSDD from the control group but humour therapy group showed reduction CMAI by a mean 0.17 points (p=0.045) points between baseline and follow-up. Change from post to follow-up on the CMAI was statistically significant at 0.21 (p=0.003) points. Residents who experienced higher doses of engagement showed greater improvement on depression, behavioral disturbance and resident-rated quality-of-life.

Strengths: Large sample size, randomization occurred at nursing home level (cluster design) and low levels of attrition (Low 2013).

Limitations: Data was unblinded over time for 15 of 35 homes; homes not necessarily representative of Australian nursing homes in general; and variation in number of humor therapy sessions received (between 9-12); unbalanced groups at baseline although differences were adjusted in analysis (Low 2013).

III. Conclusions

High-quality research into non-pharmacological therapies for people with dementia continues to be a priority (Kelly 2012). Identified above are two of the innovative methodologies for assessing the outcomes of dementia therapies in usual care settings. More work evaluating and sharing best practices in methodological designs will aid in assessing which evaluations are effective in the support and care of people with dementia, and can provide a roadmap for future researchers in conducting studies in this population.

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Appendix: Search String for Inventory Studies

Search (((((((research design[MeSH Terms]) OR research designs[MeSH Terms]) AND Review[ptyp] AND “last 5 years”[PDat])) OR (research methodology[MeSH Terms] AND Review[ptyp] AND “last 5 years”[PDat])) OR (behavioral research[MeSH Terms] AND Review[ptyp] AND “last 5 years”[PDat]))) AND (((dementia[MeSH Terms]) OR (dementia[Text Word] OR dementias[Text Word])))

OR

Search ((((((((((research methodology[MeSH Terms]) OR research design[MeSH Terms]) OR research designs[MeSH Terms]))))) OR behavioral research[MeSH Terms])) AND (((“non-pharmacologic”[Text Word] OR “non pharmacologic”[Text Word] OR “non-pharmacologic*”[Text Word] OR “nonpharmacologic*”[Text Word])) AND (“therapy”[Text Word] OR “intervention”[Text Word]))) AND Review[ptyp] AND “last 5 years[PDat])