**Summary of Research:**

***What is the problem being addressed?***

In the UK, the numbers living with dementia are expected to rise to over a million people with dementia by 2020. There are currently no disease modifying treatments available for the common dementias, and our research knowledge and funding on dementia lagging behind other major diseases such as cancer or heart disease. The National Institute of Health Research (NIHR) has identified the area of disease modification in dementia as important, particularly in the mild and moderate stages, and are supporting trials evaluating potential disease modifying treatments.

However, across previous and current disease modification trials various measurements of outcome are used, making it difficult to compare trials of the same or different types of potential treatments in modifying the disease course of dementia. A standardised set of the most valid and appropriate outcome measures for use in disease modification trials in dementia would improve the efficiency of future trials, and enhance the interpretation of data across studies; enabling meta-analyses combining small data sets that could inform practice.

***What does this study aim to do?***

The aim of this project is to identify outcome measures for all disease modification trials in mild (including early) and moderate dementia. This is in order to report which outcomes are used in trials, and to assess the frequency of outcome use. We will then examine validation measures and use this information to determine possible standardised 'core set' of health outcome measures for trials of disease modification treatments in mild and moderate dementia. We will consult with focus groups from the Alzheimer's society and then present possible measures at a consensus conference to decide upon agreed measures. This will allow direct comparison of future trials of such treatments and will shape and optimise the design of future NIHR and other funders' dementia trials.

***Why is the research needed now?***

The National Institute of Health Research (NIHR) has already identified this as an important area of research. Current trials are developed, funded and set up without any liaison between the trial teams with different choice of outcome measures so that these cannot be compared. Researchers do not agree on which measures should be used. Use

of an agreed set of outcome measures would improve efficiency. This applies to both drugs and non-pharmacological interventions so that the efficacy of, for example, exercise or diet changes, could be compared to new drugs.

## Study Design

There are four stages to this project.

***1. Use of current knowledge:***

We will use any relevant data from existing systematic reviews by co-applicants of the group, including reference lists, to inform the design of this study and avoid duplication.

***2. Systematic review:***

We will conduct a brief systematic review of literature available to date. The review will focus on trials involving people with mild to moderate dementia, aiming to develop a disease modifying therapy. We will exclude studies set in a care home, as very few people resident in care homes will have mild to moderate dementia, and studies where all patients have severe dementia. We will also exclude outcomes that are qualitative, economic, only about carers or those where there are no validation data in people with mild to moderate dementia published or known to the group. This review will inform a list of most frequently used trial measures, including information about the therapy under investigation, the acceptability of the test and the length time associated with the measure.

***3. Patient and Public Involvement (PPI consultation):***

We will conduct three to four focus groups, in partnership with the Alzheimer's Society involving people with dementia, and family carers, to assess which of the included outcomes are most important and appropriate to both people with dementia and carers.

***4. Consensus conference:***

Towards the end of the project all researchers involved in the study will be invited to a consensus conference. This meeting will agree a core set of outcomes to be recommended for use in NIHR applications for trials of disease modification in mild to moderate dementia.

## Why have you been invited to take part?

An important part of this research is to seek the advice of people with dementia and carers on the measures and tests used during clinical trials. You have been invited to participate in a focus group as you have direct experience of dementia as part of the third stage of the project.

You do not have to take part, and if you do decide to join a focus group you are free to withdraw at any time. If you withdraw during the research, we will ask to use the information you gave during any group you have attended, unless you request that it is destroyed.

**What will happen during the focus group?**

The focus groups will last between 2 and 2.5 hours. The discussion will be recorded and transcribed – recordings will be destroyed after transcription. All your contributions will be strictly confidential.

Expenses and refreshments will be provided for all focus group participants. We will also offer you a £30 voucher as a token of our appreciation of you giving up your time to take part.

**What are the possible benefits of taking part?**

There will be no direct benefit but your experience and knowledge could influence future trials to help provide more consistent clinical trials in dementia and thus improve knowledge of the effect of disease modifying treatments.

**Are there any risks involved?**

We don't anticipate any harm as a result of taking part. However, discussions about research participation may raise potentially distressing subjects relating to medical testing. A trained clinician will be part of the meeting and support will be available if any aspect of the discussion causes distress.

**Further Information**

**The dissemination policy:**

We will draft a press release with the UCL media office and the Alzheimer's Society. We have previously worked together in disseminating published work of interest in dementia and have had results in national, international and local press as well as

interviews on TV and the radio. The communications departments of the institutions to which the expert group members belong will be invited to help disseminate the findings.

**Approval by ethics committees**

This project will not be using individual or identifiable patient data, so we do not envisage ethical problems in the carrying out of this research. We have not required research ethics committee permission as the data is in the public domain and we are not engaged in any primary research. We will draw conclusions about outcomes. We will discuss with our research team if any of the interventions included in the review raise ethical dilemmas, and discuss these in the final report.

**Further Questions**

If you have any further questions about this research, please contact

**Anna Grinbergs-Saull (Research Engagement Officer, Alzheimer's Society)**

[redacted]

**Lucy Webster (Research Assistant, UCL Division of Psychiatry)**

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